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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Day 1	n	4	4	4	8	12
	Mean	111.0	92.8	94.0	93.4	99.3
	Median	109.5	92.0	98.5	95.5	100.0
	SD (SE)	11.97 (5.99)	7.72 (3.86)	23.22 (11.61)	16.04 (5.67)	16.67 (4.81)
	Min, Max	98, 127	85, 102	62, 117	62, 117	62, 127
Baseline[1]	n	4	4	4	8	12
	Mean	111.0	92.8	94.0	93.4	99.3
	Median	109.5	92.0	98.5	95.5	100.0
	SD (SE)	11.97 (5.99)	7.72 (3.86)	23.22 (11.61)	16.04 (5.67)	16.67 (4.81)
	Min, Max	98, 127	85, 102	62, 117	62, 117	62, 127

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 12	n	4	4	4	8	12
	Mean	99.0	94.5	93.5	94.0	95.7
	Median	97.5	95.0	95.0	95.0	95.0
	SD (SE)	22.82 (11.41)	10.34 (5.17)	7.72 (3.86)	8.47 (2.99)	13.92 (4.02)
	Min, Max	73, 128	82, 106	83, 101	82, 106	73, 128
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-12.0	1.8	-0.5	0.6	-3.6
	Median	-12.0	4.5	-1.5	1.0	-1.5
	SD (SE)	11.11 (5.55)	15.88 (7.94)	16.78 (8.39)	15.17 (5.37)	14.79 (4.27)
	Min, Max	-25, 1	-20, 18	-20, 21	-20, 21	-25, 21
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-11.7	2.8	3.4	3.1	-1.8
	Median	-11.0	5.1	-1.5	1.1	-1.5
	SD (SE)	11.18 (5.59)	16.65 (8.33)	21.59 (10.79)	17.85 (6.31)	17.02 (4.91)
	Min, Max	-26, 1	-20, 20	-17, 34	-20, 34	-26, 34

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 24	n	4	4	4	8	12
	Mean	90.3	89.0	85.5	87.3	88.3
	Median	95.5	91.0	85.0	87.5	88.5
	SD (SE)	20.19 (10.09)	7.75 (3.87)	7.37 (3.69)	7.25 (2.56)	12.11 (3.50)
	Min, Max	63, 107	78, 96	77, 95	77, 96	63, 107
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-20.8	-3.8	-8.5	-6.1	-11.0
	Median	-15.5	-6.5	-8.5	-7.0	-8.5
	SD (SE)	17.80 (8.90)	8.02 (4.01)	19.23 (9.61)	13.87 (4.90)	16.15 (4.66)
	Min, Max	-46, -6	-10, 8	-32, 15	-32, 15	-46, 15
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-18.7	-3.8	-5.1	-4.5	-9.2
	Median	-13.5	-7.2	-8.7	-7.5	-9.0
	SD (SE)	16.25 (8.13)	8.72 (4.36)	21.49 (10.75)	15.20 (5.37)	16.37 (4.72)
	Min, Max	-42, -5	-10, 9	-27, 24	-27, 24	-42, 24

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 36	n	4	4	4	8	12
	Mean	94.5	98.0	87.3	92.6	93.3
	Median	90.5	100.5	87.0	91.5	91.5
	SD (SE)	26.34 (13.17)	6.88 (3.44)	4.92 (2.46)	7.98 (2.82)	15.18 (4.38)
	Min, Max	72, 125	88, 103	83, 92	83, 103	72, 125
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-16.5	5.3	-6.8	-0.8	-6.0
	Median	-14.0	3.0	-11.5	2.0	-2.0
	SD (SE)	17.23 (8.62)	5.91 (2.95)	26.70 (13.35)	19.02 (6.72)	19.27 (5.56)
	Min, Max	-36, -2	1, 14	-34, 30	-34, 30	-36, 30
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-15.7	5.9	-1.0	2.4	-3.6
	Median	-14.2	3.3	-11.7	2.1	-1.7
	SD (SE)	16.43 (8.21)	6.78 (3.39)	33.95 (16.97)	22.96 (8.12)	22.12 (6.38)
	Min, Max	-33, -2	1, 16	-29, 48	-29, 48	-33, 48

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 48	n	4	4	4	8	12
	Mean	98.5	91.3	90.8	91.0	93.5
	Median	103.5	91.0	91.0	91.0	92.0
	SD (SE)	28.73 (14.37)	10.24 (5.12)	13.07 (6.54)	10.88 (3.85)	17.72 (5.12)
	Min, Max	61, 126	79, 104	75, 106	75, 106	61, 126
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-12.5	-1.5	-3.3	-2.4	-5.8
	Median	-9.0	-2.0	2.0	1.0	-0.5
	SD (SE)	18.79 (9.39)	5.26 (2.63)	28.06 (14.03)	18.72 (6.62)	18.55 (5.35)
	Min, Max	-37, 5	-6, 4	-42, 25	-42, 25	-42, 25
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-12.4	-1.7	2.1	0.2	-4.0
	Median	-8.2	-2.1	2.0	1.0	-0.4
	SD (SE)	18.93 (9.47)	5.83 (2.91)	31.16 (15.58)	20.85 (7.37)	20.32 (5.87)
	Min, Max	-38, 5	-7, 5	-36, 40	-36, 40	-38, 40

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 62	n	4	4	4	8	12
	Mean	98.3	87.8	100.5	94.1	95.5
	Median	98.5	90.5	103.5	100.0	100.0
	SD (SE)	24.92 (12.46)	15.13 (7.56)	17.10 (8.55)	16.43 (5.81)	18.58 (5.36)
	Min, Max	73, 123	69, 101	77, 118	69, 118	69, 123
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-12.8	-5.0	6.5	0.8	-3.8
	Median	-10.5	-7.5	5.0	1.5	0.0
	SD (SE)	18.14 (9.07)	12.57 (6.28)	6.45 (3.23)	11.11 (3.93)	14.57 (4.21)
	Min, Max	-36, 6	-16, 11	1, 15	-16, 15	-36, 15
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-12.0	-5.5	8.9	1.7	-2.9
	Median	-10.2	-7.8	5.2	1.4	-0.1
	SD (SE)	16.87 (8.43)	14.19 (7.10)	10.75 (5.38)	13.95 (4.93)	15.72 (4.54)
	Min, Max	-33, 5	-19, 13	1, 24	-19, 24	-33, 24

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 74	n	4	4	4	8	12
	Mean	89.0	87.0	80.0	83.5	85.3
	Median	92.0	86.5	79.5	83.0	83.0
	SD (SE)	25.17 (12.58)	4.69 (2.35)	6.48 (3.24)	6.44 (2.28)	14.37 (4.15)
	Min, Max	60, 112	83, 92	73, 88	73, 92	60, 112
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-22.0	-5.8	-14.0	-9.9	-13.9
	Median	-24.0	-7.0	-13.5	-12.5	-13.0
	SD (SE)	16.59 (8.30)	8.18 (4.09)	20.83 (10.42)	15.30 (5.41)	16.12 (4.65)
	Min, Max	-38, -2	-13, 4	-40, 11	-40, 11	-40, 11
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-20.7	-5.8	-11.0	-8.4	-12.5
	Median	-21.0	-7.1	-13.7	-12.7	-12.7
	SD (SE)	16.87 (8.44)	8.45 (4.23)	21.44 (10.72)	15.34 (5.42)	16.25 (4.69)
	Min, Max	-39, -2	-14, 5	-34, 18	-34, 18	-39, 18

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 84	n	4	4	4	8	12
	Mean	89.3	85.0	75.0	80.0	83.1
	Median	96.5	84.5	80.0	84.5	90.0
	SD (SE)	25.38 (12.69)	5.83 (2.92)	26.20 (13.10)	18.37 (6.49)	20.28 (5.85)
	Min, Max	53, 111	80, 91	42, 98	42, 98	42, 111
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-21.8	-7.8	-19.0	-13.4	-16.2
	Median	-16.5	-9.0	-13.5	-10.5	-14.5
	SD (SE)	15.90 (7.95)	8.54 (4.27)	24.54 (12.27)	18.04 (6.38)	17.12 (4.94)
	Min, Max	-45, -9	-16, 3	-53, 4	-53, 4	-53, 4
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-20.6	-8.0	-18.4	-13.2	-15.6
	Median	-14.1	-9.3	-12.0	-10.3	-12.7
	SD (SE)	17.17 (8.58)	8.80 (4.40)	26.66 (13.33)	19.20 (6.79)	18.12 (5.23)
	Min, Max	-46, -8	-17, 3	-56, 6	-56, 6	-56, 6

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 96	n	4	4	4	8	12
	Mean	102.3	90.3	72.8	81.5	88.4
	Median	113.0	89.5	79.0	83.0	86.5
	SD (SE)	26.42 (13.21)	11.27 (5.63)	19.05 (9.53)	17.25 (6.10)	22.00 (6.35)
	Min, Max	63, 120	78, 104	45, 88	45, 104	45, 120
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-8.8	-2.5	-21.3	-11.9	-10.8
	Median	-2.5	1.0	-25.0	-7.0	-3.5
	SD (SE)	18.23 (9.11)	10.63 (5.32)	28.35 (14.17)	22.21 (7.85)	20.17 (5.82)
	Min, Max	-35, 5	-18, 6	-50, 15	-50, 15	-50, 15
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-8.7	-2.5	-18.2	-10.4	-9.8
	Median	-1.8	1.0	-22.2	-6.9	-2.8
	SD (SE)	18.50 (9.25)	11.21 (5.61)	32.46 (16.23)	24.00 (8.49)	21.46 (6.20)
	Min, Max	-36, 5	-19, 7	-53, 24	-53, 24	-53, 24

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 120	n	4	4	4	8	12
	Mean	94.0	83.0	82.5	82.8	86.5
	Median	102.0	83.5	86.5	86.0	98.5
	SD (SE)	26.96 (13.48)	17.94 (8.97)	24.37 (12.18)	19.81 (7.00)	21.88 (6.32)
	Min, Max	55, 117	66, 99	52, 105	52, 105	52, 117
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-17.0	-9.8	-11.5	-10.6	-12.8
	Median	-9.5	-10.0	-7.5	-8.0	-9.5
	SD (SE)	17.42 (8.71)	17.44 (8.72)	23.22 (11.61)	19.03 (6.73)	17.98 (5.19)
	Min, Max	-43, -6	-30, 11	-43, 12	-43, 12	-43, 12
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-16.4	-10.4	-9.8	-10.1	-12.2
	Median	-8.0	-11.4	-6.6	-7.1	-8.0
	SD (SE)	18.38 (9.19)	18.90 (9.45)	26.80 (13.40)	21.47 (7.59)	19.88 (5.74)
	Min, Max	-44, -6	-31, 13	-45, 19	-45, 19	-45, 19

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 144	n	4	4	4	8	12
	Mean	93.5	84.8	71.5	78.1	83.3
	Median	101.5	84.5	74.0	75.5	84.5
	SD (SE)	28.90 (14.45)	21.17 (10.59)	15.97 (7.98)	18.75 (6.63)	22.56 (6.51)
	Min, Max	52, 119	65, 105	52, 86	52, 105	52, 119
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-17.5	-8.0	-22.5	-15.3	-16.0
	Median	-8.5	-7.0	-25.0	-18.0	-13.0
	SD (SE)	19.02 (9.51)	19.77 (9.88)	19.62 (9.81)	19.81 (7.00)	18.70 (5.40)
	Min, Max	-46, -7	-31, 13	-43, 3	-43, 13	-46, 13
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-17.0	-8.6	-21.4	-15.0	-15.7
	Median	-7.3	-8.5	-22.6	-19.3	-13.4
	SD (SE)	20.00 (10.00)	21.38 (10.69)	20.75 (10.37)	20.66 (7.30)	19.53 (5.64)
	Min, Max	-47, -6	-32, 15	-45, 5	-45, 15	-47, 15

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 % of Predicted Value (%)						
Week 168	n	4	4	4	8	12
	Mean	94.0	82.0	78.0	80.0	84.7
	Median	95.0	83.0	78.5	78.5	81.5
	SD (SE)	11.60 (5.80)	19.08 (9.54)	4.69 (2.35)	13.04 (4.61)	13.87 (4.00)
	Min, Max	79, 107	61, 101	72, 83	61, 101	61, 107
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-17.0	-10.8	-16.0	-13.4	-14.6
	Median	-18.0	-7.5	-17.0	-14.5	-16.0
	SD (SE)	3.56 (1.78)	18.34 (9.17)	20.51 (10.26)	18.23 (6.44)	14.77 (4.26)
	Min, Max	-20, -12	-35, 7	-40, 10	-40, 10	-40, 10
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-15.4	-11.5	-13.1	-12.3	-13.3
	Median	-15.6	-8.7	-17.2	-16.1	-15.8
	SD (SE)	3.43 (1.72)	19.47 (9.73)	21.11 (10.55)	18.82 (6.65)	15.20 (4.39)
	Min, Max	-19, -11	-36, 8	-34, 16	-36, 16	-36, 16

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Day 1	n	4	4	4	8	12
	Mean	1.515	1.635	1.323	1.479	1.491
	Median	1.480	1.650	1.285	1.445	1.480
	SD (SE)	0.1370 (0.0685)	0.2330 (0.1165)	0.1597 (0.0798)	0.2492 (0.0881)	0.2120 (0.0612)
	Min, Max	1.39, 1.71	1.35, 1.89	1.18, 1.54	1.18, 1.89	1.18, 1.89
Baseline[1]	n	4	4	4	8	12
	Mean	1.515	1.635	1.323	1.479	1.491
	Median	1.480	1.650	1.285	1.445	1.480
	SD (SE)	0.1370 (0.0685)	0.2330 (0.1165)	0.1597 (0.0798)	0.2492 (0.0881)	0.2120 (0.0612)
	Min, Max	1.39, 1.71	1.35, 1.89	1.18, 1.54	1.18, 1.89	1.18, 1.89

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 12	n	4	4	4	8	12
	Mean	1.363	1.695	1.360	1.528	1.473
	Median	1.290	1.870	1.340	1.465	1.340
	SD (SE)	0.2551 (0.1276)	0.4122 (0.2061)	0.1508 (0.0754)	0.3386 (0.1197)	0.3119 (0.0900)
	Min, Max	1.14, 1.73	1.08, 1.96	1.20, 1.56	1.08, 1.96	1.08, 1.96
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-0.153	0.060	0.038	0.049	-0.018
	Median	-0.140	0.100	0.000	0.050	-0.005
	SD (SE)	0.1537 (0.0769)	0.2425 (0.1212)	0.2540 (0.1270)	0.2302 (0.0814)	0.2236 (0.0645)
	Min, Max	-0.35, 0.02	-0.27, 0.31	-0.23, 0.38	-0.27, 0.38	-0.35, 0.38
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-10.450	2.762	4.267	3.514	-1.140
	Median	-9.739	5.587	-0.100	2.971	-0.635
	SD (SE)	10.2100 (5.1050)	16.6725 (8.3363)	19.9854 (9.9927)	17.0574 (6.0307)	16.1510 (4.6624)
	Min, Max	-23.49, 1.17	-20.00, 19.87	-14.94, 32.20	-20.00, 32.20	-23.49, 32.20

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 24	n	4	4	4	8	12
	Mean	1.288	1.620	1.280	1.450	1.396
	Median	1.360	1.750	1.280	1.435	1.380
	SD (SE)	0.2943 (0.1472)	0.2740 (0.1370)	0.1899 (0.0950)	0.2840 (0.1004)	0.2852 (0.0823)
	Min, Max	0.87, 1.56	1.21, 1.77	1.07, 1.49	1.07, 1.77	0.87, 1.77
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-0.228	-0.015	-0.043	-0.029	-0.095
	Median	-0.130	-0.045	-0.060	-0.045	-0.115
	SD (SE)	0.2517 (0.1259)	0.1448 (0.0724)	0.2862 (0.1431)	0.2105 (0.0744)	0.2346 (0.0677)
	Min, Max	-0.60, -0.05	-0.14, 0.17	-0.36, 0.31	-0.36, 0.31	-0.60, 0.31
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-15.142	-1.025	-1.782	-1.403	-5.983
	Median	-8.077	-2.313	-5.012	-2.313	-6.866
	SD (SE)	17.2554 (8.6277)	9.4054 (4.7027)	21.6184 (10.8092)	15.4393 (5.4586)	16.6929 (4.8188)
	Min, Max	-40.82, -3.60	-10.37, 10.90	-23.38, 26.27	-23.38, 26.27	-40.82, 26.27

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 36	n	4	4	4	8	12
	Mean	1.328	1.813	1.365	1.589	1.502
	Median	1.290	1.915	1.350	1.590	1.460
	SD (SE)	0.2922 (0.1461)	0.2646 (0.1323)	0.2783 (0.1391)	0.3470 (0.1227)	0.3412 (0.0985)
	Min, Max	1.04, 1.69	1.43, 1.99	1.07, 1.69	1.07, 1.99	1.04, 1.99
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-0.188	0.178	0.043	0.110	0.011
	Median	-0.180	0.175	-0.005	0.125	0.060
	SD (SE)	0.2323 (0.1161)	0.1021 (0.0511)	0.3696 (0.1848)	0.2612 (0.0924)	0.2821 (0.0814)
	Min, Max	-0.43, 0.04	0.08, 0.28	-0.33, 0.51	-0.33, 0.51	-0.43, 0.51
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-12.591	10.883	4.994	7.939	1.096
	Median	-11.994	10.147	-0.907	8.560	4.084
	SD (SE)	15.8316 (7.9158)	6.2693 (3.1346)	28.9938 (14.4969)	19.6730 (6.9555)	20.4161 (5.8936)
	Min, Max	-29.25, 2.88	5.29, 17.95	-21.43, 43.22	-21.43, 43.22	-29.25, 43.22

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 48	n	4	4	4	8	12
	Mean	1.375	1.793	1.400	1.596	1.523
	Median	1.415	1.820	1.395	1.630	1.530
	SD (SE)	0.3135 (0.1568)	0.2673 (0.1336)	0.2832 (0.1416)	0.3301 (0.1167)	0.3287 (0.0949)
	Min, Max	0.97, 1.70	1.45, 2.08	1.09, 1.72	1.09, 2.08	0.97, 2.08
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-0.140	0.158	0.078	0.118	0.032
	Median	-0.085	0.170	0.110	0.170	0.115
	SD (SE)	0.2796 (0.1398)	0.0403 (0.0202)	0.4125 (0.2063)	0.2747 (0.0971)	0.2923 (0.0844)
	Min, Max	-0.52, 0.13	0.10, 0.19	-0.45, 0.54	-0.45, 0.54	-0.52, 0.54
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-9.254	9.549	8.273	8.911	2.856
	Median	-5.735	9.624	8.276	9.624	8.301
	SD (SE)	18.9886 (9.4943)	1.7247 (0.8623)	31.0896 (15.5448)	20.3956 (7.2109)	21.0486 (6.0762)
	Min, Max	-34.90, 9.35	7.41, 11.54	-29.22, 45.76	-29.22, 45.76	-34.90, 45.76

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 62	n	4	4	4	8	12
	Mean	1.400	1.738	1.573	1.655	1.570
	Median	1.440	1.755	1.595	1.645	1.595
	SD (SE)	0.2678 (0.1339)	0.2691 (0.1346)	0.1338 (0.0669)	0.2156 (0.0762)	0.2548 (0.0735)
	Min, Max	1.06, 1.66	1.40, 2.04	1.39, 1.71	1.39, 2.04	1.06, 2.04
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-0.115	0.103	0.250	0.176	0.079
	Median	-0.110	0.100	0.205	0.165	0.155
	SD (SE)	0.2419 (0.1209)	0.1408 (0.0704)	0.1252 (0.0626)	0.1464 (0.0518)	0.2240 (0.0646)
	Min, Max	-0.41, 0.17	-0.06, 0.27	0.16, 0.43	-0.06, 0.43	-0.41, 0.43
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-7.499	6.375	19.600	12.987	6.159
	Median	-7.167	5.820	15.459	12.024	9.488
	SD (SE)	16.7462 (8.3731)	8.6717 (4.3358)	11.5932 (5.7966)	11.8236 (4.1803)	16.3457 (4.7186)
	Min, Max	-27.89, 12.23	-3.45, 17.31	11.04, 36.44	-3.45, 36.44	-27.89, 36.44

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 74	n	4	4	4	8	12
	Mean	1.300	1.810	1.298	1.554	1.469
	Median	1.320	1.900	1.265	1.445	1.445
	SD (SE)	0.3112 (0.1556)	0.4222 (0.2111)	0.1812 (0.0906)	0.4068 (0.1438)	0.3839 (0.1108)
	Min, Max	0.97, 1.59	1.28, 2.16	1.14, 1.52	1.14, 2.16	0.97, 2.16
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-0.215	0.175	-0.025	0.075	-0.022
	Median	-0.245	0.185	-0.020	0.065	-0.020
	SD (SE)	0.2940 (0.1470)	0.2044 (0.1022)	0.3049 (0.1525)	0.2630 (0.0930)	0.2966 (0.0856)
	Min, Max	-0.52, 0.15	-0.07, 0.40	-0.40, 0.34	-0.40, 0.40	-0.52, 0.40
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-14.074	9.625	-0.153	4.736	-1.534
	Median	-16.094	10.348	-1.726	4.325	-1.473
	SD (SE)	20.2064 (10.1032)	11.9719 (5.9860)	22.6729 (11.3364)	17.5799 (6.2154)	19.8443 (5.7286)
	Min, Max	-34.90, 10.79	-5.19, 22.99	-25.97, 28.81	-25.97, 28.81	-34.90, 28.81

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 84	n	4	4	4	8	12
	Mean	1.320	1.735	1.243	1.489	1.433
	Median	1.415	1.840	1.445	1.475	1.460
	SD (SE)	0.3045 (0.1523)	0.3508 (0.1754)	0.4357 (0.2178)	0.4510 (0.1594)	0.4020 (0.1161)
	Min, Max	0.88, 1.57	1.26, 2.00	0.59, 1.49	0.59, 2.00	0.59, 2.00
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-0.195	0.100	-0.080	0.010	-0.058
	Median	-0.120	0.115	0.035	0.115	-0.005
	SD (SE)	0.2913 (0.1456)	0.1440 (0.0720)	0.3981 (0.1990)	0.2934 (0.1037)	0.2968 (0.0857)
	Min, Max	-0.61, 0.07	-0.09, 0.26	-0.64, 0.25	-0.64, 0.26	-0.64, 0.26
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-12.723	5.447	-6.212	-0.382	-4.496
	Median	-7.495	6.756	3.000	6.756	-0.079
	SD (SE)	19.7246 (9.8623)	8.9831 (4.4915)	32.4253 (16.2126)	22.8915 (8.0934)	21.8288 (6.3014)
	Min, Max	-40.94, 5.04	-6.67, 14.94	-52.03, 21.19	-52.03, 21.19	-52.03, 21.19

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 96	n	4	4	4	8	12
	Mean	1.518	1.878	1.238	1.558	1.544
	Median	1.645	1.930	1.315	1.590	1.645
	SD (SE)	0.3215 (0.1607)	0.3272 (0.1636)	0.4580 (0.2290)	0.5028 (0.1778)	0.4353 (0.1257)
	Min, Max	1.04, 1.74	1.48, 2.17	0.62, 1.70	0.62, 2.17	0.62, 2.17
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	0.003	0.243	-0.085	0.079	0.053
	Median	0.105	0.230	-0.125	0.155	0.155
	SD (SE)	0.3153 (0.1577)	0.1109 (0.0554)	0.4929 (0.2465)	0.3742 (0.1323)	0.3430 (0.0990)
	Min, Max	-0.45, 0.25	0.13, 0.38	-0.61, 0.52	-0.61, 0.52	-0.61, 0.52
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	0.446	14.455	-5.246	4.605	3.218
	Median	7.000	13.177	-7.729	10.584	10.584
	SD (SE)	21.5083 (10.7542)	5.3679 (2.6840)	39.9426 (19.9713)	28.4077 (10.0436)	25.3752 (7.3252)
	Min, Max	-30.20, 17.99	9.63, 21.84	-49.59, 44.07	-49.59, 44.07	-49.59, 44.07

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 120	n	4	4	4	8	12
	Mean	1.418	1.833	1.435	1.634	1.562
	Median	1.520	1.915	1.635	1.680	1.635
	SD (SE)	0.3384 (0.1692)	0.3167 (0.1583)	0.4384 (0.2192)	0.4129 (0.1460)	0.3887 (0.1122)
	Min, Max	0.93, 1.70	1.39, 2.11	0.78, 1.69	0.78, 2.11	0.78, 2.11
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-0.098	0.198	0.113	0.155	0.071
	Median	0.035	0.190	0.205	0.190	0.090
	SD (SE)	0.3120 (0.1560)	0.1500 (0.0750)	0.4156 (0.2078)	0.2928 (0.1035)	0.3107 (0.0897)
	Min, Max	-0.56, 0.10	0.04, 0.37	-0.45, 0.49	-0.45, 0.49	-0.56, 0.49
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-6.403	11.839	8.739	10.289	4.725
	Median	2.585	11.564	15.008	11.564	5.788
	SD (SE)	21.0422 (10.5211)	8.8271 (4.4136)	33.9351 (16.9676)	23.0148 (8.1369)	22.9208 (6.6167)
	Min, Max	-37.58, 6.80	2.96, 21.26	-36.59, 41.53	-36.59, 41.53	-37.58, 41.53

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 144	n	4	4	4	8	12
	Mean	1.440	1.995	1.300	1.648	1.578
	Median	1.545	2.060	1.430	1.530	1.540
	SD (SE)	0.3831 (0.1915)	0.3765 (0.1883)	0.3409 (0.1704)	0.4986 (0.1763)	0.4568 (0.1319)
	Min, Max	0.89, 1.78	1.52, 2.34	0.80, 1.54	0.80, 2.34	0.80, 2.34
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-0.075	0.360	-0.023	0.169	0.088
	Median	0.075	0.335	0.015	0.255	0.160
	SD (SE)	0.3518 (0.1759)	0.1791 (0.0895)	0.3405 (0.1703)	0.3244 (0.1147)	0.3393 (0.0979)
	Min, Max	-0.60, 0.15	0.17, 0.60	-0.43, 0.31	-0.43, 0.60	-0.60, 0.60
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-4.985	21.499	-1.200	10.149	5.104
	Median	4.768	19.460	1.943	16.986	11.692
	SD (SE)	23.6992 (11.8496)	9.2477 (4.6238)	27.3934 (13.6967)	22.4825 (7.9488)	23.0297 (6.6481)
	Min, Max	-40.27, 10.79	12.59, 34.48	-34.96, 26.27	-34.96, 34.48	-40.27, 34.48

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1 (L)						
Week 168	n	4	4	4	8	12
	Mean	1.465	1.890	1.445	1.668	1.600
	Median	1.455	1.880	1.455	1.655	1.550
	SD (SE)	0.1015 (0.0507)	0.3253 (0.1626)	0.2445 (0.1222)	0.3571 (0.1263)	0.3064 (0.0885)
	Min, Max	1.36, 1.59	1.51, 2.29	1.20, 1.67	1.20, 2.29	1.20, 2.29
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-0.050	0.255	0.123	0.189	0.109
	Median	-0.050	0.200	0.170	0.200	0.055
	SD (SE)	0.0868 (0.0434)	0.2086 (0.1043)	0.3593 (0.1796)	0.2810 (0.0994)	0.2572 (0.0742)
	Min, Max	-0.13, 0.03	0.07, 0.55	-0.34, 0.49	-0.34, 0.55	-0.34, 0.55
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-3.066	15.637	11.272	13.455	7.948
	Median	-2.789	13.618	12.820	13.618	3.478
	SD (SE)	5.5979 (2.7990)	11.7176 (5.8588)	27.1746 (13.5873)	19.5133 (6.8990)	17.8050 (5.1399)
	Min, Max	-8.72, 2.04	3.70, 31.61	-22.08, 41.53	-22.08, 41.53	-22.08, 41.53

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Day 1	n	4	4	4	8	12
	Mean	0.85	0.88	0.90	0.89	0.88
	Median	0.90	0.90	0.95	0.90	0.90
	SD (SE)	0.100 (0.050)	0.050 (0.025)	0.141 (0.071)	0.099 (0.035)	0.097 (0.028)
	Min, Max	0.7, 0.9	0.8, 0.9	0.7, 1.0	0.7, 1.0	0.7, 1.0
Baseline[1]	n	4	4	4	8	12
	Mean	0.85	0.88	0.90	0.89	0.88
	Median	0.90	0.90	0.95	0.90	0.90
	SD (SE)	0.100 (0.050)	0.050 (0.025)	0.141 (0.071)	0.099 (0.035)	0.097 (0.028)
	Min, Max	0.7, 0.9	0.8, 0.9	0.7, 1.0	0.7, 1.0	0.7, 1.0

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 12	n	4	4	4	8	12
	Mean	0.75	0.88	0.93	0.90	0.85
	Median	0.70	0.90	0.95	0.90	0.90
	SD (SE)	0.100 (0.050)	0.126 (0.063)	0.096 (0.048)	0.107 (0.038)	0.124 (0.036)
	Min, Max	0.7, 0.9	0.7, 1.0	0.8, 1.0	0.7, 1.0	0.7, 1.0
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-0.10	0.00	0.03	0.01	-0.03
	Median	-0.10	0.00	0.00	0.00	0.00
	SD (SE)	0.115 (0.058)	0.163 (0.082)	0.126 (0.063)	0.136 (0.048)	0.136 (0.039)
	Min, Max	-0.2, 0.0	-0.2, 0.2	-0.1, 0.2	-0.2, 0.2	-0.2, 0.2
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-11.11	0.69	4.37	2.53	-2.02
	Median	-11.11	0.00	0.00	0.00	0.00
	SD (SE)	12.830 (6.415)	19.295 (9.648)	16.966 (8.483)	16.934 (5.987)	16.507 (4.765)
	Min, Max	-22.2, 0.0	-22.2, 25.0	-11.1, 28.6	-22.2, 28.6	-22.2, 28.6

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 24	n	4	4	4	8	12
	Mean	0.83	0.95	0.88	0.91	0.88
	Median	0.95	1.00	0.85	0.95	0.95
	SD (SE)	0.287 (0.144)	0.100 (0.050)	0.096 (0.048)	0.099 (0.035)	0.175 (0.051)
	Min, Max	0.4, 1.0	0.8, 1.0	0.8, 1.0	0.8, 1.0	0.4, 1.0
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-0.03	0.08	-0.03	0.03	0.01
	Median	0.05	0.10	-0.05	0.05	0.05
	SD (SE)	0.189 (0.095)	0.050 (0.025)	0.096 (0.048)	0.089 (0.031)	0.124 (0.036)
	Min, Max	-0.3, 0.1	0.0, 0.1	-0.1, 0.1	-0.1, 0.1	-0.3, 0.1
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-5.16	8.33	-1.71	3.31	0.49
	Median	5.56	11.11	-5.00	5.56	5.56
	SD (SE)	25.672 (12.836)	5.556 (2.778)	11.774 (5.887)	10.072 (3.561)	16.177 (4.670)
	Min, Max	-42.9, 11.1	0.0, 11.1	-11.1, 14.3	-11.1, 14.3	-42.9, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 36	n	4	4	4	8	12
	Mean	0.80	0.88	0.88	0.88	0.85
	Median	0.80	0.90	0.85	0.90	0.90
	SD (SE)	0.115 (0.058)	0.050 (0.025)	0.096 (0.048)	0.071 (0.025)	0.090 (0.026)
	Min, Max	0.7, 0.9	0.8, 0.9	0.8, 1.0	0.8, 1.0	0.7, 1.0
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-0.05	0.00	-0.03	-0.01	-0.03
	Median	0.00	0.00	-0.05	0.00	0.00
	SD (SE)	0.100 (0.050)	0.000 (0.000)	0.096 (0.048)	0.064 (0.023)	0.075 (0.022)
	Min, Max	-0.2, 0.0	0.0, 0.0	-0.1, 0.1	-0.1, 0.1	-0.2, 0.1
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-5.56	0.00	-1.71	-0.85	-2.42
	Median	0.00	0.00	-5.00	0.00	0.00
	SD (SE)	11.111 (5.556)	0.000 (0.000)	11.774 (5.887)	7.762 (2.744)	8.796 (2.539)
	Min, Max	-22.2, 0.0	0.0, 0.0	-11.1, 14.3	-11.1, 14.3	-22.2, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 48	n	4	4	4	8	12
	Mean	0.85	0.88	0.83	0.85	0.85
	Median	0.85	0.90	0.85	0.90	0.90
	SD (SE)	0.058 (0.029)	0.050 (0.025)	0.171 (0.085)	0.120 (0.042)	0.100 (0.029)
	Min, Max	0.8, 0.9	0.8, 0.9	0.6, 1.0	0.6, 1.0	0.6, 1.0
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	0.00	0.00	-0.08	-0.04	-0.03
	Median	0.00	0.00	-0.05	0.00	0.00
	SD (SE)	0.082 (0.041)	0.000 (0.000)	0.171 (0.085)	0.119 (0.042)	0.106 (0.030)
	Min, Max	-0.1, 0.1	0.0, 0.0	-0.3, 0.1	-0.3, 0.1	-0.3, 0.1
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	0.79	0.00	-7.26	-3.63	-2.16
	Median	0.00	0.00	-5.00	0.00	0.00
	SD (SE)	10.409 (5.204)	0.000 (0.000)	20.035 (10.018)	13.679 (4.836)	12.384 (3.575)
	Min, Max	-11.1, 14.3	0.0, 0.0	-33.3, 14.3	-33.3, 14.3	-33.3, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 62	n	4	4	4	8	12
	Mean	0.85	0.85	0.88	0.86	0.86
	Median	0.90	0.85	0.90	0.90	0.90
	SD (SE)	0.100 (0.050)	0.058 (0.029)	0.050 (0.025)	0.052 (0.018)	0.067 (0.019)
	Min, Max	0.7, 0.9	0.8, 0.9	0.8, 0.9	0.8, 0.9	0.7, 0.9
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	0.00	-0.03	-0.03	-0.03	-0.02
	Median	0.00	0.00	-0.05	0.00	0.00
	SD (SE)	0.000 (0.000)	0.050 (0.025)	0.096 (0.048)	0.071 (0.025)	0.058 (0.017)
	Min, Max	0.0, 0.0	-0.1, 0.0	-0.1, 0.1	-0.1, 0.1	-0.1, 0.1
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	0.00	-2.78	-1.43	-2.10	-1.40
	Median	0.00	0.00	-5.00	0.00	0.00
	SD (SE)	0.000 (0.000)	5.556 (2.778)	11.488 (5.744)	8.385 (2.965)	6.769 (1.954)
	Min, Max	0.0, 0.0	-11.1, 0.0	-10.0, 14.3	-11.1, 14.3	-11.1, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 74	n	4	4	4	8	12
	Mean	0.83	0.93	0.88	0.90	0.88
	Median	0.85	0.95	0.85	0.90	0.90
	SD (SE)	0.206 (0.103)	0.096 (0.048)	0.096 (0.048)	0.093 (0.033)	0.136 (0.039)
	Min, Max	0.6, 1.0	0.8, 1.0	0.8, 1.0	0.8, 1.0	0.6, 1.0
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-0.03	0.05	-0.03	0.01	0.00
	Median	0.05	0.05	-0.05	0.00	0.00
	SD (SE)	0.189 (0.095)	0.058 (0.029)	0.096 (0.048)	0.083 (0.030)	0.121 (0.035)
	Min, Max	-0.3, 0.1	0.0, 0.1	-0.1, 0.1	-0.1, 0.1	-0.3, 0.1
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-2.78	5.56	-1.71	1.92	0.36
	Median	5.56	5.56	-5.00	0.00	0.00
	SD (SE)	21.033 (10.516)	6.415 (3.208)	11.774 (5.887)	9.598 (3.393)	13.588 (3.922)
	Min, Max	-33.3, 11.1	0.0, 11.1	-11.1, 14.3	-11.1, 14.3	-33.3, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 84	n	4	4	4	8	12
	Mean	1.00	1.00	0.90	0.95	0.97
	Median	1.00	1.00	0.90	0.95	1.00
	SD (SE)	0.000 (0.000)	0.000 (0.000)	0.000 (0.000)	0.053 (0.019)	0.049 (0.014)
	Min, Max	1.0, 1.0	1.0, 1.0	0.9, 0.9	0.9, 1.0	0.9, 1.0
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	0.15	0.13	0.00	0.06	0.09
	Median	0.10	0.10	-0.05	0.10	0.10
	SD (SE)	0.100 (0.050)	0.050 (0.025)	0.141 (0.071)	0.119 (0.042)	0.116 (0.034)
	Min, Max	0.1, 0.3	0.1, 0.2	-0.1, 0.2	-0.1, 0.2	-0.1, 0.3
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	19.05	14.58	2.14	8.36	11.92
	Median	11.11	11.11	-5.00	11.11	11.11
	SD (SE)	15.873 (7.937)	6.944 (3.472)	18.239 (9.119)	14.403 (5.092)	15.113 (4.363)
	Min, Max	11.1, 42.9	11.1, 25.0	-10.0, 28.6	-10.0, 28.6	-10.0, 42.9

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 96	n	4	4	4	8	12
	Mean	0.83	0.85	0.83	0.84	0.83
	Median	0.85	0.85	0.80	0.80	0.80
	SD (SE)	0.096 (0.048)	0.058 (0.029)	0.126 (0.063)	0.092 (0.032)	0.089 (0.026)
	Min, Max	0.7, 0.9	0.8, 0.9	0.7, 1.0	0.7, 1.0	0.7, 1.0
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-0.03	-0.03	-0.08	-0.05	-0.04
	Median	0.00	0.00	-0.10	0.00	0.00
	SD (SE)	0.126 (0.063)	0.050 (0.025)	0.150 (0.075)	0.107 (0.038)	0.108 (0.031)
	Min, Max	-0.2, 0.1	-0.1, 0.0	-0.2, 0.1	-0.2, 0.1	-0.2, 0.1
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-1.98	-2.78	-6.98	-4.88	-3.92
	Median	0.00	0.00	-10.00	0.00	0.00
	SD (SE)	15.079 (7.540)	5.556 (2.778)	17.347 (8.674)	12.135 (4.290)	12.560 (3.626)
	Min, Max	-22.2, 14.3	-11.1, 0.0	-22.2, 14.3	-22.2, 14.3	-22.2, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 120	n	4	4	4	8	12
	Mean	0.85	0.85	0.75	0.80	0.82
	Median	0.85	0.85	0.80	0.80	0.80
	SD (SE)	0.058 (0.029)	0.058 (0.029)	0.173 (0.087)	0.131 (0.046)	0.111 (0.032)
	Min, Max	0.8, 0.9	0.8, 0.9	0.5, 0.9	0.5, 0.9	0.5, 0.9
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	0.00	-0.03	-0.15	-0.09	-0.06
	Median	-0.05	0.00	-0.10	-0.05	-0.05
	SD (SE)	0.141 (0.071)	0.050 (0.025)	0.252 (0.126)	0.181 (0.064)	0.168 (0.048)
	Min, Max	-0.1, 0.2	-0.1, 0.0	-0.5, 0.1	-0.5, 0.1	-0.5, 0.2
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	1.59	-2.78	-14.21	-8.49	-5.13
	Median	-5.56	0.00	-10.56	-5.00	-5.00
	SD (SE)	18.736 (9.368)	5.556 (2.778)	26.585 (13.292)	18.800 (6.647)	18.582 (5.364)
	Min, Max	-11.1, 28.6	-11.1, 0.0	-50.0, 14.3	-50.0, 14.3	-50.0, 28.6

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 144	n	4	4	4	8	12
	Mean	0.78	0.88	0.80	0.84	0.82
	Median	0.80	0.90	0.80	0.90	0.85
	SD (SE)	0.126 (0.063)	0.050 (0.025)	0.115 (0.058)	0.092 (0.032)	0.103 (0.030)
	Min, Max	0.6, 0.9	0.8, 0.9	0.7, 0.9	0.7, 0.9	0.6, 0.9
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-0.08	0.00	-0.10	-0.05	-0.06
	Median	-0.05	0.00	-0.10	0.00	0.00
	SD (SE)	0.171 (0.085)	0.000 (0.000)	0.082 (0.041)	0.076 (0.027)	0.108 (0.031)
	Min, Max	-0.3, 0.1	0.0, 0.0	-0.2, 0.0	-0.2, 0.0	-0.3, 0.1
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-7.54	0.00	-10.56	-5.28	-6.03
	Median	-5.56	0.00	-10.00	0.00	0.00
	SD (SE)	20.094 (10.047)	0.000 (0.000)	9.095 (4.547)	8.203 (2.900)	12.417 (3.584)
	Min, Max	-33.3, 14.3	0.0, 0.0	-22.2, 0.0	-22.2, 0.0	-33.3, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FEV1/FVC Ratio						
Week 168	n	4	4	4	8	12
	Mean	0.80	0.85	0.80	0.83	0.82
	Median	0.80	0.85	0.85	0.85	0.80
	SD (SE)	0.082 (0.041)	0.058 (0.029)	0.141 (0.071)	0.104 (0.037)	0.094 (0.027)
	Min, Max	0.7, 0.9	0.8, 0.9	0.6, 0.9	0.6, 0.9	0.6, 0.9
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-0.05	-0.03	-0.10	-0.06	-0.06
	Median	-0.05	0.00	-0.10	-0.05	-0.05
	SD (SE)	0.129 (0.065)	0.050 (0.025)	0.163 (0.082)	0.119 (0.042)	0.116 (0.034)
	Min, Max	-0.2, 0.1	-0.1, 0.0	-0.3, 0.1	-0.3, 0.1	-0.3, 0.1
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-4.76	-2.78	-9.76	-6.27	-5.77
	Median	-5.56	0.00	-10.00	-5.00	-5.00
	SD (SE)	15.606 (7.803)	5.556 (2.778)	19.442 (9.721)	13.754 (4.863)	13.688 (3.951)
	Min, Max	-22.2, 14.3	-11.1, 0.0	-33.3, 14.3	-33.3, 14.3	-33.3, 14.3

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen: 30 mg/kg for 168 Weeks (N=4)	Eteplirsen: 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Day 1	n	4	4	4	8	12
	Mean	116.3	95.3	92.3	93.8	101.3
	Median	119.0	98.0	88.0	93.5	98.0
	SD (SE)	15.44 (7.72)	7.80 (3.90)	11.59 (5.79)	9.29 (3.28)	15.57 (4.50)
	Min, Max	96, 131	84, 101	84, 109	84, 109	84, 131
Baseline[1]	n	4	4	4	8	12
	Mean	116.3	95.3	92.3	93.8	101.3
	Median	119.0	98.0	88.0	93.5	98.0
	SD (SE)	15.44 (7.72)	7.80 (3.90)	11.59 (5.79)	9.29 (3.28)	15.57 (4.50)
	Min, Max	96, 131	84, 101	84, 109	84, 109	84, 131

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 12	n	4	4	4	8	12
	Mean	115.8	99.0	91.8	95.4	102.2
	Median	121.0	103.0	87.0	96.5	103.0
	SD (SE)	18.46 (9.23)	9.38 (4.69)	14.01 (7.00)	11.70 (4.14)	16.75 (4.84)
	Min, Max	90, 131	85, 105	81, 112	81, 112	81, 131
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-0.5	3.8	-0.5	1.6	0.9
	Median	-2.0	3.5	-0.5	1.5	1.0
	SD (SE)	14.36 (7.18)	12.26 (6.13)	2.89 (1.44)	8.55 (3.02)	10.19 (2.94)
	Min, Max	-16, 18	-11, 19	-4, 3	-11, 19	-16, 19
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-0.2	4.5	-0.8	1.9	1.2
	Median	-2.3	3.5	-0.5	1.4	0.8
	SD (SE)	12.17 (6.08)	13.97 (6.99)	3.09 (1.54)	9.78 (3.46)	10.12 (2.92)
	Min, Max	-12, 16	-11, 23	-5, 3	-11, 23	-12, 23

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 24	n	4	4	4	8	12
	Mean	105.5	83.8	85.8	84.8	91.7
	Median	106.5	84.0	84.5	84.5	85.5
	SD (SE)	19.87 (9.94)	2.63 (1.31)	4.35 (2.17)	3.49 (1.24)	14.83 (4.28)
	Min, Max	85, 124	81, 86	82, 92	81, 92	81, 124
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-10.8	-11.5	-6.5	-9.0	-9.6
	Median	-9.0	-14.0	-4.5	-8.0	-8.5
	SD (SE)	7.41 (3.71)	9.98 (4.99)	7.42 (3.71)	8.57 (3.03)	7.90 (2.28)
	Min, Max	-21, -4	-20, 2	-17, 0	-20, 2	-21, 2
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-9.6	-11.5	-6.4	-8.9	-9.2
	Median	-8.4	-14.2	-5.1	-8.5	-8.5
	SD (SE)	6.91 (3.45)	10.08 (5.04)	6.68 (3.34)	8.36 (2.96)	7.59 (2.19)
	Min, Max	-19, -3	-20, 2	-16, 0	-20, 2	-20, 2

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 36	n	4	4	4	8	12
	Mean	106.0	100.3	91.8	96.0	99.3
	Median	104.0	101.0	92.0	98.5	99.0
	SD (SE)	13.88 (6.94)	4.86 (2.43)	9.18 (4.59)	8.18 (2.89)	10.92 (3.15)
	Min, Max	92, 124	94, 105	82, 101	82, 105	82, 124
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-10.3	5.0	-0.5	2.3	-1.9
	Median	-4.0	3.5	-0.5	1.5	-1.5
	SD (SE)	14.57 (7.28)	7.26 (3.63)	8.66 (4.33)	7.96 (2.81)	11.67 (3.37)
	Min, Max	-32, -1	-2, 15	-11, 10	-11, 15	-32, 15
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-8.2	5.7	-0.1	2.8	-0.9
	Median	-3.9	3.5	-0.6	1.6	-1.4
	SD (SE)	10.89 (5.45)	8.62 (4.31)	8.75 (4.37)	8.61 (3.04)	10.44 (3.01)
	Min, Max	-24, -1	-2, 18	-10, 11	-10, 18	-24, 18

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 48	n	4	4	4	8	12
	Mean	101.5	92.5	95.3	93.9	96.4
	Median	108.0	92.0	94.0	94.0	95.5
	SD (SE)	24.73 (12.37)	5.92 (2.96)	6.34 (3.17)	5.87 (2.07)	14.24 (4.11)
	Min, Max	67, 123	87, 99	89, 104	87, 104	67, 123
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-14.8	-2.8	3.0	0.1	-4.8
	Median	-15.5	-3.0	4.5	1.0	-2.0
	SD (SE)	17.11 (8.56)	5.38 (2.69)	5.60 (2.80)	5.94 (2.10)	12.49 (3.61)
	Min, Max	-30, 2	-9, 4	-5, 8	-9, 8	-30, 8
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-13.2	-2.6	3.8	0.6	-4.0
	Median	-12.3	-3.0	5.2	1.2	-1.8
	SD (SE)	15.73 (7.86)	5.84 (2.92)	5.97 (2.98)	6.46 (2.28)	11.84 (3.42)
	Min, Max	-30, 2	-9, 5	-5, 9	-9, 9	-30, 9

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 62	n	4	4	4	8	12
	Mean	104.3	90.8	100.3	95.5	98.4
	Median	106.0	92.0	102.0	98.5	100.5
	SD (SE)	18.57 (9.29)	10.59 (5.30)	11.21 (5.60)	11.30 (4.00)	13.92 (4.02)
	Min, Max	81, 124	77, 102	85, 112	77, 112	77, 124
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-12.0	-4.5	8.0	1.8	-2.8
	Median	-8.0	-5.0	7.0	2.0	0.0
	SD (SE)	14.99 (7.49)	13.20 (6.60)	8.12 (4.06)	12.15 (4.30)	14.18 (4.09)
	Min, Max	-32, 0	-19, 11	0, 18	-19, 18	-32, 18
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-10.2	-4.2	9.1	2.4	-1.8
	Median	-8.2	-5.0	7.4	1.9	0.0
	SD (SE)	11.89 (5.95)	14.32 (7.16)	9.73 (4.87)	13.36 (4.72)	13.82 (3.99)
	Min, Max	-24, 0	-20, 13	0, 21	-20, 21	-24, 21

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 74	n	4	4	4	8	12
	Mean	96.3	85.0	82.0	83.5	87.8
	Median	98.5	85.0	82.0	82.5	85.5
	SD (SE)	8.06 (4.03)	4.76 (2.38)	3.37 (1.68)	4.14 (1.46)	8.25 (2.38)
	Min, Max	85, 103	80, 90	78, 86	78, 90	78, 103
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-20.0	-10.3	-10.3	-10.3	-13.5
	Median	-19.5	-9.0	-8.0	-9.0	-12.5
	SD (SE)	8.04 (4.02)	8.26 (4.13)	9.84 (4.92)	8.41 (2.97)	9.26 (2.67)
	Min, Max	-30, -11	-21, -2	-23, -2	-23, -2	-30, -2
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-16.8	-10.4	-10.3	-10.3	-12.5
	Median	-16.3	-9.1	-8.9	-9.1	-13.1
	SD (SE)	4.81 (2.41)	8.00 (4.00)	8.96 (4.48)	7.87 (2.78)	7.46 (2.15)
	Min, Max	-23, -11	-21, -2	-21, -2	-21, -2	-23, -2

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 84	n	4	4	4	8	12
	Mean	79.0	76.8	75.3	76.0	77.0
	Median	85.5	76.0	79.5	76.0	80.5
	SD (SE)	22.32 (11.16)	4.50 (2.25)	29.09 (14.55)	19.29 (6.82)	19.36 (5.59)
	Min, Max	47, 98	73, 82	40, 102	40, 102	40, 102
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-37.3	-18.5	-17.0	-17.8	-24.3
	Median	-38.0	-22.5	-14.5	-22.0	-23.5
	SD (SE)	13.62 (6.81)	11.21 (5.61)	21.12 (10.56)	15.67 (5.54)	17.29 (4.99)
	Min, Max	-49, -24	-27, -2	-44, 5	-44, 5	-49, 5
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-32.8	-18.8	-19.8	-19.3	-23.8
	Median	-29.5	-22.9	-16.2	-22.9	-22.9
	SD (SE)	14.30 (7.15)	11.14 (5.57)	25.28 (12.64)	18.09 (6.40)	17.56 (5.07)
	Min, Max	-51, -21	-27, -2	-52, 5	-52, 5	-52, 5

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 96	n	4	4	4	8	12
	Mean	110.3	94.5	81.5	88.0	95.4
	Median	119.0	94.5	82.0	90.0	94.5
	SD (SE)	21.75 (10.87)	5.69 (2.84)	23.74 (11.87)	17.43 (6.16)	21.03 (6.07)
	Min, Max	78, 125	88, 101	52, 110	52, 110	52, 125
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-6.0	-0.8	-10.8	-5.8	-5.8
	Median	-5.0	-2.0	-6.0	-2.5	-4.0
	SD (SE)	9.09 (4.55)	10.44 (5.22)	15.11 (7.55)	13.16 (4.65)	11.52 (3.33)
	Min, Max	-18, 4	-12, 13	-32, 1	-32, 13	-32, 13
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-5.7	-0.2	-12.6	-6.4	-6.2
	Median	-3.9	-2.1	-6.6	-2.7	-3.7
	SD (SE)	9.37 (4.68)	11.56 (5.78)	17.92 (8.96)	15.46 (5.47)	13.27 (3.83)
	Min, Max	-19, 4	-12, 15	-38, 1	-38, 15	-38, 15

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 120	n	4	4	4	8	12
	Mean	97.8	85.8	96.0	90.9	93.2
	Median	108.0	86.5	91.5	91.5	95.0
	SD (SE)	27.15 (13.57)	13.18 (6.59)	13.09 (6.54)	13.34 (4.71)	18.04 (5.21)
	Min, Max	58, 117	72, 98	86, 115	72, 115	58, 117
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-18.5	-9.5	3.8	-2.9	-8.1
	Median	-18.0	-11.0	4.0	2.0	-1.5
	SD (SE)	17.54 (8.77)	17.67 (8.84)	2.22 (1.11)	13.64 (4.82)	16.17 (4.67)
	Min, Max	-38, 0	-28, 12	1, 6	-28, 12	-38, 12
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-16.8	-9.1	4.0	-2.6	-7.3
	Median	-13.9	-11.4	4.4	2.2	-1.5
	SD (SE)	17.61 (8.81)	18.76 (9.38)	2.20 (1.10)	14.21 (5.02)	16.20 (4.68)
	Min, Max	-40, 0	-28, 14	1, 6	-28, 14	-40, 14

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 144	n	4	4	4	8	12
	Mean	104.8	87.8	80.3	84.0	90.9
	Median	110.5	86.5	83.0	83.0	91.5
	SD (SE)	18.61 (9.30)	17.95 (8.98)	21.88 (10.94)	18.96 (6.70)	20.68 (5.97)
	Min, Max	78, 120	71, 107	51, 104	51, 107	51, 120
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-11.5	-7.5	-12.0	-9.8	-10.3
	Median	-11.5	-8.0	-6.0	-6.0	-6.0
	SD (SE)	11.50 (5.75)	21.30 (10.65)	14.09 (7.05)	16.89 (5.97)	14.78 (4.27)
	Min, Max	-24, 1	-29, 15	-33, -3	-33, 15	-33, 15
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	-10.0	-7.0	-13.8	-10.4	-10.3
	Median	-11.2	-8.5	-6.1	-6.1	-6.1
	SD (SE)	10.00 (5.00)	22.53 (11.26)	17.10 (8.55)	18.86 (6.67)	15.93 (4.60)
	Min, Max	-19, 1	-29, 18	-39, -4	-39, 18	-39, 18

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC % of Predicted Value (%)						
Week 168	n	4	4	4	8	12
	Mean	102.5	85.3	88.0	86.6	91.9
	Median	107.0	87.5	83.0	83.5	90.0
	SD (SE)	12.97 (6.49)	13.60 (6.80)	14.49 (7.25)	13.09 (4.63)	14.70 (4.24)
	Min, Max	84, 112	68, 98	77, 109	68, 109	68, 112
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-13.8	-10.0	-4.3	-7.1	-9.3
	Median	-12.5	-9.0	-3.5	-5.0	-9.0
	SD (SE)	10.72 (5.36)	17.87 (8.93)	5.74 (2.87)	12.67 (4.48)	12.00 (3.46)
	Min, Max	-28, -2	-32, 10	-11, 1	-32, 10	-32, 10
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	-11.5	-9.7	-4.8	-7.2	-8.7
	Median	-11.5	-9.3	-4.2	-5.7	-9.4
	SD (SE)	8.05 (4.02)	18.66 (9.33)	6.44 (3.22)	13.18 (4.66)	11.52 (3.32)
	Min, Max	-21, -2	-32, 12	-12, 1	-32, 12	-32, 12

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Day 1	n	4	4	4	8	12
	Mean	1.795	1.888	1.503	1.695	1.728
	Median	1.785	1.925	1.500	1.650	1.660
	SD (SE)	0.1940 (0.0970)	0.3433 (0.1716)	0.2484 (0.1242)	0.3454 (0.1221)	0.2977 (0.0859)
	Min, Max	1.61, 2.00	1.52, 2.18	1.23, 1.78	1.23, 2.18	1.23, 2.18
Baseline[1]	n	4	4	4	8	12
	Mean	1.795	1.888	1.503	1.695	1.728
	Median	1.785	1.925	1.500	1.650	1.660
	SD (SE)	0.1940 (0.0970)	0.3433 (0.1716)	0.2484 (0.1242)	0.3454 (0.1221)	0.2977 (0.0859)
	Min, Max	1.61, 2.00	1.52, 2.18	1.23, 1.78	1.23, 2.18	1.23, 2.18

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 12	n	4	4	4	8	12
	Mean	1.800	1.948	1.508	1.728	1.752
	Median	1.845	1.975	1.540	1.710	1.765
	SD (SE)	0.1623 (0.0811)	0.2830 (0.1415)	0.2419 (0.1209)	0.3387 (0.1197)	0.2854 (0.0824)
	Min, Max	1.57, 1.94	1.58, 2.26	1.23, 1.72	1.23, 2.26	1.23, 2.26
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	0.005	0.060	0.005	0.033	0.023
	Median	-0.030	0.070	0.005	0.035	0.015
	SD (SE)	0.1964 (0.0982)	0.2535 (0.1268)	0.0695 (0.0348)	0.1746 (0.0617)	0.1735 (0.0501)
	Min, Max	-0.19, 0.27	-0.26, 0.36	-0.08, 0.09	-0.26, 0.36	-0.26, 0.36
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	0.866	4.312	0.439	2.376	1.872
	Median	-1.903	3.809	0.365	2.200	0.886
	SD (SE)	11.4467 (5.7234)	13.6824 (6.8412)	4.1007 (2.0503)	9.5772 (3.3861)	9.7292 (2.8086)
	Min, Max	-9.50, 16.77	-11.93, 21.56	-4.49, 5.52	-11.93, 21.56	-11.93, 21.56

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2.8.1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 24	n	4	4	4	8	12
	Mean	1.590	1.728	1.453	1.590	1.590
	Median	1.540	1.770	1.420	1.595	1.540
	SD (SE)	0.2522 (0.1261)	0.3917 (0.1958)	0.2370 (0.1185)	0.3338 (0.1180)	0.2971 (0.0858)
	Min, Max	1.34, 1.94	1.21, 2.16	1.20, 1.77	1.20, 2.16	1.20, 2.16
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-0.205	-0.160	-0.050	-0.105	-0.138
	Median	-0.200	-0.165	-0.020	-0.025	-0.095
	SD (SE)	0.1353 (0.0676)	0.2186 (0.1093)	0.0891 (0.0445)	0.1654 (0.0585)	0.1575 (0.0455)
	Min, Max	-0.36, -0.06	-0.38, 0.07	-0.18, 0.02	-0.38, 0.07	-0.38, 0.07
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-11.600	-8.638	-3.146	-5.892	-7.795
	Median	-12.324	-9.174	-1.500	-1.678	-5.439
	SD (SE)	7.4310 (3.7155)	12.1071 (6.0536)	5.5001 (2.7501)	9.1871 (3.2481)	8.7561 (2.5277)
	Min, Max	-18.75, -3.00	-20.39, 4.19	-11.04, 1.46	-20.39, 4.19	-20.39, 4.19

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run: 06MAR2015:14:00
Reference listing: L.16.2.6.8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 36	n	4	4	4	8	12
	Mean	1 688	2 075	1 575	1 825	1 779
	Median	1 635	2 165	1 540	1 800	1 635
	SD (SE)	0 1441 (0 0720)	0 3535 (0 1768)	0 3420 (0 1710)	0 4185 (0 1480)	0 3488 (0 1007)
	Min, Max	1 58, 1 90	1 61, 2 36	1.20, 2 02	1 20, 2 36	1 20, 2 36
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-0 108	0 188	0 073	0 130	0 051
	Median	-0 015	0 170	0 040	0 130	0 055
	SD (SE)	0 2090 (0 1045)	0 0964 (0 0482)	0 1263 (0 0632)	0 1208 (0 0427)	0 1868 (0 0539)
	Min, Max	-0 42, 0 02	0 09, 0 32	-0 03, 0 24	-0.03, 0 32	-0 42, 0 32
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-5 351	10 170	4.279	7 224	3 032
	Median	-0 824	7 798	3 036	7 319	3 582
	SD (SE)	10 4793 (5 2396)	6 0711 (3 0356)	7 5119 (3 7560)	7 0636 (2 4974)	10 0020 (2 8873)
	Min, Max	-21 00, 1 24	5 92, 19 16	-2 44, 13 48	-2 44, 19 16	-21 00, 19 16

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015·14 00
Reference listing L 16 2 6 8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 48	n	4	4	4	8	12
	Mean	1 603	2 033	1 658	1 845	1 764
	Median	1 665	2 070	1 630	1 775	1 715
	SD (SE)	0 2903	0 4113	0 2988	0 3885	0 3651
		(0 1452)	(0 2056)	(0 1494)	(0 1374)	(0 1054)
	Min, Max	1 20, 1 88	1 55, 2 44	1 33, 2 04	1 33, 2 44	1 20, 2 44
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-0 193	0 145	0 155	0 150	0 036
	Median	-0 215	0 145	0 140	0 145	0 105
	SD (SE)	0 2708	0 0961	0 0823	0 0830	0 2298
		(0 1354)	(0 0480)	(0 0411)	(0 0293)	(0 0663)
	Min, Max	-0 45, 0 11	0 03, 0 26	0 08, 0 26	0 03, 0 26	-0 45, 0 26
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-10 506	7 396	10 196	8 796	2 362
	Median	-10.792	7 842	10 634	9 155	6.168
	SD (SE)	15 6373	4 5186	4 4850	4 4284	13 0187
		(7 8186)	(2 2593)	(2 2425)	(1 5657)	(3 7582)
	Min, Max	-27 27, 6 83	1 97, 11 93	4 91, 14.61	1 97, 14 61	-27 27, 14 61

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 62	n	4	4	4	8	12
	Mean	1 683	2 018	1 780	1 899	1 827
	Median	1 680	2 020	1 795	1 900	1 795
	SD (SE)	0 1723	0 3424	0 1722	0 2812	0 2641
		(0 0862)	(0 1712)	(0 0861)	(0 0994)	(0 0762)
	Min, Max	1 48, 1 89	1 60, 2 43	1 56, 1 97	1 56, 2 43	1 48, 2 43
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-0 113	0 130	0 278	0 204	0 098
	Median	-0 100	0 165	0 270	0 230	0 155
	SD (SE)	0 2085	0 1783	0 0922	0 1532	0 2259
		(0 1043)	(0 0892)	(0 0461)	(0 0542)	(0 0652)
	Min, Max	-0 37, 0 12	-0 10, 0 29	0 19, 0 38	-0 10, 0 38	-0 37, 0 38
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-5 728	7 377	19 531	13 454	7 060
	Median	-5 933	8 366	19 856	12 176	9 064
	SD (SE)	11 1827	9 3828	9 0045	10 7090	14 0102
		(5 5913)	(4 6914)	(4 5022)	(3 7862)	(4 0444)
	Min, Max	-18 50, 7 45	-4 59, 17 37	10 67, 27.74	-4 59, 27 74	-18 50, 27 74

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 74	n	4	4	4	8	12
	Mean	1 598	2 000	1 505	1 753	1 701
	Median	1 595	2 085	1 400	1 545	1 595
	SD (SE)	0 0665 (0 0333)	0 6316 (0 3158)	0 2817 (0 1409)	0 5244 (0 1854)	0 4266 (0 1232)
	Min, Max	1 54, 1 66	1 28, 2 55	1 30, 1 92	1 28, 2 55	1 28, 2 55
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-0 198	0 113	0 003	0 058	-0 028
	Median	-0 190	0 160	0 035	0 035	-0 040
	SD (SE)	0 1284 (0 0642)	0 2903 (0 1452)	0 1466 (0 0733)	0 2209 (0 0781)	0 2265 (0 0654)
	Min, Max	-0 34, -0 07	-0 24, 0 37	-0 20, 0 14	-0 24, 0 37	-0 34, 0 37
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-10 519	3 930	0 322	2 126	-2 089
	Median	-10 365	7 269	2 846	2 846	-2 473
	SD (SE)	5 9856 (2 9928)	15 3306 (7 6653)	9 0256 (4 5128)	11 8051 (4 1737)	11 7141 (3 3816)
	Min, Max	-17 00, -4 35	-15 79, 16 97	-12 27, 7 87	-15 79, 16 97	-17 00, 16 97

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 84	n	4	4	4	8	12
	Mean	1 320	1 735	1 393	1 564	1 483
	Median	1 415	1 840	1 610	1 690	1 545
	SD (SE)	0 3045	0 3508	0 5092	0 4443	0 4066
		(0 1523)	(0 1754)	(0 2546)	(0 1571)	(0 1174)
	Min, Max	0 88, 1 57	1 26, 2 00	0 64, 1 71	0 64, 2 00	0 64, 2 00
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-0 475	-0 153	-0 110	-0 131	-0 246
	Median	-0 490	-0 180	-0 095	-0 180	-0 220
	SD (SE)	0 2783	0 1147	0 4032	0 2754	0 3131
		(0 1391)	(0 0574)	(0 2016)	(0 0974)	(0 0904)
	Min, Max	-0 77, -0 15	-0 26, 0 01	-0 59, 0 34	-0 59, 0 34	-0 77, 0 34
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-26 428	-8 255	-8 366	-8 310	-14 350
	Median	-24 865	-8 257	-5 156	-8 257	-11 962
	SD (SE)	16 2823	7 2277	30 9228	20 7894	20 6620
		(8 1412)	(3 6138)	(15 4614)	(7 3502)	(5 9646)
	Min, Max	-46 67, -9 32	-17 11, 0 60	-47 97, 24 82	-47 97, 24 82	-47 97, 24 82

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 96	n	4	4	4	8	12
	Mean	1 845	2 200	1 545	1 873	1 863
	Median	1 925	2 250	1 645	1 910	1 925
	SD (SE)	0 2769 (0 1385)	0 4855 (0 2427)	0 5446 (0 2723)	0 5922 (0 2094)	0 4942 (0 1427)
	Min, Max	1 45, 2 08	1 62, 2 68	0 82, 2 07	0 82, 2 68	0 82, 2 68
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	0 050	0 313	0 043	0 178	0 135
	Median	0 070	0 325	0 145	0 245	0 150
	SD (SE)	0 1894 (0 0947)	0 1640 (0 0820)	0 3126 (0 1563)	0 2724 (0 0963)	0 2469 (0 0713)
	Min, Max	-0 20, 0 26	0 10, 0 50	-0 41, 0.29	-0 41, 0 50	-0 41, 0 50
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	2 788	15 954	0 450	8 202	6 397
	Median	3 563	17 150	9 420	13 704	9 424
	SD (SE)	11 5814 (5 7907)	7 0141 (3 5070)	22.8724 (11 4362)	17 7192 (6 2647)	15 6039 (4 5045)
	Min, Max	-12 12, 16 15	6 58, 22 94	-33 33, 16 29	-33 33, 22 94	-33 33, 22 94

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 120	n	4	4	4	8	12
	Mean	1 660	2 125	1 845	1 985	1 877
	Median	1 805	2 185	1 875	1 975	1 885
	SD (SE)	0 3725 (0 1862)	0 4429 (0 2215)	0 2864 (0 1432)	0 3763 (0 1331)	0 3919 (0 1131)
	Min, Max	1 11, 1 92	1 57, 2 56	1 48, 2 15	1 48, 2 56	1 11, 2 56
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-0 135	0 238	0 343	0 290	0 148
	Median	-0 120	0 260	0 355	0 325	0 245
	SD (SE)	0 3336 (0 1668)	0 1431 (0 0716)	0 0680 (0 0340)	0 1180 (0 0417)	0 2881 (0 0832)
	Min, Max	-0 54, 0 24	0 05, 0 38	0 25, 0 41	0 05, 0 41	-0 54, 0 41
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	-7 455	12 229	22 974	17 602	9 249
	Median	-6 000	13 532	20 823	19 444	16 169
	SD (SE)	20 1244 (10 0622)	7 1609 (3 5804)	4 6411 (2 3205)	8 0123 (2 8328)	17 4215 (5 0292)
	Min, Max	-32 73, 14 91	3 29, 18 56	20 33, 29 93	3 29, 29 93	-32 73, 29 93

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 144	n	4	4	4	8	12
	Mean	1 815	2 295	1 645	1 970	1 918
	Median	1 885	2 330	1 800	1 945	1 885
	SD (SE)	0 2313 (0 1157)	0 4778 (0 2389)	0 5162 (0.2581)	0 5769 (0 2040)	0 4818 (0 1391)
	Min, Max	1 48, 2 01	1 76, 2 76	0 90, 2 08	0 90, 2 76	0 90, 2 76
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	0 020	0 408	0 143	0 275	0 190
	Median	-0 020	0 405	0 265	0 330	0 265
	SD (SE)	0 2132 (0 1066)	0 1436 (0 0718)	0 3201 (0 1601)	0 2699 (0 0954)	0 2730 (0 0788)
	Min, Max	-0 17, 0 29	0 24, 0.58	-0 33, 0 37	-0 33, 0 58	-0 33, 0 58
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	1 474	21 149	7 786	14 467	10 136
	Median	-0 906	21 100	15 482	18 748	16 322
	SD (SE)	12 7296 (6 3648)	4 4317 (2 2159)	23 7339 (11 8670)	17 3451 (6 1324)	16 6304 (4 8008)
	Min, Max	-10 30, 18 01	15 79, 26 61	-26 83, 27 01	-26 83, 27 01	-26 83, 27 01

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
FVC (L)						
Week 168	n	4	4	4	8	12
	Mean	1 800	2 198	1 803	2 000	1 933
	Median	1 835	2 165	1 825	1 935	1 885
	SD (SE)	0 1208 (0 0604)	0 5087 (0 2544)	0 3413 (0 1707)	0 4532 (0 1602)	0 3800 (0 1097)
	Min, Max	1 63, 1 90	1.64, 2 82	1 37, 2 19	1 37, 2 82	1 37, 2 82
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	0 005	0 310	0 300	0 305	0 205
	Median	-0 035	0 240	0 325	0 285	0 225
	SD (SE)	0 2057 (0 1028)	0 2324 (0 1162)	0 1225 (0 0612)	0 1720 (0 0608)	0 2285 (0 0659)
	Min, Max	-0 20, 0 29	0 12, 0 64	0.14, 0 41	0 12, 0 64	-0 20, 0 64
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	1 049	15 868	19 679	17 774	12 199
	Median	-1 908	13 110	19 799	17 264	13 973
	SD (SE)	11 9485 (5 9742)	10 1303 (5 0652)	7 1816 (3 5908)	8 3806 (2 9630)	12 3062 (3 5525)
	Min, Max	-10 00, 18 01	7 89, 29 36	11 38, 27 74	7 89, 29 36	-10 00, 29 36

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Day 1	n	4	4	4	8	12
	Mean	71 8	109 2	56 8	83 0	79 3
	Median	69 9	103 3	41 2	83 9	78 0
	SD (SE)	19 04 (9 52)	31 11 (15 55)	34 21 (17 10)	41 23 (14 58)	34 80 (10 04)
	Min, Max	51, 97	83, 147	37, 108	37, 147	37, 147
Baseline[1]	n	4	4	4	8	12
	Mean	71 8	109 2	56 8	83 0	79 3
	Median	69 9	103 3	41 2	83 9	78 0
	SD (SE)	19 04 (9 52)	31 11 (15 55)	34 21 (17 10)	41 23 (14 58)	34 80 (10 04)
	Min, Max	51, 97	83, 147	37, 108	37, 147	37, 147

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 12	n	4	4	4	8	12
	Mean	76 4	100 5	66 0	83 3	81 0
	Median	68 9	102 8	64 2	85 8	79 6
	SD (SE)	31 30 (15 65)	18 43 (9 22)	23 23 (11 62)	26 80 (9 47)	27 12 (7 83)
	Min, Max	48, 120	78, 118	45, 91	45, 118	45, 120
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	4 6	-8.6	9.2	0 3	1 7
	Median	2 9	-7 7	7 9	-0 8	1 0
	SD (SE)	15 06 (7 53)	16 07 (8 04)	22 86 (11 43)	20 63 (7 29)	18 36 (5 30)
	Min, Max	-11, 24	-29, 10	-17, 38	-29, 38	-29, 38
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	4 2	-5 7	28 9	11 6	9 1
	Median	3 7	-7 6	20 9	2 5	3 1
	SD (SE)	17 95 (8 97)	13 24 (6 62)	44 70 (22 35)	35 67 (12 61)	30 19 (8 71)
	Min, Max	-15, 24	-20, 12	-16, 90	-20, 90	-20, 90

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 24	n	4	4	4	8	12
	Mean	76 0	80 9	69 4	75 1	75 4
	Median	74 0	72 0	69 0	72 0	72 0
	SD (SE)	16 79 (8 39)	19 51 (9 75)	28 18 (14 09)	23 28 (8 23)	20 54 (5 93)
	Min, Max	58, 98	70, 110	42, 98	42, 110	42, 110
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	4 2	-28 2	12 5	-7 8	-3 8
	Median	2.8	-25 2	6 9	-12 5	-3 6
	SD (SE)	11 82 (5 91)	19 01 (9 51)	30 76 (15 38)	32 18 (11 38)	27 06 (7 81)
	Min, Max	-9, 20	-51, -12	-19, 55	-51, 55	-51, 55
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	8 4	-24 2	36 9	6 3	7 0
	Median	3 6	-20 5	17 9	-14 9	-5 7
	SD (SE)	22 06 (11 03)	12 68 (6 34)	63 63 (31 82)	53 56 (18 94)	44 27 (12 78)
	Min, Max	-13, 39	-42, -14	-17, 129	-42, 129	-42, 129

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 36	n	4	4	4	8	12
	Mean	77 0	89 7	66 4	78 0	77.7
	Median	77 7	85 8	65 3	79 9	78 4
	SD (SE)	16 59 (8 30)	15 17 (7 59)	21 47 (10 73)	21 22 (7 50)	19 03 (5 49)
	Min, Max	56, 97	76, 111	45, 90	45, 111	45, 111
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	5 2	-19 5	9 6	-4.9	-1 6
	Median	5 2	-20 1	10 2	0 6	5 0
	SD (SE)	4 23 (2 11)	25 39 (12 70)	22 22 (11 11)	27 02 (9 55)	22 23 (6 42)
	Min, Max	0, 10	-45, 7	-18, 36	-45, 36	-45, 36
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	8 2	-14 3	30 5	8 1	8 1
	Median	8 7	-14 6	27 3	2 4	8 1
	SD (SE)	6 47 (3 24)	20 65 (10 33)	42 83 (21 41)	39 27 (13 89)	31 51 (9 10)
	Min, Max	0, 15	-37, 9	-16, 84	-37, 84	-37, 84

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 48	n	4	4	4	8	12
	Mean	62 9	99 6	68 6	84 1	77 0
	Median	61 1	100 1	70 7	85 6	81 3
	SD (SE)	24 86 (12 43)	19 02 (9.51)	29 53 (14 77)	28 33 (10 02)	28 08 (8 11)
	Min, Max	39, 90	79, 119	33, 100	33, 119	33, 119
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-8 9	-9 6	11 8	1 1	-2 2
	Median	-6 2	-7 4	7 1	-4 8	-5 8
	SD (SE)	13.38 (6 69)	14 06 (7 03)	22 37 (11 19)	20 74 (7 33)	18 63 (5 38)
	Min, Max	-28, 4	-29, 5	-8, 41	-29, 41	-29, 41
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-13 5	-6 9	30 8	12.0	3 5
	Median	-9 2	-7.1	19 3	-6 9	-6 9
	SD (SE)	20 00 (10 00)	10 44 (5 22)	50 20 (25 10)	39 15 (13 84)	35.24 (10 17)
	Min, Max	-41, 6	-19, 6	-11, 95	-19, 95	-41, 95

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 62	n	4	4	4	8	12
	Mean	63 4	90 9	60 7	75 8	71 7
	Median	61 3	96 7	55 4	81 0	72 2
	SD (SE)	18 35 (9 18)	12 61 (6.31)	23 00 (11 50)	23 59 (8 34)	21 98 (6 35)
	Min, Max	46, 85	72, 98	42, 90	42, 98	42, 98
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-8 4	-18 2	3 9	-7 2	-7 6
	Median	-8 1	-17 0	3 9	-4 0	-7 6
	SD (SE)	6.85 (3 43)	24 87 (12 43)	17 97 (8 98)	23 30 (8 24)	18 94 (5 47)
	Min, Max	-16, -1	-49, 10	-18, 26	-49, 26	-49, 26
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-11 7	-13 4	16 1	1 4	-3 0
	Median	-10 5	-16 0	10 4	-3 3	-10 5
	SD (SE)	9 56 (4 78)	18 97 (9 48)	32 37 (16 18)	29 17 (10 31)	24 66 (7 12)
	Min, Max	-24, -1	-33, 12	-17, 60	-33, 60	-33, 60

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 74	n	4	4	4	8	12
	Mean	73 7	92 4	67 9	80 1	78 0
	Median	63 7	87 0	63 7	81 9	77 2
	SD (SE)	26 24 (13 12)	18 84 (9 42)	34 70 (17 35)	29 00 (10 25)	27 07 (7 82)
	Min, Max	56, 112	76, 120	32, 112	32, 120	32, 120
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	1 9	-16 7	11 0	-2 8	-1 3
	Median	-2 1	-18 3	8 1	-2 0	-2 0
	SD (SE)	28 22 (14 11)	18 80 (9 40)	18 26 (9 13)	22 69 (8 02)	23 46 (6.77)
	Min, Max	-27, 39	-36, 6	-7, 36	-36, 36	-36, 39
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	5 5	-13 0	25 5	6 3	6 0
	Median	-1 5	-14 6	18 8	-3 5	-3 5
	SD (SE)	36 18 (18 09)	15 42 (7 71)	44 23 (22 12)	36 94 (13 06)	35 00 (10 10)
	Min, Max	-28, 53	-30, 7	-19, 83	-30, 83	-30, 83

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 84	n	4	4	4	8	12
	Mean	66 6	81 0	68 1	74 5	71 9
	Median	66 5	81 7	56.5	73 3	73 3
	SD (SE)	21 61 (10 81)	14 75 (7 38)	39 32 (19 66)	28 35 (10 02)	25 58 (7 38)
	Min, Max	47, 87	63, 98	36, 123	36, 123	36, 123
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-5 2	-28 2	11 3	-8 4	-7 4
	Median	-7 2	-29 1	11 3	-4 3	-4 3
	SD (SE)	14 52 (7 26)	19 09 (9 54)	12 64 (6 32)	25 86 (9 14)	22 04 (6 36)
	Min, Max	-20, 13	-50, -5	-4, 26	-50, 26	-50, 26
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-7 0	-23 8	21 6	-1 1	-3 1
	Median	-8 2	-27 9	17 0	-7 6	-7 6
	SD (SE)	20 15 (10 08)	12 32 (6 16)	29 47 (14 74)	32 03 (11 32)	27 78 (8 02)
	Min, Max	-30, 18	-34, -6	-9, 62	-34, 62	-34, 62

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 96	n	4	4	4	8	12
	Mean	76 3	94 3	54 8	74 5	75 1
	Median	74 7	91 9	49 5	87 8	86 9
	SD (SE)	17 21 (8 61)	8 50 (4 25)	25 65 (12 83)	27 53 (9 73)	23 75 (6 86)
	Min, Max	60, 95	88, 106	31, 90	31, 106	31, 106
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	4 5	-14 9	-2 0	-8 5	-4 1
	Median	3 1	-15 5	-2 2	-3 1	-0 2
	SD (SE)	14 43 (7 22)	26 98 (13 49)	14 70 (7 35)	21 26 (7 52)	19 63 (5 67)
	Min, Max	-10, 22	-42, 13	-18, 15	-42, 15	-42, 22
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	8 4	-9 2	1 9	-3 7	0 3
	Median	6 9	-12 2	-1 8	-6 8	-0 8
	SD (SE)	19 52 (9 76)	22 36 (11 18)	26 96 (13 48)	23 69 (8.38)	22 28 (6 43)
	Min, Max	-11, 30	-28, 16	-23, 34	-28, 34	-28, 34

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 120	n	4	4	4	8	12
	Mean	78 7	94 9	64 5	79 7	79 3
	Median	81 7	93 8	59 8	89 7	86 7
	SD (SE)	14 15 (7 07)	8 28 (4 14)	22 03 (11 02)	22 40 (7 92)	19 34 (5 58)
	Min, Max	60, 91	86, 106	44, 95	44, 106	44, 106
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	6 9	-14 3	7 7	-3 3	0 1
	Median	9 3	-13 2	10 4	2 4	6 5
	SD (SE)	8 34 (4 17)	24 22 (12 11)	16 21 (8 10)	22 40 (7 92)	19 06 (5 50)
	Min, Max	-5, 14	-41, 11	-13, 23	-41, 23	-41, 23
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	11 5	-9 1	24 5	7.7	8 9
	Median	16 0	-10 7	27 8	5 4	11 3
	SD (SE)	11 43 (5 72)	19 38 (9 69)	31 35 (15 68)	30 08 (10 64)	24 80 (7 16)
	Min, Max	-5, 19	-28, 13	-12, 55	-28, 55	-28, 55

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 144	n	4	4	4	8	12
	Mean	72 3	94 8	60 1	77.4	75 7
	Median	70 6	92 2	57 1	83 8	83 8
	SD (SE)	19 18 (9 59)	13 35 (6 67)	22 62 (11 31)	25 30 (8 94)	22 67 (6 54)
	Min, Max	55, 93	84, 111	36, 90	36, 111	36, 111
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	0 5	-14 4	3 3	-5.6	-3 5
	Median	-2 7	-5 8	5 9	-0 9	-0 9
	SD (SE)	15 10 (7 55)	22 30 (11 15)	16 37 (8 18)	20 42 (7 22)	18 34 (5 30)
	Min, Max	-12, 20	-47, 1	-18, 19	-47, 19	-47, 20
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	2 2	-10 1	14 5	2 2	2 2
	Median	-0 5	-5 0	14 8	-1 5	-1 5
	SD (SE)	20 50 (10 25)	15 09 (7 54)	28 55 (14 27)	24 90 (8 80)	22 56 (6 51)
	Min, Max	-17, 27	-32, 1	-17, 45	-32, 45	-32, 45

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP % of Predicted Value (%)						
Week 168	n	4	4	4	8	12
	Mean	73 8	88 3	60 7	74 5	74 3
	Median	73 9	89 8	58 7	78 0	78 0
	SD (SE)	20 56 (10 28)	12 22 (6 11)	19 72 (9 86)	21 18 (7 49)	20 02 (5 78)
	Min, Max	53, 94	75, 99	41, 85	41, 99	41, 99
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	2 0	-20 9	3 9	-8 5	-5 0
	Median	-2 5	-16 4	5 3	-3 2	-2 5
	SD (SE)	13 55 (6 77)	34 12 (17 06)	21 11 (10 55)	29 41 (10 40)	25 04 (7 23)
	Min, Max	-8, 21	-66, 16	-23, 28	-66, 28	-66, 28
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	3 5	-14 2	18.9	2 3	2 7
	Median	-1 6	-15 3	13 1	-0 9	-1 6
	SD (SE)	18 18 (9 09)	26 17 (13 08)	38 34 (19 17)	35 14 (12 43)	29 61 (8 55)
	Min, Max	-11, 29	-45, 19	-22, 71	-45, 71	-45 71

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H20)						
Day 1	n	4	4	4	8	12
	Mean	59 3	97 0	48 0	72 5	68 1
	Median	58 5	93 5	35 0	74 5	68 0
	SD (SE)	13 00 (6 50)	27 87 (13 93)	28 04 (14 02)	36 82 (13 02)	30 84 (8 90)
	Min, Max	46, 74	70, 131	32, 90	32, 131	32, 131
Baseline[1]	n	4	4	4	8	12
	Mean	59 3	97 0	48 0	72 5	68 1
	Median	58 5	93 5	35 0	74 5	68 0
	SD (SE)	13 00 (6 50)	27 87 (13 93)	28 04 (14 02)	36 82 (13 02)	30 84 (8 90)
	Min, Max	46, 74	70, 131	32, 90	32, 131	32, 131

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H2O)						
Week 12	n	4	4	4	8	12
	Mean	63 5	90 8	56 3	73 5	70 2
	Median	58 0	91 0	54 0	75 5	68 0
	SD (SE)	21 39 (10 70)	16 19 (8 10)	16 66 (8 33)	23 90 (8 45)	22 64 (6 54)
	Min, Max	44, 94	74, 107	40, 77	40, 107	40, 107
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	4 3	-6 3	8 3	1 0	2 1
	Median	3 0	-5 5	8 0	0 0	1 5
	SD (SE)	12 61 (6 30)	13 91 (6 96)	17 73 (8 86)	16 66 (5 89)	14 92 (4 31)
	Min, Max	-9, 20	-24, 10	-13, 30	-24, 30	-24, 30
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	6 2	-4 0	31 3	13 6	11 2
	Median	5 7	-5 9	22.9	4 4	5 0
	SD (SE)	18 52 (9 26)	13 51 (6 75)	45 76 (22 88)	36 47 (12 90)	30 88 (8 91)
	Min, Max	-14, 27	-18, 14	-14, 94	-18, 94	-18, 94

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H2O)						
Week 24	n	4	4	4	8	12
	Mean	65 5	74 3	59 5	66 9	66 4
	Median	69 0	67 5	60 0	67 5	68 0
	SD (SE)	13 89 (6 95)	18 21 (9 10)	19 67 (9 84)	19 24 (6 80)	16 99 (4 90)
	Min, Max	46, 78	61, 101	41, 77	41, 101	41, 101
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	6 3	-22.8	11 5	-5 6	-1 7
	Median	5 0	-19 5	7 5	-9 0	-0 5
	SD (SE)	10 34 (5 17)	16 74 (8 37)	23 76 (11 88)	26 40 (9 33)	22 52 (6 50)
	Min, Max	-5, 20	-43, -9	-13, 44	-43, 44	-43, 44
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	12 0	-21 7	41 5	9 9	10 6
	Median	7 2	-17 9	21 4	-12 1	-2 2
	SD (SE)	22 50 (11 25)	13 09 (6 54)	66 30 (33 15)	55 67 (19 68)	45 95 (13 26)
	Min, Max	-10, 43	-40, -11	-14, 138	-40, 138	-40, 138

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H20)						
Week 36	n	4	4	4	8	12
	Mean	66 8	83 3	58 5	70 9	69 5
	Median	68 0	79 5	57 0	75 0	72 5
	SD (SE)	11 41 (5 71)	13 77 (6 88)	16 13 (8 07)	19 18 (6.78)	16 54 (4 78)
	Min, Max	53, 78	71, 103	41, 79	41, 103	41, 103
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	7.5	-13 8	10.5	-1 6	1 4
	Median	7 5	-14 0	11 5	3 0	6 5
	SD (SE)	2 89 (1 44)	22.34 (11 17)	17 37 (8 68)	22 61 (7 99)	18 65 (5 38)
	Min, Max	4, 11	-37, 10	-11, 30	-37, 30	-37, 30
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	13 6	-10 3	36 8	13 2	13 4
	Median	13 7	-10 7	32 9	7 1	13 2
	SD (SE)	6 72 (3 36)	21 66 (10.83)	45 36 (22 68)	41 45 (14 65)	33 25 (9 60)
	Min, Max	5, 22	-34, 14	-12, 94	-34, 94	-34, 94

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H2O)						
Week 48	n	4	4	4	8	12
	Mean	55 8	93 8	60 8	77 3	70 1
	Median	58 5	92 5	60 5	78 5	74 0
	SD (SE)	21 55 (10 77)	17 78 (8 89)	23 47 (11 74)	26 13 (9 24)	25 95 (7 49)
	Min, Max	32, 74	78, 112	33, 89	33, 112	32, 112
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-3 5	-3 3	12 8	4 8	2 0
	Median	-1 5	-1 5	9 0	-1 0	-1 0
	SD (SE)	11 33 (5 66)	11 62 (5 81)	17 71 (8 85)	16 29 (5 76)	14 84 (4 29)
	Min, Max	-19, 8	-19, 9	-2, 35	-19, 35	-19, 35
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-7 9	-1 2	39 2	19 0	10 0
	Median	-3 3	-1 6	26 6	-1 2	-1 2
	SD (SE)	21 03 (10 52)	11 18 (5 59)	54 14 (27 07)	42 15 (14 90)	37 77 (10 90)
	Min, Max	-37, 12	-15, 13	-6, 109	-15, 109	-37, 109

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H2O)						
Week 62	n	4	4	4	8	12
	Mean	57 0	87 3	55 0	71 1	66 4
	Median	57 5	94 0	49 5	73 5	68 0
	SD (SE)	15 64 (7 82)	14 17 (7 09)	18 67 (9 34)	23 08 (8 16)	21 31 (6 15)
	Min, Max	42, 71	66, 95	40, 81	40, 95	40, 95
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-2 3	-9 8	7 0	-1 4	-1.7
	Median	-2 0	-9 0	6 5	0 5	-2 0
	SD (SE)	5 38 (2 69)	22 04 (11 02)	13 54 (6 77)	19 15 (6 77)	15 54 (4 49)
	Min, Max	-9, 4	-37, 16	-9, 24	-37, 24	-37, 24
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-4 5	-6 7	25 5	9 4	4 8
	Median	-3 1	-9 3	18 6	4 3	-3 1
	SD (SE)	9 83 (4 92)	20 26 (10 13)	35 79 (17 90)	31.96 (11 30)	26 89 (7 76)
	Min, Max	-18, 6	-28, 20	-10, 75	-28, 75	-28, 75

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H20)						
Week 74	n	4	4	4	8	12
	Mean	68 3	89 5	62 3	75 9	73 3
	Median	58 0	82 5	58 0	79 5	71 0
	SD (SE)	28 32 (14 16)	17 86 (8 93)	29 95 (14 97)	27.08 (9 57)	26 45 (7 63)
	Min, Max	47, 110	77, 116	31, 102	31, 116	31, 116
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	9 0	-7 5	14 3	3 4	5 3
	Median	3 5	-8 5	14 0	5 0	4 5
	SD (SE)	25 65 (12 83)	16 05 (8 03)	15 20 (7 60)	18 56 (6 56)	20 16 (5 82)
	Min, Max	-15, 44	-25, 12	-4, 33	-25, 33	-25, 44
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	15 6	-5 0	37 7	16 3	16 1
	Median	8 0	-7 0	29 5	5 4	5 4
	SD (SE)	38 79 (19 39)	17.00 (8 50)	49 50 (24 75)	41 17 (14 55)	38 59 (11 14)
	Min, Max	-20, 67	-23, 17	-11, 103	-23, 103	-23, 103

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum.sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H20)						
Week 84	n	4	4	4	8	12
	Mean	61 8	79.3	63 3	71 3	68 1
	Median	60 5	78 5	52 0	68 5	68 0
	SD (SE)	21.05 (10 52)	13 84 (6 92)	35 11 (17 56)	26 15 (9 24)	24 04 (6 94)
	Min, Max	40, 86	64, 96	35, 114	35, 114	35, 114
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	2 5	-17.8	15 3	-1 3	0 0
	Median	0 5	-19 5	17 5	1 5	1 5
	SD (SE)	13 03 (6 51)	16 07 (8 04)	12 15 (6 07)	22 02 (7 79)	18 93 (5 46)
	Min, Max	-11, 20	-35, 3	0, 26	-35, 26	-35, 26
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	3 1	-15 9	34 8	9 5	7 4
	Median	1 9	-20 6	29 0	2 1	2 1
	SD (SE)	21 56 (10 78)	13 83 (6 92)	33 89 (16 95)	36 20 (12 80)	31 15 (8 99)
	Min, Max	-22, 30	-27, 4	0, 81	-27, 81	-27, 81

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16.2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H20)						
Week 96	n	4	4	4	8	12
	Mean	71 8	93 5	51 8	72 6	72 3
	Median	68 0	91 0	46 5	85 5	79 5
	SD (SE)	18 21 (9 10)	7 90 (3 95)	22 95 (11 48)	27 40 (9 69)	23 84 (6 88)
	Min, Max	55, 96	87, 105	30, 84	30, 105	30, 105
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	12 5	-3 5	3 8	0 1	4 3
	Median	9 5	-4 5	2 0	2 0	6 5
	SD (SE)	13 13 (6 56)	23 47 (11 74)	11 18 (5 59)	17 46 (6 17)	16 67 (4 81)
	Min, Max	1, 30	-26, 21	-6, 17	-26, 21	-26, 30
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	21 8	1 5	14 5	8 0	12 6
	Median	20 2	-2 1	9 5	4 3	11 5
	SD (SE)	20 73 (10 37)	25 13 (12 56)	31 06 (15.53)	27 06 (9 57)	25 09 (7 24)
	Min, Max	1, 45	-20, 30	-14, 53	-20, 53	-20, 53

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H2O)						
Week 120	n	4	4	4	8	12
	Mean	75 5	96 5	62 8	79 6	78 3
	Median	75 0	93 5	58 0	91 0	86 0
	SD (SE)	12 79 (6 40)	8 02 (4 01)	19 96 (9 98)	22 88 (8 09)	19 55 (5 64)
	Min, Max	62, 90	91, 108	44, 91	44, 108	44, 108
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	16 3	-0 5	14 8	7 1	10 2
	Median	16 5	0 0	16 0	10 5	14.0
	SD (SE)	6 55 (3 28)	20 47 (10 23)	11 79 (5 89)	17 48 (6 18)	15 04 (4 34)
	Min, Max	8, 24	-23, 21	1, 26	-23, 26	-23, 26
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	28 8	4 1	43 4	23 8	25 5
	Median	34.1	2 0	45 7	20 5	27 9
	SD (SE)	12 07 (6 04)	22 32 (11 16)	36 66 (18 33)	35 09 (12 41)	28 80 (8 31)
	Min, Max	11, 36	-18, 30	1, 81	-18, 81	-18, 81

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H2O)						
Week 144	n	4	4	4	8	12
	Mean	71 8	99 0	59 8	79 4	76 8
	Median	69 0	98 0	55 0	86 5	81 0
	SD (SE)	20 79 (10 40)	14 31 (7 15)	20 76 (10.38)	26 69 (9 44)	24 20 (6 98)
	Min, Max	51, 98	84, 116	40, 89	40, 116	40, 116
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	12 5	2 0	11 8	6 9	8 8
	Median	9 0	10 0	12 0	10 0	10 0
	SD (SE)	14 27 (7 14)	18 83 (9 42)	11 70 (5 85)	15 42 (5 45)	14 65 (4 23)
	Min, Max	0, 32	-26, 14	-1, 24	-26, 24	-26, 32
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	21 1	5 7	35 6	20 7	20 8
	Median	17 9	11 3	34 3	14 7	14 7
	SD (SE)	22 57 (11 29)	17 80 (8 90)	35 14 (17 57)	30 34 (10 73)	26 92 (7 77)
	Min, Max	0, 48	-20, 20	-1, 75	-20, 75	-20, 75

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MEP (cm H2O)						
Week 168	n	4	4	4	8	12
	Mean	75 0	94 3	62 5	78 4	77 3
	Median	71 0	94 0	59 0	84 5	83 5
	SD (SE)	22 06 (11.03)	11 00 (5 50)	19 89 (9 95)	22 57 (7 98)	21 44 (6 19)
	Min, Max	56, 102	83, 106	46, 86	46, 106	46, 106
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	15 8	-2 8	14 5	5 9	9 2
	Median	11 0	1 0	12.5	7 5	10 5
	SD (SE)	13 82 (6 91)	31 02 (15 51)	16 94 (8 47)	24 91 (8 81)	21 69 (6 26)
	Min, Max	5, 36	-44, 31	-4, 37	-44, 37	-44, 37
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	26 0	3 5	44 1	23 8	24 5
	Median	19 8	1 6	37 6	18 2	19 8
	SD (SE)	20 27 (10 14)	31 99 (15 99)	45 88 (22 94)	42 57 (15 05)	35 59 (10 27)
	Min, Max	10, 55	-34, 44	-4, 106	-34, 106	-34, 106

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Day 1	n	4	4	4	8	12
	Mean	80 8	109 3	85 0	97 1	91 7
	Median	64 1	115 6	84 9	103 1	90 9
	SD (SE)	43 70 (21 85)	15 60 (7 80)	22 66 (11 33)	22 20 (7 85)	29 98 (8.65)
	Min, Max	50, 145	86, 120	59, 111	59, 120	50, 145
Baseline[1]	n	4	4	4	8	12
	Mean	80 8	109 3	85 0	97 1	91 7
	Median	64 1	115 6	84 9	103 1	90 9
	SD (SE)	43 70 (21 85)	15 60 (7 80)	22 66 (11 33)	22 20 (7 85)	29 98 (8 65)
	Min, Max	50, 145	86, 120	59, 111	59, 120	50, 145

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 12	n	4	4	4	8	12
	Mean	74 4	102 1	70 2	86 2	82 3
	Median	72 0	101 9	69 3	99 3	90 0
	SD (SE)	16 71 (8 36)	1 43 (0 71)	25 79 (12 90)	24 02 (8 49)	21 84 (6 30)
	Min, Max	60, 94	101, 104	44, 98	44, 104	44, 104
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-6 4	-7 1	-14 8	-11 0	-9 4
	Median	6 6	-12 9	-11 2	-11 4	-9 9
	SD (SE)	29 99 (14 99)	16 05 (8 02)	10 38 (5 19)	13 16 (4 65)	18 99 (5 48)
	Min, Max	-51, 12	-19, 16	-30, -7	-30, 16	-51, 16
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	1 9	-4 8	-18 3	-11 6	-7 1
	Median	11 8	-11 1	-11 5	-11 5	-10 8
	SD (SE)	25 37 (12.69)	16 08 (8 04)	14 64 (7 32)	15 97 (5 65)	19 54 (5 64)
	Min, Max	-35, 19	-16, 19	-40, -10	-40, 19	-40, 19

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 24	n	4	4	4	8	12
	Mean	80 8	97 8	73 9	85 9	84 2
	Median	75 1	97 3	73 0	88 3	85 9
	SD (SE)	16 85 (8 43)	11 17 (5 59)	15 20 (7 60)	17 78 (6 29)	16 88 (4 87)
	Min, Max	68, 105	88, 109	60, 90	60, 109	60, 109
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	-0 1	-11 4	-11 1	-11 3	-7 5
	Median	9 2	-9 5	-10 1	-10 1	-5 5
	SD (SE)	27 28 (13.64)	13 99 (7 00)	12 19 (6 10)	12 15 (4 30)	18 09 (5 22)
	Min, Max	-40, 21	-30, 3	-26, 2	-30, 3	-40, 21
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	11 1	-9 5	-11 4	-10 4	-3 3
	Median	14 6	-8 0	-12 7	-9 1	-5 3
	SD (SE)	29 11 (14 55)	12.22 (6 11)	12 51 (6 26)	11 49 (4 06)	20 68 (5 97)
	Min, Max	-28, 43	-25, 3	-24, 4	-25, 4	-28, 43

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 36	n	4	4	4	8	12
	Mean	79 0	98 3	73 2	85 8	83.5
	Median	70 3	101 2	72 3	87 4	75 3
	SD (SE)	29 66 (14 83)	25 78 (12 89)	26 39 (13 19)	27 61 (9 76)	27 13 (7 83)
	Min, Max	54, 122	64, 127	42, 106	42, 127	42, 127
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	-1 8	-11 0	-11 8	-11 4	-8 2
	Median	-0 5	-3 9	-12 4	-12 3	-4 7
	SD (SE)	17 21 (8 61)	32 55 (16 28)	18 43 (9 22)	24 49 (8 66)	22 02 (6 36)
	Min, Max	-24, 17	-53, 17	-32, 10	-53, 17	-53, 17
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	3 6	-7 9	-13 0	-10 5	-5 8
	Median	0 4	-3 0	-12 6	-10 4	-5 5
	SD (SE)	20 25 (10 12)	29 36 (14 68)	25 55 (12 77)	25 62 (9 06)	24 04 (6 94)
	Min, Max	-16, 30	-45, 20	-43, 17	-45, 20	-45, 30

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6.8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 48	n	4	4	4	8	12
	Mean	79 4	95 7	86 1	90 9	87 1
	Median	75 8	93 3	82 5	90 3	86 5
	SD (SE)	17 29 (8 64)	7 65 (3 82)	12 09 (6 05)	10 66 (3.77)	13 63 (3 93)
	Min, Max	64, 102	90, 107	76, 104	76, 107	64, 107
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	-1 4	-13 6	1 1	-6 2	-4 6
	Median	11 7	-17 6	0 1	-7 3	0 1
	SD (SE)	27 78 (13 89)	18.11 (9 05)	13 12 (6.56)	16 63 (5 88)	19 80 (5 72)
	Min, Max	-43, 14	-29, 9	-12, 17	-29, 17	-43, 17
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	8 7	-10 8	4 6	-3 1	0 8
	Median	18 2	-15 1	1 7	-6 5	1 7
	SD (SE)	25 99 (12 99)	16 54 (8 27)	18 11 (9 06)	18 05 (6 38)	20 62 (5 95)
	Min Max	-30, 28	-24, 11	-13, 28	-24, 28	-30, 28

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 62	n	4	4	4	8	12
	Mean	68 5	95 3	87 2	91 2	83 6
	Median	73 2	91 6	79 5	88 6	79 8
	SD (SE)	13 26 (6 63)	15 38 (7 69)	34 73 (17 36)	25 24 (8 92)	24 06 (6 94)
	Min, Max	49, 78	81, 117	55, 135	55, 135	49, 135
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-12 4	-14 0	2.2	-5 9	-8 1
	Median	0 2	-13 5	2 0	-1 8	0 2
	SD (SE)	37.11 (18 56)	23 57 (11 79)	18 90 (9 45)	21 59 (7 63)	26 12 (7 54)
	Min, Max	-67, 17	-38, 10	-19, 24	-38, 24	-67, 24
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-4.2	-11 0	1 6	-4.7	-4 6
	Median	0 0	-11 5	5 3	-2 1	0 0
	SD (SE)	31 12 (15 56)	20 90 (10 45)	22 13 (11 07)	21 04 (7 44)	23 36 (6 74)
	Min, Max	-46, 29	-32, 11	-26, 22	-32, 22	-46, 29

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run: 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 74	n	4	4	4	8	12
	Mean	70 7	107 5	91 2	99 4	89 8
	Median	66 8	105 0	90 6	94 8	90 5
	SD (SE)	20 22 (10 11)	21 16 (10 58)	34 34 (17 17)	27 81 (9 83)	28.33 (8 18)
	Min, Max	51, 98	87, 133	50, 134	50, 134	50, 134
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-10 1	-1 7	6 2	2 2	-1 9
	Median	-3 9	-2 0	6 5	4 6	0 7
	SD (SE)	28 95 (14 47)	30 86 (15 43)	28 06 (14.03)	27 63 (9 77)	27 41 (7 91)
	Min, Max	-47, 14	-33, 30	-24, 36	-33, 36	-47, 36
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-3 0	1 1	9 6	5 3	2 6
	Median	-2 3	-1 4	5 2	3 2	3 2
	SD (SE)	31 14 (15 57)	29 55 (14.77)	40 53 (20 26)	33 15 (11 72)	31 31 (9 04)
	Min, Max	-32, 25	-27, 35	-33, 61	-33, 61	-33, 61

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/Time of run 06MAR2015 14 00
Reference listing L 16.2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 84	n	4	4	4	8	12
	Mean	76 4	103 2	97 1	100 2	92 2
	Median	77.5	95 8	92 6	92 6	87 1
	SD (SE)	13 91 (6 96)	28 92 (14 46)	26 98 (13 49)	26 10 (9 23)	24 97 (7 21)
	Min, Max	58, 92	79, 142	69, 134	69, 142	58, 142
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	-4 5	-6 0	12 1	3 0	0 5
	Median	2 6	-7 1	9 0	8 1	6 1
	SD (SE)	36 88 (18 44)	35 71 (17 86)	20 89 (10 44)	28 76 (10 17)	30 18 (8 71)
	Min, Max	-53, 30	-38, 28	-6, 36	-38, 36	-53, 36
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	9 0	-3 2	17.2	7 0	7 7
	Median	6 4	-2 5	7 4	7 4	7 4
	SD (SE)	43 95 (21.97)	32 27 (16 14)	31 71 (15 85)	31 55 (11 16)	34 08 (9 84)
	Min, Max	-37, 60	-33, 25	-7, 61	-33, 61	-37, 61

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 96	n	4	4	4	8	12
	Mean	72 5	100 4	80 7	90 5	84 5
	Median	71 7	99 0	81 0	88 8	83 7
	SD (SE)	6 06 (3 03)	13 67 (6 83)	25 91 (12 95)	21 87 (7 73)	19 82 (5 72)
	Min, Max	67, 80	88, 116	49, 111	49, 116	49, 116
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-8 3	-8 9	-4 3	-6 6	-7 2
	Median	11 5	-17 5	-3 7	-7 0	-2 6
	SD (SE)	47 30 (23 65)	27 89 (13 95)	16.79 (8 39)	21 46 (7 59)	30 06 (8 68)
	Min, Max	-78, 22	-30, 29	-25, 15	-30, 29	-78, 29
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	6 9	-5 2	-4 0	-4 6	-0 8
	Median	21 8	-15 0	-3 9	-6 8	-2 2
	SD (SE)	43 19 (21 60)	27 89 (13 94)	24 42 (12 21)	24 27 (8 58)	30 27 (8 74)
	Min, Max	-54, 38	-25, 34	-34, 25	-34, 34	-54, 38

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 120	n	4	4	4	8	12
	Mean	86 0	105 9	93 7	99 8	95 2
	Median	86 0	103 6	90.3	94 2	88 1
	SD (SE)	1 74 (0 87)	28 74 (14 37)	25 27 (12 63)	25 88 (9 15)	21 75 (6 28)
	Min, Max	84, 88	74, 142	67, 128	67, 142	67, 142
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	5 2	-3 4	8 7	2 7	3 5
	Median	20 4	1 9	4 9	4 9	15 7
	SD (SE)	42 73 (21 36)	36 10 (18 05)	19 76 (9 88)	27 71 (9 80)	31 43 (9 07)
	Min, Max	-57, 37	-46, 28	-8, 33	-46, 33	-57, 37
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	25 3	-0 6	13 1	6 3	12 6
	Median	33 0	3 5	3 9	3 9	17 9
	SD (SE)	48 56 (24 28)	32 81 (16 41)	30 32 (15 16)	30 16 (10 66)	36 19 (10 45)
	Min, Max	-40, 75	-38, 29	-10, 55	-38, 55	-40, 75

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 144	n	4	4	4	8	12
	Mean	89 7	102 4	89 4	95 9	93 9
	Median	91 8	99 2	82 6	83 3	85 6
	SD (SE)	17 23 (8 61)	25 42 (12 71)	17 81 (8 91)	21 47 (7 59)	19 59 (5 66)
	Min, Max	67, 108	79, 132	77, 116	77, 132	67, 132
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	8 8	-6 8	4 5	-1.2	2.2
	Median	17 3	-9 1	3 8	3 8	3 8
	SD (SE)	36.22 (18 11)	35 22 (17 61)	15 18 (7 59)	25 82 (9 13)	28 40 (8 20)
	Min, Max	-37, 38	-38, 29	-13, 24	-38, 29	-38, 38
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	27 8	-3 4	8 4	2 5	10 9
	Median	30 4	-7 2	4 0	4 0	4 0
	SD (SE)	50 40 (25 20)	33 12 (16 56)	22 55 (11 28)	26 97 (9 54)	36 19 (10 45)
	Min, Max	-26, 76	-32, 33	-14, 40	-32, 40	-32, 76

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP % of Predicted Value (%)						
Week 168	n	4	4	4	8	12
	Mean	86 1	92 9	89 4	91 2	89 5
	Median	87 5	87 8	86 9	86 9	87 5
	SD (SE)	20 40 (10 20)	29 55 (14 77)	14 99 (7 50)	21 78 (7 70)	20 53 (5 93)
	Min, Max	60, 110	64, 133	74, 110	64, 133	60, 133
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	5 3	-16 3	4 4	-6 0	-2 2
	Median	13 2	-15 2	6 9	4 6	10 0
	SD (SE)	28 53 (14 26)	35 87 (17 94)	12 91 (6 45)	27 31 (9 65)	26 97 (7 78)
	Min, Max	-36, 30	-53, 18	-11, 15	-53, 18	-53, 30
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	17 8	-12 9	8 1	-2 4	4 4
	Median	21 6	-11 0	9 8	5 4	13 9
	SD (SE)	31 76 (15 88)	31 38 (15.69)	17 41 (8 71)	26 03 (9 20)	28 37 (8 19)
	Min, Max	-24, 52	-46, 16	-12, 25	-46, 25	-46, 52

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Day 1	n	4	4	4	8	12
	Mean	55 3	77 3	56 8	67 0	63 1
	Median	41 5	79 5	59 0	71 0	65 5
	SD (SE)	32 71 (16 36)	13 23 (6 61)	17 08 (8 54)	17 89 (6 32)	23 00 (6 64)
	Min, Max	34, 104	61, 89	37, 72	37, 89	34, 104
Baseline[1]	n	4	4	4	8	12
	Mean	55 3	77 3	56 8	67 0	63 1
	Median	41 5	79 5	59 0	71 0	65.5
	SD (SE)	32 71 (16 36)	13 23 (6 61)	17 08 (8 54)	17 89 (6 32)	23 00 (6 64)
	Min, Max	34, 104	61, 89	37, 72	37, 89	34, 104

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1.1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Week 12	n	4	4	4	8	12
	Mean	51 3	74 3	48 3	61 3	57 9
	Median	47 0	76 0	49 0	66 0	65 5
	SD (SE)	13 23 (6 61)	6 18 (3 09)	19 99 (9 99)	19 51 (6 90)	17 73 (5 12)
	Min, Max	41, 70	66, 79	29, 66	29, 79	29, 79
Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	-4 0	-3 0	-8 5	-5 8	-5 2
	Median	5 0	-7 0	-5 5	-6 5	-5 0
	SD (SE)	20 12 (10 06)	10 13 (5 07)	7 14 (3 57)	8 63 (3 05)	12 59 (3 63)
	Min, Max	-34, 8	-10, 12	19, -4	-19, 12	-34, 12
Percent Change from Baseline to Week 12	n	4	4	4	8	12
	Mean	3 5	-2 3	-16 5	-9 4	-5 1
	Median	13 1	-8 8	-10 3	-9 5	-8 8
	SD (SE)	24 81 (12 40)	14 68 (7 34)	15 57 (7 79)	15 93 (5 63)	19 22 (5 55)
	Min, Max	-33, 21	-11, 20	-40, -6	-40, 20	-40, 21

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Week 24	n	4	4	4	8	12
	Mean	56 5	72 3	51 8	62 0	60 2
	Median	49 5	69 5	48 5	67 0	61 0
	SD (SE)	15 02 (7 51)	8 77 (4 39)	15 26 (7 63)	15 90 (5 62)	15.16 (4 38)
	Min, Max	48, 79	65, 85	39, 71	39, 85	39, 85
Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	1 3	-5 0	-5 0	-5 0	-2 9
	Median	7 0	-3 0	-3 5	-3 0	-0 5
	SD (SE)	18 03 (9 01)	9 31 (4 65)	8 04 (4 02)	8 05 (2 85)	11 80 (3 41)
	Min, Max	-25, 16	-18, 4	-15, 2	-18, 4	-25, 16
Percent Change from Baseline to Week 24	n	4	4	4	8	12
	Mean	14 2	-5 4	-7 7	-6 5	0 4
	Median	17 0	-3 6	-7 6	-3 6	-0 7
	SD (SE)	29 30 (14 65)	11 32 (5 66)	13 01 (6 51)	11 36 (4 02)	20 51 (5 92)
	Min, Max	-24, 47	-21, 7	-21, 5	-21, 7	-24, 47

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing: L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Week 36	n	4	4	4	8	12
	Mean	56 5	72 5	51 3	61 9	60 1
	Median	47 5	78 0	52 0	66 5	57 0
	SD (SE)	25 30 (12 65)	14 73 (7 37)	19 52 (9 76)	19 63 (6 94)	20 66 (5 96)
	Min, Max	38, 93	51, 83	28, 73	28, 83	28, 93
Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	1 3	-4 8	-5 5	-5 1	-3 0
	Median	1 0	1 5	-4 5	-3 5	-0 5
	SD (SE)	10 50 (5 25)	23 00 (11 50)	11 96 (5 98)	16 97 (6 00)	14 94 (4 31)
	Min, Max	-11, 14	-36, 14	-20, 7	-36, 14	-36, 14
Percent Change from Baseline to Week 36	n	4	4	4	8	12
	Mean	7 9	-3 0	-8 9	-6 0	-1 4
	Median	3 6	3 1	-6 4	-3 8	-1 6
	SD (SE)	20 40 (10 20)	28 96 (14 48)	25 71 (12 85)	25 54 (9 03)	23 99 (6 92)
	Min, Max	-11, 35	-41, 23	-42, 19	-42, 23	-42, 35

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L.16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Week 48	n	4	4	4	8	12
	Mean	56 5	73.0	60 5	66 8	63 3
	Median	50 5	72 5	60 5	70 5	68 5
	SD (SE)	15 20 (7 60)	3 16 (1 58)	10 79 (5 39)	9 94 (3 51)	12 30 (3 55)
	Min, Max	46, 79	70, 77	49, 72	49, 77	46, 79
Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	1 3	-4 3	3 8	-0 3	0 3
	Median	9 0	-7 0	3 0	-1 0	3 0
	SD (SE)	17 56 (8 78)	10 72 (5 36)	6 65 (3 33)	9 30 (3 29)	11 82 (3 41)
	Min, Max	-25, 12	-13, 10	-3, 12	-13, 12	-25, 12
Percent Change from Baseline to Week 48	n	4	4	4	8	12
	Mean	13 7	-3 7	10 2	3 2	6 7
	Median	21 7	-8 1	6 3	-1 4	6 3
	SD (SE)	25 95 (12 98)	14 45 (7 23)	16 47 (8 23)	16 14 (5 71)	19 39 (5 60)
	Min, Max	-24, 35	-15, 16	-4, 32	-15, 32	-24, 35

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source, P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Week 62	n	4	4	4	8	12
	Mean	49 3	73 3	62 8	68 0	61 8
	Median	50 0	72 5	59 5	72 5	65 5
	SD (SE)	11 03 (5 51)	3 59 (1 80)	26 74 (13 37)	18 53 (6 55)	18 36 (5 30)
	Min, Max	36, 61	70, 78	37, 95	37, 95	36, 95
Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	-6 0	-4 0	6 0	1 0	-1 3
	Median	2 0	-3 5	6 0	5 0	3 0
	SD (SE)	25 42 (12 71)	14 17 (7 08)	13 98 (6 99)	14 08 (4 98)	17 73 (5 12)
	Min, Max	-43, 15	-19, 10	-11, 23	-19, 23	-43, 23
Percent Change from Baseline to Week 62	n	4	4	4	8	12
	Mean	1 7	-2 9	9 1	3 1	2 6
	Median	5 3	-3 3	13 7	7 0	5 8
	SD (SE)	32 46 (16 23)	18 11 (9 05)	23 91 (11 96)	20 65 (7 30)	23 65 (6 83)
	Min, Max	-41, 38	-21, 16	-23, 32	-23, 32	-41, 38

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H20)						
Week 74	n	4	4	4	8	12
	Mean	51 5	83 0	65 0	74 0	66 5
	Median	48 5	84 0	66 0	78 0	72 5
	SD (SE)	19 05 (9 53)	6 32 (3 16)	25 55 (12 77)	19 73 (6 98)	21 67 (6 26)
	Min, Max	32, 77	75, 89	33, 95	33, 95	32, 95
Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	-3 8	5 8	8.3	7 0	3 4
	Median	-0 5	5 5	11 5	8 5	5 0
	SD (SE)	18 82 (9 41)	18 84 (9 42)	19 21 (9 60)	17 66 (6 24)	17 98 (5 19)
	Min, Max	-27, 13	-14, 26	-15, 25	-15, 26	-27, 26
Percent Change from Baseline to Week 74	n	4	4	4	8	12
	Mean	2 6	10 9	17.1	14 0	10 2
	Median	1 9	8 4	16 0	11 8	11 8
	SD (SE)	32 78 (16 39)	27 04 (13 52)	42 42 (21 21)	33 10 (11 70)	31 96 (9 23)
	Min, Max	-26, 33	-16, 43	-31, 68	-31, 68	-31, 68

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Week 84	n	4	4	4	8	12
	Mean	55 5	80 0	70 0	75 0	68 5
	Median	56 5	77 5	68 5	74.5	70 5
	SD (SE)	14 34 (7 17)	11 75 (5 87)	20 77 (10 38)	16 51 (5 84)	17 94 (5 18)
	Min, Max	37, 72	69, 96	47, 96	47, 96	37, 96
Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	0 3	2 8	13 3	8 0	5 4
	Median	5 0	2 5	14 5	12 5	10 5
	SD (SE)	24 80 (12 40)	22 32 (11 16)	13.23 (6 61)	17 89 (6 32)	19 64 (5 67)
	Min, Max	-32, 23	-18, 24	-1, 25	-18, 25	-32, 25
Percent Change from Baseline to Week 84	n	4	4	4	8	12
	Mean	15 7	7 1	26 5	16 8	16 5
	Median	13 0	8 0	20 2	20 0	20 0
	SD (SE)	45 93 (22 96)	29 97 (14 98)	31 22 (15 61)	30 16 (10 66)	33 98 (9 81)
	Min, Max	-31, 68	-21, 33	-2, 68	-21, 68	-31, 68

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H20)						
Week 96	n	4	4	4	8	12
	Mean	52 5	78 8	59 0	68 9	63 4
	Median	51 0	77 0	61 5	74 0	66 5
	SD (SE)	5 45 (2 72)	6 90 (3 45)	21 21 (10 61)	18 02 (6 37)	16 72 (4 83)
	Min, Max	48, 60	73, 88	34, 79	34, 88	34, 88
Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	-2 8	1 5	2 3	1 9	0 3
	Median	10 0	-4 0	5 5	2 5	4 5
	SD (SE)	32 77 (16 38)	17 99 (9 00)	11 32 (5 66)	13 92 (4 92)	20 53 (5 93)
	Min, Max	-51, 20	-13, 27	-14, 12	-14, 27	-51, 27
Percent Change from Baseline to Week 96	n	4	4	4	8	12
	Mean	14 2	5 1	4 7	4 9	8 0
	Median	27 9	-4 4	7 7	3 6	7 7
	SD (SE)	45 40 (22 70)	26 96 (13 48)	25 44 (12 72)	24 27 (8 58)	30 95 (8 93)
	Min, Max	-49, 50	-15, 44	-29, 32	-29, 44	-49, 50

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H2O)						
Week 120	n	4	4	4	8	12
	Mean	63 0	84 5	69 5	77 0	72 3
	Median	63 5	86 5	70 0	81 5	68 5
	SD (SE)	6 16 (3 08)	12 92 (6 46)	19 89 (9 95)	17.48 (6 18)	15 88 (4 58)
	Min, Max	55, 70	67, 98	46, 92	46, 98	46, 98
Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	7 8	7.3	12 8	10 0	9 3
	Median	18 0	12 5	14 0	14 0	16 0
	SD (SE)	28 73 (14 37)	22 56 (11 28)	12 15 (6 07)	17 03 (6 02)	20 27 (5 85)
	Min, Max	-34, 29	-22, 26	-2, 25	-22, 26	-34, 29
Percent Change from Baseline to Week 120	n	4	4	4	8	12
	Mean	35 1	13 0	25 7	19 3	24 6
	Median	44 0	18 6	19 6	19 6	27 8
	SD (SE)	50 95 (25 47)	30 50 (15 25)	30 84 (15 42)	29 19 (10 32)	36 21 (10 45)
	Min, Max	-33, 85	-25, 39	-4, 68	-25, 68	-33, 85

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H20)						
Week 144	n	4	4	4	8	12
	Mean	66 5	83 8	67 0	75.4	72 4
	Median	68 5	84 0	64 0	78 0	75 5
	SD (SE)	17 48 (8 74)	6 70 (3 35)	14 31 (7 15)	13 68 (4 84)	14 88 (4 30)
	Min, Max	44, 85	77, 90	55, 85	55, 90	44, 90
Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	11 3	6 5	10 3	8 4	9 3
	Median	15 0	4 0	10 0	10 0	10 0
	SD (SE)	24 85 (12 43)	19 49 (9 74)	7 37 (3 68)	13 78 (4 87)	17 07 (4 93)
	Min, Max	-19, 34	-10, 28	2, 19	-10, 28	-19, 34
Percent Change from Baseline to Week 144	n	4	4	4	8	12
	Mean	38 6	12 0	21 7	16 9	24 1
	Median	43 7	6 9	16 3	16 3	16 3
	SD (SE)	54 42 (27 21)	28 34 (14 17)	20 80 (10 40)	23.59 (8 34)	35 72 (10 31)
	Min, Max	-18, 85	-11, 46	3, 51	-11, 51	-18, 85

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8.1 1
Summary and Change from Baseline Pulmonary Function Test
ITT Population

Timepoint		Placebo to Eteplirsen (N=4)	Eteplirsen 30 mg/kg for 168 Weeks (N=4)	Eteplirsen 50 mg/kg for 168 Weeks (N=4)	Eteplirsen for 168 Weeks (N=8)	Overall (N=12)
MIP (cm H20)						
Week 168	n	4	4	4	8	12
	Mean	64 8	76 8	68 8	72 8	70 1
	Median	63 5	76 5	71 0	75 5	72 5
	SD (SE)	18 79 (9 39)	11 44 (5 72)	14 10 (7 05)	12 63 (4 47)	14 61 (4 22)
	Min, Max	44, 88	63, 91	50, 83	50, 91	44, 91
Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	9 5	-0 5	12 0	5 8	7 0
	Median	12 0	1 5	12 0	12 0	12 0
	SD (SE)	19 07 (9 54)	20 86 (10 43)	5 77 (2 89)	15 66 (5 54)	16 09 (4 64)
	Min, Max	-16, 30	-24, 19	5, 19	-24, 19	-24, 30
Percent Change from Baseline to Week 168	n	4	4	4	8	12
	Mean	30 4	2 5	24.3	13 4	19 1
	Median	31 0	5 6	25 2	19 9	25 5
	SD (SE)	36 93 (18 46)	27 20 (13 60)	15 56 (7 78)	23 60 (8 34)	28 22 (8 15)
	Min, Max	-15, 75	-28, 26	7, 40	-28, 40	-28, 75

[1] Baseline is last non-missing value before the first dose of study drug on Study Day 1
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_effsum sas, Date/time of run 06MAR2015 14 00
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-6 3 (8 82)			
		30 mg/kg for 168 weeks[1]	-1 7 (8 65)	0 718	4 6 (12 71)	(-20 6, 29 8)
		50 mg/kg for 168 weeks[1]	-2 7 (8 53)	0 772	3 6 (12 47)	(-21 1, 28 4)
	2	Eteplirsen for 144 Weeks[2]	-6 2 (8 55)			
		Eteplirsen for 168 weeks[2]	-2 3 (5 92)	0 715	3 9 (10 69)	(-17 3, 25 1)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-15 1 (8 82)			
		30 mg/kg for 168 weeks[1]	-7 2 (8 65)	0 539	7 8 (12 71)	(-17.4, 33 1)
		50 mg/kg for 168 weeks[1]	-10 7 (8.53)	0 726	4 4 (12 47)	(-20 4, 29 1)
	2	Eteplirsen for 144 Weeks[2]	-14 9 (8 55)			
		Eteplirsen for 168 weeks[2]	-9 0 (5 92)	0 581	5 9 (10 69)	(-15 3, 27 1)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-10 8 (8 82)			
		30 mg/kg for 168 weeks[1]	1 8 (8 65)	0 324	12 6 (12 71)	(-12.6, 37 8)
		50 mg/kg for 168 weeks[1]	-8 9 (8 53)	0 881	1 9 (12 47)	(-22 9, 26 6)
	2	Eteplirsen for 144 Weeks[2]	-10 7 (8 55)			
		Eteplirsen for 168 weeks[2]	-3 7 (5 92)	0 511	7 0 (10 69)	(-14 2, 28 2)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-6 8 (8 82)			
		30 mg/kg for 168 weeks[1]	-5 0 (8 65)	0 885	1 8 (12 71)	(-23 4, 27 1)
		50 mg/kg for 168 weeks[1]	-5 4 (8 53)	0 912	1 4 (12 47)	(-23 4, 26 1)
	2	Eteplirsen for 144 Weeks[2]	-6 7 (8 55)			
		Eteplirsen for 168 weeks[2]	-5 3 (5 92)	0 895	1 4 (10 69)	(-19 8, 22 6)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-7 1 (8 82)			
		30 mg/kg for 168 weeks[1]	-8.5 (8 65)	0 912	-1 4 (12 71)	(-26 6, 23 8)
		50 mg/kg for 168 weeks[1]	4 3 (8 53)	0 364	11 4 (12 47)	(-13 4, 36 1)
	2	Eteplirsen for 144 Weeks[2]	-6 9 (8 55)			
		Eteplirsen for 168 weeks[2]	-2 2 (5 92)	0 655	4 8 (10 69)	(-16 4, 26 0)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-16 3 (8 82)			
		30 mg/kg for 168 weeks[1]	-9 2 (8 65)	0 578	7 1 (12 71)	(-18 1, 32 3)
		50 mg/kg for 168 weeks[1]	-16 2 (8 53)	0 992	0 1 (12 47)	(-24 6, 24 9)
	2	Eteplirsen for 144 Weeks[2]	-16 2 (8 55)			
		Eteplirsen for 168 weeks[2]	-12 8 (5 92)	0 750	3.4 (10 69)	(-17 8, 24 6)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-16 1 (8 82)			
		30 mg/kg for 168 weeks[1]	-11 2 (8 65)	0 704	4 8 (12 71)	(-20 4, 30 1)
		50 mg/kg for 168 weeks[1]	-21 2 (8 53)	0 682	-5 1 (12 47)	(-29 9, 19 6)
	2	Eteplirsen for 144 Weeks[2]	-15 9 (8 55)			
		Eteplirsen for 168 weeks[2]	-16 3 (5 92)	0 975	-0 3 (10 69)	(-21 5, 20 9)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	-3 1 (8 82)			
		30 mg/kg for 168 weeks[1]	-6 0 (8 65)	0 820	-2 9 (12 71)	(-28 1, 22 3)
		50 mg/kg for 168 weeks[1]	-23 4 (8 53)	0 106	-20 4 (12 47)	(-45 1, 4 4)
	2	Eteplirsen for 144 Weeks[2]	-2 9 (8 55)			
		Eteplirsen for 168 weeks[2]	-14 8 (5 92)	0 271	-11 8 (10 69)	(-33 0, 9 4)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	-11 3 (8 82)			
		30 mg/kg for 168 weeks[1]	-13 2 (8 65)	0 881	-1 9 (12 71)	(-27 1, 23 3)
		50 mg/kg for 168 weeks[1]	-13 7 (8 53)	0 849	-2 4 (12 47)	(-27 1, 22 4)
	2	Eteplirsen for 144 Weeks[2]	-11 2 (8 55)			
		Eteplirsen for 168 weeks[2]	-13 5 (5 92)	0 828	-2 3 (10 69)	(-23 5, 18 9)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	-11 8 (8 82)			
		30 mg/kg for 168 weeks[1]	-11 5 (8 65)	0 978	0 3 (12 71)	(-24 9, 25 6)
		50 mg/kg for 168 weeks[1]	-24 7 (8 53)	0 305	-12 9 (12 47)	(-37 6, 11 9)
	2	Eteplirsen for 144 Weeks[2]	-11 7 (8 55)			
		Eteplirsen for 168 weeks[2]	-18 2 (5 92)	0 547	-6 5 (10 69)	(-27 7, 14 7)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13:53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	-11 3 (8 82)			
		30 mg/kg for 168 weeks[1]	-14 2 (8 65)	0 820	-2 9 (12 71)	(-28 1, 22 3)
		50 mg/kg for 168 weeks[1]	-18 2 (8 53)	0 583	-6 9 (12 47)	(-31 6, 17 9)
	2	Eteplirsen for 144 Weeks[2]	-11 2 (8 55)			
		Eteplirsen for 168 weeks[2]	-16 3 (5 92)	0 636	-5 1 (10 69)	(-26 3, 16 1)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-0 145 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 085 (0 1504)	0 266	0 230 (0 2053)	(-0 178, 0 638)
		50 mg/kg for 168 weeks[1]	0 006 (0 1542)	0 481	0 151 (0 2135)	(-0.273, 0 575)
	2	Eteplirsen for 144 Weeks[2]	-0 153 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 049 (0 1003)	0 250	0 202 (0 1745)	(-0 144, 0 548)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-0 220 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 010 (0 1504)	0 266	0 230 (0 2053)	(-0 178, 0 638)
		50 mg/kg for 168 weeks[1]	-0 074 (0 1542)	0 495	0 146 (0 2135)	(-0 278, 0 570)
	2	Eteplirsen for 144 Weeks[2]	-0 228 (0 1422)			
		Eteplirsen for 168 weeks[2]	-0 028 (0 1003)	0 256	0 200 (0 1745)	(-0 147, 0 546)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L.16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-0 180 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 202 (0.1504)	0 066	0 382 (0 2053)	(-0 026, 0 790)
		50 mg/kg for 168 weeks[1]	0 011 (0 1542)	0 373	0 191 (0 2135)	(-0 233, 0 615)
	2	Eteplirsen for 144 Weeks[2]	-0 188 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 110 (0 1003)	0 091	0.298 (0 1745)	(-0 048, 0.645)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-0 133 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 182 (0 1504)	0 129	0 315 (0 2053)	(-0 093, 0 723)
		50 mg/kg for 168 weeks[1]	0 046 (0 1542)	0 405	0 179 (0 2135)	(-0 246, 0 603)
	2	Eteplirsen for 144 Weeks[2]	-0 141 (0 1422)			
		Eteplirsen for 168 weeks[2]	0.118 (0 1003)	0 142	0 258 (0 1745)	(-0 088, 0 605)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-0 108 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 127 (0 1504)	0 256	0 235 (0 2053)	(-0 173, 0 643)
		50 mg/kg for 168 weeks[1]	0 218 (0 1542)	0 130	0 326 (0 2135)	(-0 098, 0.750)
	2	Eteplirsen for 144 Weeks[2]	-0 116 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 177 (0 1003)	0 097	0 292 (0 1745)	(-0 054, 0 638)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-0 208 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 200 (0 1504)	0 050	0 407 (0 2053)	(-0 001, 0 815)
		50 mg/kg for 168 weeks[1]	-0 057 (0 1542)	0 481	0 151 (0 2135)	(-0 273, 0 575)
	2	Eteplirsen for 144 Weeks[2]	-0 216 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 075 (0 1003)	0 099	0.291 (0 1745)	(-0 055, 0 637)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-0 188 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 125 (0 1504)	0 132	0 312 (0 2053)	(-0 096, 0 720)
		50 mg/kg for 168 weeks[1]	-0 112 (0 1542)	0 722	0 076 (0 2135)	(-0 348, 0 500)
	2	Eteplirsen for 144 Weeks[2]	-0 196 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 010 (0 1003)	0 241	0 206 (0 1745)	(-0 140, 0 552)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	0 010 (0 1430)			
		30 mg/kg for 168 ,weeks[1]	0 267 (0 1504)	0.213	0 257 (0 2053)	(-0 151, 0 665)
		50 mg/kg for 168 weeks[1]	-0 117 (0 1542)	0 556	-0 126 (0 2135)	(-0 551, 0 298)
	2	Eteplirsen for 144 Weeks[2]	0 002 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 079 (0 1003)	0 660	0 077 (0 1745)	(-0 269, 0 423)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	-0 090 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 222 (0 1504)	0 132	0 312 (0 2053)	(-0 096, 0 720)
		50 mg/kg for 168 weeks[1]	0 081 (0 1542)	0 425	0 171 (0 2135)	(-0 253, 0 595)
	2	Eteplirsen for 144 Weeks[2]	-0 098 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 155 (0 1003)	0 150	0 253 (0 1745)	(-0 093, 0 600)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	-0 068 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 385 (0 1504)	0 030	0 452 (0 2053)	(0 044, 0 860)
		50 mg/kg for 168 weeks[1]	-0 054 (0 1542)	0 949	0 014 (0 2135)	(-0 411, 0 438)
	2	Eteplirsen for 144 Weeks[2]	-0 076 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 169 (0 1003)	0 164	0 245 (0 1745)	(-0 102, 0 591)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	-0 043 (0 1430)			
		30 mg/kg for 168 weeks[1]	0 280 (0 1504)	0 120	0 322 (0 2053)	(-0 086, 0 730)
		50 mg/kg for 168 weeks[1]	0 091 (0 1542)	0 533	0 134 (0 2135)	(-0 291, 0 558)
	2	Eteplirsen for 144 Weeks[2]	-0 051 (0 1422)			
		Eteplirsen for 168 weeks[2]	0 189 (0 1003)	0 173	0 240 (0 1745)	(-0 107, 0 586)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-0 12 (0 052)			
		30 mg/kg for 168 weeks[1]	0 00 (0 052)	0 116	0 12 (0 073)	(-0 03, 0 26)
		50 mg/kg for 168 weeks[1]	0 04 (0 052)	0 034	0 16 (0 074)	(0.01, 0 30)
	2	Eteplirsen for 144 Weeks[2]	-0 12 (0 051)			
		Eteplirsen for 168 weeks[2]	0 02 (0 036)	0 028	0 14 (0 063)	(0.02, 0 27)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-0 04 (0 052)			
		30 mg/kg for 168 weeks[1]	0 07 (0 052)	0 116	0 12 (0 073)	(-0 03, 0 26)
		50 mg/kg for 168 weeks[1]	-0 01 (0 052)	0 650	0 03 (0 074)	(-0 11, 0 18)
	2	Eteplirsen for 144 Weeks[2]	-0 04 (0 051)			
		Eteplirsen for 168 weeks[2]	0 03 (0 036)	0 221	0.08 (0 063)	(-0 05, 0 20)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-0 07 (0 052)			
		30 mg/kg for 168 weeks[1]	0 00 (0 052)	0 369	0 07 (0 073)	(-0 08, 0 21)
		50 mg/kg for 168 weeks[1]	-0 01 (0 052)	0 429	0 06 (0 074)	(-0 09, 0 20)
	2	Eteplirsen for 144 Weeks[2]	-0 07 (0 051)			
		Eteplirsen for 168 weeks[2]	0 00 (0 036)	0 303	0 07 (0 063)	(-0 06, 0 19)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-0 02 (0 052)			
		30 mg/kg for 168 weeks[1]	0 00 (0 052)	0 828	0 02 (0 073)	(-0 13, 0 16)
		50 mg/kg for 168 weeks[1]	-0 06 (0 052)	0 574	-0 04 (0.074)	(-0 19, 0 10)
	2	Eteplirsen for 144 Weeks[2]	-0 02 (0 051)			
		Eteplirsen for 168 weeks[2]	-0 03 (0 036)	0 877	-0 01 (0 063)	(-0 13, 0 12)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2.8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-0 02 (0 052)			
		30 mg/kg for 168 weeks[1]	-0 03 (0 052)	0 901	-0 01 (0 073)	(-0 15, 0 14)
		50 mg/kg for 168 weeks[1]	-0 01 (0 052)	0 909	0 01 (0 074)	(-0 14, 0 15)
	2	Eteplirsen for 144 Weeks[2]	-0 02 (0 051)			
		Eteplirsen for 168 weeks[2]	-0 02 (0 036)	0 966	0 00 (0 063)	(-0 12, 0 13)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-0 04 (0 052)			
		30 mg/kg for 168 weeks[1]	0 05 (0 052)	0 216	0 09 (0 073)	(-0 05, 0 24)
		50 mg/kg for 168 weeks[1]	-0 01 (0 052)	0 650	0 03 (0 074)	(-0 11, 0 18)
	2	Eteplirsen for 144 Weeks[2]	-0 04 (0 051)			
		Eteplirsen for 168 weeks[2]	0 02 (0 036)	0 303	0 07 (0 063)	(-0 06, 0 19)

[1] LS Mean, standaid error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8.1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	0 13 (0 052)			
		30 mg/kg for 168 weeks[1]	0 12 (0 052)	0 901	-0 01 (0 073)	(-0 15, 0 14)
		50 mg/kg for 168 weeks[1]	0 02 (0 052)	0 117	-0 12 (0 074)	(-0 26, 0 03)
	2	Eteplirsen for 144 Weeks[2]	0 13 (0 051)			
		Eteplirsen for 168 weeks[2]	0 07 (0 036)	0 345	-0 06 (0 063)	(-0 18, 0 07)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	-0 04 (0 052)			
		30 mg/kg for 168 weeks[1]	-0 03 (0 052)	0 828	0 02 (0 073)	(-0 13, 0 16)
		50 mg/kg for 168 weeks[1]	-0 06 (0 052)	0 823	-0 02 (0 074)	(-0 16, 0 13)
	2	Eteplirsen for 144 Weeks[2]	-0 04 (0 051)			
		Eteplirsen for 168 weeks[2]	-0 04 (0 036)	0 966	0 00 (0 063)	(-0 12, 0 13)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8.1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	-0 02 (0 052)			
		30 mg/kg for 168 weeks[1]	-0 03 (0 052)	0 901	-0 01 (0 073)	(-0 15, 0 14)
		50 mg/kg for 168 weeks[1]	-0 13 (0 052)	0 117	-0 12 (0 074)	(-0 26, 0 03)
	2	Eteplirsen for 144 Weeks[2]	-0 02 (0 051)			
		Eteplirsen for 168 weeks[2]	-0 08 (0 036)	0 345	-0.06 (0 063)	(-0 18, 0 07)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	-0 09 (0 052)			
		30 mg/kg for 168 weeks[1]	0 00 (0 052)	0 216	0 09 (0 073)	(-0 05, 0 24)
		50 mg/kg for 168 weeks[1]	-0 08 (0 052)	0 909	0 01 (0 074)	(-0 14, 0 15)
	2	Eteplirsen for 144 Weeks[2]	-0 09 (0 051)			
		Eteplirsen for 168 weeks[2]	-0 04 (0 036)	0 405	0 05 (0 063)	(-0 07, 0 18)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2.6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	-0 07 (0 052)			
		30 mg/kg for 168 weeks[1]	-0 03 (0 052)	0 577	0 04 (0 073)	(-0 10, 0 19)
		50 mg/kg for 168 weeks[1]	-0 08 (0 052)	0 823	-0 02 (0 074)	(-0 16, 0 13)
	2	Eteplirsen for 144 Weeks[2]	-0 07 (0 051)			
		Eteplirsen for 168 weeks[2]	-0 05 (0 036)	0 810	0.02 (0 063)	(-0 11, 0 14)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen,-Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	3 0 (6 95)			
		30 mg/kg for 168 weeks[1]	1 9 (6 57)	0 911	-1 1 (9 89)	(-20 8, 18 5)
		50 mg/kg for 168 weeks[1]	-2 2 (6 60)	0 605	-5 2 (9.96)	(-25 0, 14 6)
	2	Eteplirsen for 144 Weeks[2]	3 0 (6 73)			
		Eteplirsen for 168 weeks[2]	-0 1 (4 60)	0 710	-3 2 (8 50)	(-20 0, 13 7)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-7 2 (6 95)			
		30 mg/kg for 168 weeks[1]	-13 3 (6 57)	0 538	-6 1 (9 89)	(-25 8, 13 5)
		50 mg/kg for 168 weeks[1]	-8 2 (6.60)	0 926	-0 9 (9 96)	(-20 7, 18 9)
	2	Eteplirsen for 144 Weeks[2]	-7 2 (6.73)			
		Eteplirsen for 168 weeks[2]	-10 8 (4 60)	0 677	-3 5 (8 50)	(-20 4, 13 3)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-6 7 (6 95)			
		30 mg/kg for 168 weeks[1]	3 2 (6 57)	0 320	9 9 (9 89)	(-9 8, 29 5)
		50 mg/kg for 168 weeks[1]	-2 2 (6 60)	0 647	4 6 (9 96)	(-15 2, 24 4)
	2	Eteplirsen for 144 Weeks[2]	-6 7 (6 73)			
		Eteplirsen for 168 weeks[2]	0 5 (4 60)	0 399	7 2 (8 50)	(-9 7, 24 1)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-11 2 (6.95)			
		30 mg/kg for 168 weeks[1]	-4 6 (6 57)	0 504	6 6 (9.89)	(-13 0, 26 3)
		50 mg/kg for 168 weeks[1]	1 3 (6 60)	0 210	12 6 (9 96)	(-7 2, 32 4)
	2	Eteplirsen for 144 Weeks[2]	-11 2 (6 73)			
		Eteplirsen for 168 weeks[2]	-1 6 (4 60)	0 263	9 6 (8 50)	(-7 3, 26 4)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-8 5 (6 95)			
		30 mg/kg for 168 weeks[1]	-6 3 (6 57)	0 829	2 1 (9 89)	(-17 5, 21 8)
		50 mg/kg for 168 weeks[1]	6 3 (6 60)	0 140	14 8 (9 96)	(-5 0, 34 6)
	2	Eteplirsen for 144 Weeks[2]	-8 5 (6 73)			
		Eteplirsen for 168 weeks[2]	0 0 (4 60)	0 323	8 5 (8 50)	(-8 4, 25 3)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-16 5 (6 95)			
		30 mg/kg for 168 weeks[1]	-12 1 (6 57)	0 658	4 4 (9 89)	(-15 3, 24 0)
		50 mg/kg for 168 weeks[1]	-11 9 (6 60)	0 647	4 6 (9 96)	(-15 2, 24 4)
	2	Eteplirsen for 144 Weeks[2]	-16 5 (6 73)			
		Eteplirsen for 168 weeks[2]	-12 0 (4 60)	0 602	4 5 (8 50)	(-12 4, 21 3)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-33 7 (6 95)			
		30 mg/kg for 168 weeks[1]	-20 3 (6 57)	0 179	13 4 (9 89)	(-6 3, 33 0)
		50 mg/kg for 168 weeks[1]	-18 7 (6 60)	0 134	15 1 (9 96)	(-4 7, 34 9)
	2	Eteplirsen for 144 Weeks[2]	-33 7 (6 73)			
		Eteplirsen for 168 weeks[2]	-19 5 (4 60)	0 098	14 2 (8 50)	(-2 7, 31 1)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	-2 5 (6 95)			
		30 mg/kg for 168 weeks[1]	-2.6 (6 57)	0 991	-0 1 (9 89)	(-19 8, 19 5)
		50 mg/kg for 168 weeks[1]	-12 4 (6 60)	0 322	-9 9 (9 96)	(-29 7, 9 9)
	2	Eteplirsen for 144 Weeks[2]	-2 5 (6.73)			
		Eteplirsen for 168 weeks[2]	-7 5 (4 60)	0 554	-5 0 (8 50)	(-21 9, 11 8)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	-15 0 (6 95)			
		30 mg/kg for 168 weeks[1]	-11 3 (6 57)	0 714	3 6 (9 89)	(-16 0, 23 3)
		50 mg/kg for 168 weeks[1]	2.1 (6 60)	0 090	17 1 (9 96)	(-2 7, 36 9)
	2	Eteplirsen for 144 Weeks[2]	-15 0 (6 73)			
		Eteplirsen for 168 weeks[2]	-4 6 (4 60)	0 227	10 3 (8 50)	(-6 5, 27 2)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	-8 0 (6 95)			
		30 mg/kg for 168 weeks[1]	-9 3 (6 57)	0 891	-1 4 (9 89)	(-21 0, 18 3)
		50 mg/kg for 168 weeks[1]	-13 7 (6 60)	0 570	-5 7 (9 96)	(-25 5, 14 1)
	2	Eteplirsen for 144 Weeks[2]	-8 0 (6 73)			
		Eteplirsen for 168 weeks[2]	-11 5 (4 60)	0 677	-3 5 (8 50)	(-20 4, 13 3)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	-10 2 (6 95)			
		30 mg/kg for 168 weeks[1]	-11 8 (6 57)	0 871	-1 6 (9 89)	(-21.3, 18 0)
		50 mg/kg for 168 weeks[1]	-5 9 (6 60)	0 665	4 3 (9 96)	(-15 5, 24 1)
	2	Eteplirsen for 144 Weeks[2]	-10 2 (6 73)			
		Eteplirsen for 168 weeks[2]	-8 9 (4 60)	0 876	1 3 (8 50)	(-15 5, 18 2)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-0 008 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 040 (0 1057)	0 744	0 048 (0 1478)	(-0 245, 0 342)
		50 mg/kg for 168 weeks[1]	0 038 (0 1075)	0 761	0 046 (0 1516)	(-0 255, 0 348)
	2	Eteplirsen for 144 Weeks[2]	-0 008 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 039 (0 0727)	0 715	0 046 (0 1263)	(-0 204, 0 297)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-0 218 (0 1047)			
		30 mg/kg for 168 weeks[1]	-0 180 (0 1057)	0 795	0 038 (0 1478)	(-0 255, 0 332)
		50 mg/kg for 168 weeks[1]	-0 017 (0 1075)	0 188	0.201 (0 1516)	(-0 100, 0 503)
	2	Eteplirsen for 144 Weeks[2]	-0 218 (0 1030)			
		Eteplirsen for 168 weeks[2]	-0 099 (0 0727)	0 349	0 119 (0 1263)	(-0 132, 0 369)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-0 121 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 168 (0 1057)	0 054	0 288 (0 1478)	(-0 005, 0 582)
		50 mg/kg for 168 weeks[1]	0 106 (0 1075)	0 139	0 226 (0 1516)	(-0 075, 0 528)
	2	Eteplirsen for 144 Weeks[2]	-0 120 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 136 (0 0727)	0 045	0 256 (0 1263)	(0 006, 0 507)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-0 206 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 125 (0 1057)	0 028	0 331 (0 1478)	(0 037, 0 625)
		50 mg/kg for 168 weeks[1]	0 188 (0 1075)	0 011	0 394 (0 1516)	(0 093, 0 695)
	2	Eteplirsen for 144 Weeks[2]	-0 205 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 156 (0 0727)	0 005	0 361 (0 1263)	(0 111, 0 612)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-0 126 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 110 (0 1057)	0 114	0 236 (0 1478)	(-0 058, 0 530)
		50 mg/kg for 168 weeks[1]	0 311 (0.1075)	0 005	0 436 (0 1516)	(0 135, 0 738)
	2	Eteplirsen for 144 Weeks[2]	-0 125 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 210 (0 0727)	0 009	0 335 (0 1263)	(0 084, 0 586)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-0 211 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 093 (0 1057)	0 043	0 303 (0 1478)	(0 010 0 597)
		50 mg/kg for 168 weeks[1]	0 036 (0 1075)	0 108	0 246 (0 1516)	(-0 055, 0 548)
	2	Eteplirsen for 144 Weeks[2]	-0 210 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 064 (0 0727)	0 033	0 274 (0 1263)	(0 023, 0 524)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-0 488 (0 1047)			
		30 mg/kg for 168 weeks[1]	-0 172 (0 1057)	0 035	0 316 (0 1478)	(0 022, 0 610)
		50 mg/kg for 168 weeks[1]	-0 077 (0 1075)	0 008	0 411 (0 1516)	(0 110, 0 713)
	2	Eteplirsen for 144 Weeks[2]	-0 488 (0 1030)			
		Eteplirsen for 168 weeks[2]	-0 125 (0.0727)	0 005	0 363 (0 1263)	(0 112, 0 613)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	0 037 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 293 (0 1057)	0 087	0 256 (0 1478)	(-0 038, 0 550)
		50 mg/kg for 168 weeks[1]	0 076 (0 1075)	0 799	0 039 (0 1516)	(-0 262, 0 340)
	2	Eteplirsen for 144 Weeks[2]	0 037 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 184 (0 0727)	0 249	0 146 (0 1263)	(-0 104, 0 397)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	-0 148 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 218 (0 1057)	0 015	0 366 (0 1478)	(0 072, 0 660)
		50 mg/kg for 168 weeks[1]	0 376 (0 1075)	< 0 001	0 524 (0 1516)	(0 223, 0 825)
	2	Eteplirsen for 144 Weeks[2]	-0 148 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 296 (0 0727)	< 0 001	0 444 (0 1263)	(0 193, 0.694)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	0 007 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 388 (0 1057)	0 012	0 381 (0 1478)	(0 087, 0 675)
		50 mg/kg for 168 weeks[1]	0 176 (0 1075)	0 269	0 169 (0 1516)	(-0 132, 0 470)
	2	Eteplirsen for 144 Weeks[2]	0 007 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 281 (0 0727)	0 033	0 274 (0 1263)	(0 023, 0 524)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing, L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	-0 008 (0 1047)			
		30 mg/kg for 168 weeks[1]	0 290 (0 1057)	0 046	0 298 (0 1478)	(0 005, 0 592)
		50 mg/kg for 168 weeks[1]	0 333 (0 1075)	0 027	0 341 (0 1516)	(0 040, 0 643)
	2	Eteplirsen for 144 Weeks[2]	-0 008 (0 1030)			
		Eteplirsen for 168 weeks[2]	0 311 (0 0727)	0 013	0 319 (0 1263)	(0 068, 0 569)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-0 7 (6 73)			
		30 mg/kg for 168 weeks[1]	6 3 (6 99)	0 475	7 1 (9 83)	(-12 5, 26 6)
		50 mg/kg for 168 weeks[1]	-0 4 (6 87)	0 972	0 3 (9 56)	(-18 7, 19 3)
	2	Eteplirsen for 144 Weeks[2]	-0 7 (6 60)			
		Eteplirsen for 168 weeks[2]	2 9 (4 66)	0 655	3 6 (8 11)	(-12 4, 19 7)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-1 1 (6 73)			
		30 mg/kg for 168 weeks[1]	-13 3 (6 99)	0 219	-12 2 (9 83)	(-31 7, 7 4)
		50 mg/kg for 168 weeks[1]	2 9 (6 87)	0 673	4 0 (9 56)	(-14 9, 23 0)
	2	Eteplirsen for 144 Weeks[2]	-1 1 (6 60)			
		Eteplirsen for 168 weeks[2]	-5 2 (4 66)	0 612	-4 1 (8 11)	(-20 2, 12 0)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-0 2 (6 73)			
		30 mg/kg for 168 weeks[1]	-4 6 (6 99)	0 656	-4.4 (9 83)	(-23 9, 15 1)
		50 mg/kg for 168 weeks[1]	0 0 (6 87)	0 984	0 2 (9 56)	(-18 8, 19 2)
	2	Eteplirsen for 144 Weeks[2]	-0 1 (6 60)			
		Eteplirsen for 168 weeks[2]	-2 3 (4 66)	0 791	-2 2 (8 11)	(-18 2, 13 9)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-14 2 (6 73)			
		30 mg/kg for 168 weeks[1]	5 4 (6 99)	0 049	19 6 (9 83)	(0 1, 39 1)
		50 mg/kg for 168 weeks[1]	2 2 (6 87)	0.088	16 5 (9 56)	(-2 5, 35 5)
	2	Eteplirsen for 144 Weeks[2]	-14 2 (6 60)			
		Eteplirsen for 168 weeks[2]	3 8 (4 66)	0 029	18 0 (8 11)	(1 9, 34 1)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-13 7 (6 73)			
		30 mg/kg for 168 weeks[1]	-3 3 (6 99)	0 293	10 4 (9 83)	(-9 1, 29 9)
		50 mg/kg for 168 weeks[1]	-5 7 (6 87)	0 407	8 0 (9 56)	(-11 0, 26 9)
	2	Eteplirsen for 144 Weeks[2]	-13 7 (6 60)			
		Eteplirsen for 168 weeks[2]	-4 5 (4 66)	0 263	9 1 (8 11)	(-7 0, 25 2)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-3 4 (6 73)			
		30 mg/kg for 168 weeks[1]	-1 8 (6 99)	0 867	1 7 (9 83)	(-17 9, 21 2)
		50 mg/kg for 168 weeks[1]	1 4 (6 87)	0 610	4 9 (9 56)	(-14 1, 23 9)
	2	Eteplirsen for 144 Weeks[2]	-3 4 (6 60)			
		Eteplirsen for 168 weeks[2]	-0 2 (4 66)	0 692	3 2 (8 11)	(-12 9, 19 3)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-10 6 (6 73)			
		30 mg/kg for 168 weeks[1]	-13 2 (6 99)	0 788	-2 6 (9 83)	(-22 2, 16 9)
		50 mg/kg for 168 weeks[1]	1 7 (6 87)	0 203	12 2 (9 56)	(-6 7, 31 2)
	2	Eteplirsen for 144 Weeks[2]	-10 5 (6 60)			
		Eteplirsen for 168 weeks[2]	-5 8 (4 66)	0 560	4 7 (8 11)	(-11 3, 20 8)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	-0 8 (6 73)			
		30 mg/kg for 168 weeks[1]	0 0 (6 99)	0 931	0 9 (9 83)	(-18 7, 20 4)
		50 mg/kg for 168 weeks[1]	-11 6 (6 87)	0 262	-10 8 (9 56)	(-29 8, 8 2)
	2	Eteplirsen for 144 Weeks[2]	-0 8 (6 60)			
		Eteplirsen for 168 weeks[2]	-5 8 (4 66)	0 537	-5 0 (8 11)	(-21 1, 11 1)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates. A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	1 5 (6 73)			
		30 mg/kg for 168 weeks[1]	0 7 (6.99)	0 929	-0 9 (9 83)	(-20 4, 18 6)
		50 mg/kg for 168 weeks[1]	-1 9 (6 87)	0 717	-3 5 (9 56)	(-22 5, 15 5)
	2	Eteplirsen for 144 Weeks[2]	1 6 (6 60)			
		Eteplirsen for 168 weeks[2]	-0 7 (4 66)	0 783	-2 2 (8 11)	(-18 3, 13 8)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	-4 8 (6 73)			
		30 mg/kg for 168 weeks[1]	0 6 (6 99)	0 586	5 4 (9 83)	(-14 2, 24 9)
		50 mg/kg for 168 weeks[1]	-6 3 (6 87)	0 874	-1 5 (9 56)	(-20 5, 17 5)
	2	Eteplirsen for 144 Weeks[2]	-4 8 (6 60)			
		Eteplirsen for 168 weeks[2]	-2 9 (4 66)	0 818	1 9 (8 11)	(-14 2, 17.9)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30.0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	-3 3 (6 73)			
		30 mg/kg for 168 weeks[1]	-5 9 (6 99)	0 793	-2 6 (9 83)	(-22 1, 16 9)
		50 mg/kg for 168 weeks[1]	-5 7 (6 87)	0 803	-2 4 (9 56)	(-21 4, 16 6)
	2	Eteplirsen for 144 Weeks[2]	-3 3 (6 60)			
		Eteplirsen for 168 weeks[2]	-5 8 (4 66)	0 754	-2 5 (8 11)	(-18 6, 13 5)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standaid error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H20)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-0 7 (6 51)			
		30 mg/kg for 168 weeks[1]	6 7 (6 87)	0 444	7 4 (9 65)	(-11 7, 26 6)
		50 mg/kg for 168 weeks[1]	0 2 (6 67)	0 924	0 9 (9 22)	(-17 4, 19 2)
	2	Eteplirsen for 144 Weeks[2]	-0 4 (6 39)			
		Eteplirsen for 168 weeks[2]	3 3 (4 51)	0 635	3 7 (7 85)	(-11 8, 19 3)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	1 3 (6 51)			
		30 mg/kg for 168 weeks[1]	-9 8 (6 87)	0 254	-11 1 (9 65)	(-30 2, 8 1)
		50 mg/kg for 168 weeks[1]	3 4 (6 67)	0 818	2 1 (9 22)	(-16 2, 20 4)
	2	Eteplirsen for 144 Weeks[2]	1 6 (6 39)			
		Eteplirsen for 168 weeks[2]	-3 3 (4 51)	0 535	-4 9 (7 85)	(-20 5, 10 7)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standaid error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	2 6 (6 51)			
		30 mg/kg for 168 weeks[1]	-0 8 (6 87)	0 731	-3 3 (9 65)	(-22 5, 15.8)
		50 mg/kg for 168 weeks[1]	2 4 (6 67)	0 990	-0 1 (9 22)	(-18 4, 18 2)
	2	Eteplirsen for 144 Weeks[2]	2 8 (6 39)			
		Eteplirsen for 168 weeks[2]	0 7 (4 51)	0 786	-2 1 (7 85)	(-17 7, 13 4)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-8 4 (6.51)			
		30 mg/kg for 168 weeks[1]	9 7 (6 87)	0 063	18 2 (9 65)	(-1 0, 37 3)
		50 mg/kg for 168 weeks[1]	4 7 (6 67)	0 158	13 1 (9 22)	(-5 2, 31 4)
	2	Eteplirsen for 144 Weeks[2]	-8 2 (6.39)			
		Eteplirsen for 168 weeks[2]	7 1 (4 51)	0 055	15 2 (7 85)	(-0 3, 30 8)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2.6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-7 2 (6.51)			
		30 mg/kg for 168 weeks[1]	3 2 (6 87)	0 283	10 4 (9 65)	(-8 7, 29 6)
		50 mg/kg for 168 weeks[1]	-1 1 (6 67)	0.508	6 1 (9 22)	(-12 2, 24 4)
	2	Eteplirsen for 144 Weeks[2]	-6.9 (6 39)			
		Eteplirsen for 168 weeks[2]	1 0 (4 51)	0 319	7 9 (7 85)	(-7 7, 23 4).
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	4 1 (6 51)			
		30 mg/kg for 168 weeks[1]	5 5 (6.87)	0 883	1 4 (9 65)	(-17 7, 20 6)
		50 mg/kg for 168 weeks[1]	6 2 (6 67)	0 818	2 1 (9 22)	(-16 2, 20 4)
	2	Eteplirsen for 144 Weeks[2]	4 3 (6 39)			
		Eteplirsen for 168 weeks[2]	5 7 (4 51)	0 863	1 4 (7 85)	(-14 2, 16 9)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-2 4 (6 51)			
		30 mg/kg for 168 weeks[1]	-4 8 (6.87)	0 810	-2 3 (9 65)	(-21 5, 16 8)
		50 mg/kg for 168 weeks[1]	7 2 (6 67)	0 299	9 6 (9 22)	(-8 7, 27 9)
	2	Eteplirsen for 144 Weeks[2]	-2 2 (6 39)			
		Eteplirsen for 168 weeks[2]	1 1 (4 51)	0 681	3 2 (7 85)	(-12 3, 18 8)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	7 6 (6 51)			
		30 mg/kg for 168 weeks[1]	9 5 (6 87)	0 842	1 9 (9 65)	(-17 2, 21 1)
		50 mg/kg for 168 weeks[1]	-4 3 (6 67)	0 201	-11 9 (9 22)	(-30 2, 6 4)
	2	Eteplirsen for 144 Weeks[2]	7.8 (6 39)			
		Eteplirsen for 168 weeks[2]	2 5 (4 51)	0 494	-5 4 (7 85)	(-21 0, 10 2)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	11 3 (6 51)			
		30 mg/kg for 168 weeks[1]	12 5 (6 87)	0 903	1 2 (9 65)	(-18 0, 20 3)
		50 mg/kg for 168 weeks[1]	6 7 (6 67)	0 617	-4.6 (9 22)	(-22 9, 13 7)
	2	Eteplirsen for 144 Weeks[2]	11 6 (6 39)			
		Eteplirsen for 168 weeks[2]	9 5 (4 51)	0 786	-2 1 (7 85)	(-17.7, 13 4)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	7.6 (6 51)			
		30 mg/kg for 168 weeks[1]	15 0 (6 87)	0 444	7 4 (9 65)	(-11.7, 26 6)
		50 mg/kg for 168 weeks[1]	3 7 (6 67)	0 676	-3 9 (9 22)	(-22 2, 14 4)
	2	Eteplirsen for 144 Weeks[2]	7 8 (6 39)			
		Eteplirsen for 168 weeks[2]	9 2 (4 51)	0 863	1 4 (7 85)	(-14 2, 16 9)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	10 8 (6 51)			
		30 mg/kg for 168 weeks[1]	10 2 (6 87)	0 952	-0 6 (9 65)	(-19 7, 18 6)
		50 mg/kg for 168 weeks[1]	6 4 (6 67)	0 637	-4 4 (9 22)	(-22 7, 13 9)
	2	Eteplirsen for 144 Weeks[2]	11 1 (6 39)			
		Eteplirsen for 168 weeks[2]	8 2 (4 51)	0.714	-2 9 (7 85)	(-18 5, 12 7)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-15 6 (9 32)			
		30 mg/kg for 168 weeks[1]	4 5 (9 41)	0 137	20 2 (13 43)	(-6 5, 46 8)
		50 mg/kg for 168 weeks[1]	-17 2 (9 29)	0 903	-1 6 (13 17)	(-27 8, 24 6)
	2	Eteplirsen for 144 Weeks[2]	-15 4 (9 13)			
		Eteplirsen for 168 weeks[2]	-6 4 (6 41)	0 426	9 0 (11 26)	(-13 3, 31 3)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-9 3 (9 32)			
		30 mg/kg for 168 weeks[1]	0 2 (9.41)	0 481	9 5 (13 43)	(-17 2, 36 2)
		50 mg/kg for 168 weeks[1]	-13 6 (9 29)	0 745	-4 3 (13 17)	(-30 5, 21 9)
	2	Eteplirsen for 144 Weeks[2]	-9 1 (9 13)			
		Eteplirsen for 168 weeks[2]	-6 7 (6 41)	0 836	2 3 (11 26)	(-20 0, 24 7)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8.1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-11 0 (9 32)			
		30 mg/kg for 168 weeks[1]	0 7 (9 41)	0 386	11 7 (13 43)	(-15 0, 38 4)
		50 mg/kg for 168 weeks[1]	-14 2 (9 29)	0 808	-3 2 (13 17)	(-29 4, 22 9)
	2	Eteplirsen for 144 Weeks[2]	-10 8 (9 13)			
		Eteplirsen for 168 weeks[2]	-6 9 (6 41)	0 725	4 0 (11 26)	(-18 4, 26 3)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-10 6 (9 32)			
		30 mg/kg for 168 weeks[1]	-1 9 (9 41)	0 520	8 7 (13 43)	(-18 0, 35 3)
		50 mg/kg for 168 weeks[1]	-1 3 (9 29)	0 483	9 3 (13 17)	(-16 9, 35 4)
	2	Eteplirsen for 144 Weeks[2]	-10 4 (9 13)			
		Eteplirsen for 168 weeks[2]	-1 7 (6 41)	0 441	8 7 (11 26)	(-13 6, 31 0)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing. L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-21 6 (9 32)			
		30 mg/kg for 168 weeks[1]	-2 3 (9 41)	0 156	19 2 (13 43)	(-7 4, 45 9)
		50 mg/kg for 168 weeks[1]	-0 3 (9 29)	0 110	21 3 (13 17)	(-4 9, 47 4)
	2	Eteplirsen for 144 Weeks[2]	-21 4 (9 13)			
		Eteplirsen for 168 weeks[2]	-1 4 (6 41)	0 079	20 0 (11 26)	(-2 4, 42 3)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-19 3 (9 32)			
		30 mg/kg for 168 weeks[1]	9 9 (9 41)	0 032	29 2 (13 43)	(2 6, 55 9)
		50 mg/kg for 168 weeks[1]	3 7 (9 29)	0 084	23 0 (13 17)	(-3 1, 49 2)
	2	Eteplirsen for 144 Weeks[2]	-19 1 (9 13)			
		Eteplirsen for 168 weeks[2]	6 7 (6 41)	0 024	25 9 (11 26)	(3 5, 48 2)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-13 7 (9 32)			
		30 mg/kg for 168 weeks[1]	5 6 (9 41)	0 153	19 3 (13 43)	(-7 3, 46 0)
		50 mg/kg for 168 weeks[1]	9 6 (9 29)	0.080	23 3 (13 17)	(-2 9, 49 5)
	2	Eteplirsen for 144 Weeks[2]	-13 5 (9 13)			
		Eteplirsen for 168 weeks[2]	7 5 (6 41)	0 065	21 0 (11 26)	(-1 3, 43 4)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	-17 5 (9 32)			
		30 mg/kg for 168 weeks[1]	2 8 (9 41)	0 135	20 3 (13 43)	(-6 4, 46 9)
		50 mg/kg for 168 weeks[1]	-6 8 (9 29)	0 417	10 7 (13 17)	(-15 4, 36 9)
	2	Eteplirsen for 144 Weeks[2]	-17 3 (9 13)			
		Eteplirsen for 168 weeks[2]	-2 1 (6 41)	0.179	15 2 (11 26)	(-7 1, 37 6)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	-4 0 (9 32)			
		30 mg/kg for 168 weeks[1]	8 3 (9 41)	0 362	12 3 (13.43)	(-14 4, 39 0)
		50 mg/kg for 168 weeks[1]	6 2 (9 29)	0 438	10 3 (13 17)	(-15 9, 36 4)
	2	Eteplirsen for 144 Weeks[2]	-3 9 (9 13)			
		Eteplirsen for 168 weeks[2]	7 2 (6 41)	0 330	11 0 (11 26)	(-11 3, 33 4)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	-0 4 (9 32)			
		30 mg/kg for 168 weeks[1]	4 8 (9 41)	0 700	5.2 (13 43)	(-21 5, 31 9)
		50 mg/kg for 168 weeks[1]	2 0 (9 29)	0 859	2 3 (13 17)	(-23 8, 28 5)
	2	Eteplirsen for 144 Weeks[2]	-0 2 (9 13)			
		Eteplirsen for 168 weeks[2]	3 3 (6 41)	0 757	3 5 (11 26)	(-18 8, 25 8)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	-3 9 (9 32)			
		30 mg/kg for 168 weeks[1]	-4 7 (9 41)	0 955	-0 8 (13 43)	(-27 4, 25 9)
		50 mg/kg for 168 weeks[1]	1 9 (9 29)	0 659	5 8 (13 17)	(-20 3, 32 0)
	2	Eteplirsen for 144 Weeks[2]	-3 7 (9 13)			
		Eteplirsen for 168 weeks[2]	-1 5 (6 41)	0 841	2 3 (11 26)	(-20 1, 24 6)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L.16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H20)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	-9 1 (6 17)			
		30 mg/kg for 168 weeks[1]	5 1 (6 25)	0 112	14 3 (8 88)	(-3 4, 31 9)
		50 mg/kg for 168 weeks[1]	-11 5 (6 17)	0 786	-2 4 (8 72)	(-19 7, 14 9)
	2	Eteplirsen for 144 Weeks[2]	-8 9 (6 15)			
		Eteplirsen for 168 weeks[2]	-3 3 (4 33)	0 465	5 6 (7 57)	(-9 5, 20 6)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	-3 9 (6 17)			
		30 mg/kg for 168 weeks[1]	3 1 (6 25)	0 432	7 0 (8 88)	(-10 6, 24 6)
		50 mg/kg for 168 weeks[1]	-8 0 (6 17)	0 637	-4 1 (8 72)	(-21 4, 13 2)
	2	Eteplirsen for 144 Weeks[2]	-3 6 (6 15)			
		Eteplirsen for 168 weeks[2]	-2 6 (4 33)	0 890	1 1 (7 57)	(-14 0, 16 1)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	-3 9 (6 17)			
		30 mg/kg for 168 weeks[1]	3 4 (6 25)	0 416	7 3 (8 88)	(-10 4, 24 9)
		50 mg/kg for 168 weeks[1]	-8 5 (6 17)	0 597	-4 6 (8 72)	(-21 9, 12 7)
	2	Eteplirsen for 144 Weeks[2]	-3 6 (6 15)			
		Eteplirsen for 168 weeks[2]	-2 7 (4 33)	0 903	0 9 (7 57)	(-14 1, 15 9)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	-3 9 (6 17)			
		30 mg/kg for 168 weeks[1]	3 9 (6 25)	0 384	7 8 (8 88)	(-9 9, 25 4)
		50 mg/kg for 168 weeks[1]	0 7 (6 17)	0 597	4 6 (8 72)	(-12 7, 21 9)
	2	Eteplirsen for 144 Weeks[2]	-3 6 (6 15)			
		Eteplirsen for 168 weeks[2]	2 2 (4 33)	0 445	5 8 (7 57)	(-9 2, 20 8)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H20)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	-11 1 (6.17)			
		30 mg/kg for 168 weeks[1]	4 1 (6 25)	0 089	15 3 (8 88)	(-2 4, 32 9)
		50 mg/kg for 168 weeks[1]	3 0 (6 17)	0 109	14 1 (8 72)	(-3 2, 31 4)
	2	Eteplirsen for 144 Weeks[2]	-10 9 (6 15)			
		Eteplirsen for 168 weeks[2]	3 4 (4 33)	0 062	14 3 (7 57)	(-0 7, 29 3)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	-8 9 (6 17)			
		30 mg/kg for 168 weeks[1]	13 9 (6 25)	0 012	22 8 (8 88)	(5 1, 40 4)
		50 mg/kg for 168 weeks[1]	5 2 (6.17)	0 109	14 1 (8 72)	(-3 2, 31 4)
	2	Eteplirsen for 144 Weeks[2]	-8 6 (6 15)			
		Eteplirsen for 168 weeks[2]	9 4 (4 33)	0 019	18 1 (7 57)	(3 0, 33 1)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates. A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	-4 9 (6 17)			
		30 mg/kg for 168 weeks[1]	10 9 (6 25)	0 079	15 8 (8 88)	(-1 9, 33 4)
		50 mg/kg for 168 weeks[1]	10 2 (6.17)	0 086	15 1 (8 72)	(-2 2, 32 4)
	2	Eteplirsen for 144 Weeks[2]	-4 6 (6 15)			
		Eteplirsen for 168 weeks[2]	10 4 (4 33)	0 050	15 1 (7 57)	(0 0, 30 1)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	-7 9 (6 17)			
		30 mg/kg for 168 weeks[1]	9 6 (6 25)	0 052	17 5 (8 88)	(-0 1, 35 1)
		50 mg/kg for 168 weeks[1]	-0 8 (6 17)	0.416	7 1 (8 72)	(-10 2, 24 4)
	2	Eteplirsen for 144 Weeks[2]	-7 6 (6 15)			
		Eteplirsen for 168 weeks[2]	4 3 (4 33)	0 118	11 9 (7 57)	(-3 1, 26 9)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	2 6 (6 17)			
		30 mg/kg for 168 weeks[1]	15 4 (6 25)	0 154	12 8 (8 88)	(-4 9, 30 4)
		50 mg/kg for 168 weeks[1]	9 7 (6 17)	0 416	7 1 (8 72)	(-10 2, 24 4)
	2	Eteplirsen for 144 Weeks[2]	2 9 (6 15)			
		Eteplirsen for 168 weeks[2]	12 4 (4 33)	0 210	9 6 (7 57)	(-5 5, 24 6)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	6 1 (6 17)			
		30 mg/kg for 168 weeks[1]	14 6 (6 25)	0 340	8 5 (8 88)	(-9 1, 26 1)
		50 mg/kg for 168 weeks[1]	7 2 (6 17)	0 898	1 1 (8 72)	(-16 2, 18 4)
	2	Eteplirsen for 144 Weeks[2]	6 4 (6 15)			
		Eteplirsen for 168 weeks[2]	10 8 (4 33)	0 560	4 4 (7 57)	(-10 6, 19 4)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 2
Analysis of Change from Baseline for Pulmonary Function Test
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	4 4 (6 17)			
		30 mg/kg for 168 weeks[1]	7 6 (6 25)	0 714	3 3 (8 88)	(-14 4, 20 9)
		50 mg/kg for 168 weeks[1]	9 0 (6 17)	0 597	4 6 (8 72)	(-12 7, 21 9)
	2	Eteplirsen for 144 Weeks[2]	4 6 (6 15)			
		Eteplirsen for 168 weeks[2]	8 2 (4 33)	0 640	3 6 (7 57)	(-11 5, 18 6)

[1] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
[2] LS Mean, standard error and p-value are from a restricted maximum likelihood-based mixed model repeated measures with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks), time and treatment-by-time interaction terms as fixed effects, subject nested in treatment as a random effect with baseline value and time since DMD Diagnosis as covariates A spatial power covariance structure is used
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb sas, Date/time of run 06MAR2015 13 53
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	5 6 (1 95)			
		30 mg/kg for 168 weeks[1]	7 7 (1 86)	0.488	2 1 (2 88)	(-4 7, 8 9)
		50 mg/kg for 168 weeks[1]	6 1 (1 80)	0 857	0 5 (2 75)	(-6 0, 7 0)
	2	Eteplirsen for 144 Weeks[2]	5 7 (1 87)			
		Eteplirsen for 168 weeks[2]	6.9 (1 25)	0.624	1 2 (2 41)	(-4 3, 6 8)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	6.1 (1 70)			
		30 mg/kg for 168 weeks[1]	7 4 (1 62)	0 619	1 3 (2 50)	(-4 6, 7 2)
		50 mg/kg for 168 weeks[1]	6 0 (1 56)	0 989	0 0 (2 40)	(-5 7, 5 6)
	2	Eteplirsen for 144 Weeks[2]	6 1 (1 63)			
		Eteplirsen for 168 weeks[2]	6 7 (1 09)	0 795	0.6 (2 09)	(-4 3, 5 4)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing 1 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	5 5 (1 53)			
		30 mg/kg for 168 weeks[1]	8 9 (1 46)	0 180	3 4 (2 26)	(-2 0, 8 7)
		50 mg/kg for 168 weeks[1]	5 1 (1.41)	0 828	-0 5 (2 16)	(-5 6, 4 6)
	2	Eteplirsen for 144 Weeks[2]	5 7 (1 77)			
		Eteplirsen for 168 weeks[2]	6 9 (1 18)	0 602	1 2 (2 27)	(-4 0, 6 5)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	6 7 (2 25)			
		30 mg/kg for 168 weeks[1]	5 8 (2 15)	0 802	-0 9 (3 33)	(-8 7, 7 0)
		50 mg/kg for 168 weeks[1]	6 9 (2 08)	0 943	0 2 (3 18)	(-7 3, 7 8)
	2	Eteplirsen for 144 Weeks[2]	6 7 (2 13)			
		Eteplirsen for 168 weeks[2]	6 4 (1 42)	0 928	-0 3 (2 74)	(-6 6, 6 1)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	4 7 (1 92)			
		30 mg/kg for 168 weeks[1]	6 0 (1 83)	0 676	1 2 (2 83)	(-5 5, 7 9)
		50 mg/kg for 168 weeks[1]	8 8 (1 76)	0 173	4 1 (2 71)	(-2 3, 10 5)
	2	Eteplirsen for 144 Weeks[2]	4 6 (1 95)			
		Eteplirsen for 168 weeks[2]	7 4 (1 31)	0 293	2 8 (2 51)	(-3 0, 8 6)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	6 3 (1 64)			
		30 mg/kg for 168 weeks[1]	7 9 (1 57)	0 523	1 6 (2 42)	(-4 1, 7 4)
		50 mg/kg for 168 weeks[1]	5 3 (1 51)	0 687	-1 0 (2 32)	(-6 5, 4 5)
	2	Eteplirsen for 144 Weeks[2]	6 4 (1 69)			
		Eteplirsen for 168 weeks[2]	6 6 (1 13)	0 933	0 2 (2 17)	(-4 8, 5 2)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	6 4 (1 78)			
		30 mg/kg for 168 weeks[1]	7 0 (1 70)	0 822	0 6 (2 63)	(-5 6, 6.8)
		50 mg/kg for 168 weeks[1]	6 0 (1 64)	0 872	-0 4 (2 51)	(-6 4, 5 5)
	2	Eteplirsen for 144 Weeks[2]	6 5 (1 69)			
		Eteplirsen for 168 weeks[2]	6 5 (1 13)	0 985	0 0 (2 17)	(-5 0, 5 0)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	8 6 (1 89)			
		30 mg/kg for 168 weeks[1]	6 3 (1 80)	0 442	-2 3 (2 79)	(-8 9, 4 3)
		50 mg/kg for 168 weeks[1]	4 6 (1 74)	0 179	-4 0 (2 67)	(-10 3, 2 3)
	2	Eteplirsen for 144 Weeks[2]	8 6 (1 82)			
		Eteplirsen for 168 weeks[2]	5 4 (1 22)	0 208	-3 2 (2 34)	(-8 6, 2 2)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	6 6 (2 08)			
		30 mg/kg for 168 weeks[1]	5 6 (1 98)	0 743	-1 0 (3 07)	(-8 3, 6 2)
		50 mg/kg for 168 weeks[1]	7 3 (1 92)	0 837	0 6 (2.94)	(-6 3, 7 6)
	2	Eteplirsen for 144 Weeks[2]	6 6 (2 00)			
		Eteplirsen for 168 weeks[2]	6 5 (1 34)	0 964	-0 1 (2 57)	(-6 0, 5 8)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	7 0 (2 29)			
		30 mg/kg for 168 weeks[1]	7 4 (2 18)	0 912	0 4 (3 38)	(-7 6, 8 4)
		50 mg/kg for 168 weeks[1]	5 1 (2 11)	0 583	-1 9 (3 23)	(-9 5, 5 8)
	2	Eteplirsen for 144 Weeks[2]	7 1 (2 22)			
		Eteplirsen for 168 weeks[2]	6 2 (1 49)	0 772	-0 9 (2.86)	(-7 4, 5 7)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	7 6 (1 80)			
		30 mg/kg for 168 weeks[1]	6 6 (1 71)	0 715	-1 0 (2 65)	(-7 3, 5 3)
		50 mg/kg for 168 weeks[1]	5 3 (1 65)	0 395	-2 3 (2 54)	(-8 3, 3 7)
	2	Eteplirsen for 144 Weeks[2]	7 6 (1 71)			
		Eteplirsen for 168 weeks[2]	5 9 (1 15)	0 457	-1 7 (2 20)	(-6 8, 3 4)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Reference listing L 16.2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	4 445 (1 8398)			
		30 mg/kg for 168 weeks[1]	7 599 (2 0563)	0 280	3 154 (2 6944)	(-3 217, 9 525)
		50 mg/kg for 168 weeks[1]	7 457 (2 1614)	0 338	3 012 (2 9309)	(-3 918, 9 943)
	2	Eteplirsen for 144 Weeks[2]	4 439 (1 7167)			
		Eteplirsen for 168 weeks[2]	7 531 (1 2073)	0 182	3 092 (2 1138)	(-1 783, 7 966)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	5 172 (2 0858)			
		30 mg/kg for 168 weeks[1]	8 540 (2 3311)	0 307	3 368 (3 0546)	(-3 855, 10 591)
		50 mg/kg for 168 weeks[1]	5 788 (2 4503)	0 858	0 615 (3 3227)	(-7 242, 8 472)
	2	Eteplirsen for 144 Weeks[2]	5 062 (2 0183)			
		Eteplirsen for 168 weeks[2]	7 219 (1 4194)	0 411	2 157 (2 4852)	(-3 574, 7 887)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	3 637 (1 6869)			
		30 mg/kg for 168 weeks[1]	9 446 (1 8854)	0 051	5 809 (2 4705)	(-0 032, 11 651)
		50 mg/kg for 168 weeks[1]	6 417 (1 9818)	0 335	2 781 (2 6874)	(-3 574, 9 135)
	2	Eteplirsen for 144 Weeks[2]	3.516 (1 6805)			
		Eteplirsen for 168 weeks[2]	7 992 (1 1819)	0 062	4 476 (2 0693)	(-0 296, 9 248)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	3 807 (1 8606)			
		30 mg/kg for 168 weeks[1]	8 635 (2 0795)	0 120	4 828 (2 7248)	(-1 615, 11 272)
		50 mg/kg for 168 weeks[1]	7 059 (2 1858)	0 309	3 252 (2 9641)	(-3 757, 10 261)
	2	Eteplirsen for 144 Weeks[2]	3 744 (1 7628)			
		Eteplirsen for 168 weeks[2]	7 878 (1 2397)	0 093	4 135 (2 1705)	(-0 871, 9 140)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	4 100 (1 7334)			
		30 mg/kg for 168 weeks[1]	6 876 (1 9373)	0.310	2 776 (2 5385)	(-3 226, 8 779)
		50 mg/kg for 168 weeks[1]	8 524 (2 0364)	0 153	4 425 (2 7614)	(-2 105, 10 954)
	2	Eteplirsen for 144 Weeks[2]	4 165 (1 6487)			
		Eteplirsen for 168 weeks[2]	7 667 (1 1595)	0 123	3 502 (2 0301)	(-1 180, 8 183)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	4 433 (1 8610)			
		30 mg/kg for 168 weeks[1]	9 111 (2 0800)	0 130	4 678 (2 7255)	(-1 766, 11 123)
		50 mg/kg for 168 weeks[1]	5 955 (2 1864)	0 623	1.522 (2 9648)	(-5 488, 8 533)
	2	Eteplirsen for 144 Weeks[2]	4 307 (1 8417)			
		Eteplirsen for 168 weeks[2]	7 596 (1 2952)	0 185	3 289 (2 2677)	(-1 940, 8 518)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	3 756 (1 8343)			
		30 mg/kg for 168 weeks[1]	8 189 (2 0500)	0 143	4.433 (2 6863)	(-1 919, 10 785)
		50 mg/kg for 168 weeks[1]	7 555 (2 1549)	0 235	3 799 (2 9221)	(-3 111, 10 709)
	2	Eteplirsen for 144 Weeks[2]	3 731 (1 7157)			
		Eteplirsen for 168 weeks[2]	7 885 (1 2067)	0 085	4 154 (2 1126)	(-0 718, 9 025)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	5 658 (2 0513)			
		30 mg/kg for 168 weeks[1]	8 803 (2 2926)	0 330	3 145 (3 0041)	(-3 959, 10 249)
		50 mg/kg for 168 weeks[1]	5 040 (2 4099)	0 855	-0 618 (3 2679)	(-8 345, 7 109)
	2	Eteplirsen for 144 Weeks[2]	5 508 (2 0491)			
		Eteplirsen for 168 weeks[2]	6 996 (1 4411)	0 571	1 489 (2 5231)	(-4 330, 7 307)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	4 084 (1 9805)			
		30 mg/kg for 168 weeks[1]	7 833 (2 2135)	0 237	3 749 (2 9004)	(-3 109, 10 608)
		50 mg/kg for 168 weeks[1]	7 583 (2 3267)	0 304	3 500 (3 1551)	(-3 961, 10 960)
	2	Eteplirsen for 144 Weeks[2]	4 074 (1 8484)			
		Eteplirsen for 168 weeks[2]	7 713 (1 2999)	0 148	3 639 (2 2759)	(-1 609, 8 888)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	3 890 (1 5693)			
		30 mg/kg for 168 weeks[1]	9 658 (1 7539)	0 040	5 768 (2 2982)	(0 334, 11 202)
		50 mg/kg for 168 weeks[1]	5 951 (1 8436)	0 437	2 061 (2 5000)	(-3 850, 7 973)
	2	Eteplirsen for 144 Weeks[2]	3 742 (1.6321)			
		Eteplirsen for 168 weeks[2]	7 879 (1 1478)	0 074	4 136 (2 0096)	(-0 498, 8 771)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16.2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1 (L)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	3 672 (1 5977)			
		30 mg/kg for 168 weeks[1]	9 900 (1 7856)	0 032	6 227 (2 3398)	(0 695, 11 760)
		50 mg/kg for 168 weeks[1]	5.928 (1 8769)	0 405	2 255 (2 5452)	(-3 763, 8 274)
	2	Eteplirsen for 144 Weeks[2]	3 514 (1 6790)			
		Eteplirsen for 168 weeks[2]	7 993 (1 1808)	0 062	4 479 (2 0674)	(-0 288, 9 246)

Note: Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	4 55 (1 483)			
		30 mg/kg for 168 weeks[1]	7 15 (1 451)	0 247	2 60 (2 058)	(-2 27, 7 47)
		50 mg/kg for 168 weeks[1]	7 80 (1 512)	0 182	3 24 (2 186)	(-1 93, 8 41)
	2	Eteplirsen for 144 Weeks[2]	4 58 (1 394)			
		Eteplirsen for 168 weeks[2]	7 46 (0 974)	0 134	2 88 (1 728)	(-1 10, 6 87)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	6 10 (2 036)			
		30 mg/kg for 168 weeks[1]	8 11 (1 991)	0 501	2 00 (2 826)	(-4 68, 8 68)
		50 mg/kg for 168 weeks[1]	5 29 (2 075)	0 793	-0 82 (3 001)	(-7 91, 6 28)
	2	Eteplirsen for 144 Weeks[2]	5 99 (2 025)			
		Eteplirsen for 168 weeks[2]	6 75 (1 414)	0 769	0 76 (2 510)	(-5 02, 6 55)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	5 32 (1 531)			
		30 mg/kg for 168 weeks[1]	7 44 (1 498)	0 353	2 12 (2 125)	(-2 91, 7 14)
		50 mg/kg for 168 weeks[1]	6 74 (1 561)	0 552	1 41 (2 257)	(-3 93, 6 75)
	2	Eteplirsen for 144 Weeks[2]	5 30 (1 441)			
		Eteplirsen for 168 weeks[2]	7 10 (1 006)	0 342	1 81 (1 786)	(-2 31, 5.92)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	6 30 (1 427)			
		30 mg/kg for 168 weeks[1]	6 93 (1 395)	0 759	0 63 (1 980)	(-4 05, 5 31)
		50 mg/kg for 168 weeks[1]	6 27 (1 454)	0 987	-0 03 (2 103)	(-5 01, 4 94)
	2	Eteplirsen for 144 Weeks[2]	6 27 (1 342)			
		Eteplirsen for 168 weeks[2]	6 61 (0 938)	0 844	0 34 (1 664)	(-3 50, 4 18)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	6 99 (1 313)			
		30 mg/kg for 168 weeks[1]	6 15 (1 284)	0 657	-0 85 (1 822)	(-5 15, 3 46)
		50 mg/kg for 168 weeks[1]	6 36 (1 338)	0.752	-0 64 (1 936)	(-5 21, 3 94)
	2	Eteplirsen for 144 Weeks[2]	7 00 (1 227)			
		Eteplirsen for 168 weeks[2]	6 25 (0 857)	0 634	-0 75 (1 521)	(-4 26, 2 75)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	6 07 (2 035)			
		30 mg/kg for 168 weeks[1]	7 51 (1 991)	0 627	1 43 (2 825)	(-5 24, 8 11)
		50 mg/kg for 168 weeks[1]	5 92 (2 075)	0 962	-0 15 (3 000)	(-7 24, 6 95)
	2	Eteplirsen for 144 Weeks[2]	6 01 (1 940)			
		Eteplirsen for 168 weeks[2]	6 75 (1 355)	0 766	0 74 (2 405)	(-4 81, 6 29)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	7 17 (0 279)			
		30 mg/kg for 168 weeks[1]	7 41 (0 273)	0 558	0 24 (0 388)	(-0 68, 1 16)
		50 mg/kg for 168 weeks[1]	4 92 (0 285)	< 0 001	-2 25 (0 412)	(-3 22, -1 28)
	2	Eteplirsen for 144 Weeks[2]	7 07 (0 667)			
		Eteplirsen for 168 weeks[2]	6 22 (0 466)	0 332	-0 85 (0 827)	(-2 76, 1 05)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	6 26 (1 588)			
		30 mg/kg for 168 weeks[1]	6 54 (1 553)	0 906	0 27 (2 204)	(-4 94, 5 48)
		50 mg/kg for 168 weeks[1]	6 70 (1 618)	0 858	0 44 (2 341)	(-5 10, 5 97)
	2	Eteplirsen for 144 Weeks[2]	6 27 (1 483)			
		Eteplirsen for 168 weeks[2]	6 61 (1 036)	0 857	0.34 (1 839)	(-3 90, 4 58)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13.56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	6 29 (1 110)			
		30 mg/kg for 168 weeks[1]	7 31 (i 086)	0 530	1 02 (1 540)	(-2 62, 4 66)
		50 mg/kg for 168 weeks[1]	5 90 (1 131)	0 819	-0 39 (1.636)	(-4 26 3 48)
	2	Eteplirsen for 144 Weeks[2]	6 23 (1 093)			
		Eteplirsen for 168 weeks[2]	6 63 (0 764)	0 776	0 40 (1 355)	(-2 73, 3 52)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	5 87 (1 390)			
		30 mg/kg for 168 weeks[1]	8 51 (1 359)	0 214	2 64 (1 929)	(-1 92, 7 20)
		50 mg/kg for 168 weeks[1]	5 12 (1 417)	0 724	-0 75 (2 049)	(-5 60, 4 09)
	2	Eteplirsen for 144 Weeks[2]	5 74 (1 544)			
		Eteplirsen for 168 weeks[2]	6 88 (1 079)	0 566	1 15 (1 914)	(-3 27, 5 56)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run. 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FEV1/FVC Ratio						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	6 15 (1 467)			
		30 mg/kg for 168 weeks[1]	7 59 (1 435)	0 503	1 44 (2 036)	(-3 38, 6 25)
		50 mg/kg for 168 weeks[1]	5 76 (1 495)	0 864	-0 38 (2 163)	(-5 50, 4 73)
	2	Eteplirsen for 144 Weeks[2]	6 07 (1 442)			
		Eteplirsen for 168 weeks[2]	6 71 (1 007)	0 730	0 64 (1 787)	(-3 48, 4 76)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	5 6 (2 82)			
		30 mg/kg for 168 weeks[1]	8 0 (2 31)	0 586	2 3 (4 06)	(-7 3, 11 9)
		50 mg/kg for 168 weeks[1]	5 9 (2 36)	0 956	0 2 (4 15)	(-9 6, 10 0)
	2	Eteplirsen for 144 Weeks[2]	5 6 (2 72)			
		Eteplirsen for 168 weeks[2]	7 0 (1 70)	0 722	1 4 (3 67)	(-7 1, 9 8)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	6 4 (2 38)			
		30 mg/kg for 168 weeks[1]	5 8 (1 95)	0 875	-0 6 (3 43)	(-8 7, 7 5)
		50 mg/kg for 168 weeks[1]	7 3 (1 99)	0.792	1 0 (3 50)	(-7 3, 9 2)
	2	Eteplirsen for 144 Weeks[2]	6 4 (2 28)			
		Eteplirsen for 168 weeks[2]	6 5 (1 42)	0 964	0 1 (3 08)	(-7 0, 7 2)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	5.4 (2 10)			
		30 mg/kg for 168 weeks[1]	8 3 (1 72)	0 374	2 9 (3 02)	(-4 3, 10 0)
		50 mg/kg for 168 weeks[1]	5 8 (1 76)	0 911	0 4 (3 09)	(-6 9, 7 7)
	2	Eteplirsen for 144 Weeks[2]	5 4 (2 12)			
		Eteplirsen for 168 weeks[2]	7 1 (1 33)	0 567	1 7 (2 87)	(-4 9, 8 3)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	6 0 (2 14)			
		30 mg/kg for 168 weeks[1]	5 5 (1 76)	0 882	-0 5 (3 09)	(-7 8, 6 8)
		50 mg/kg for 168 weeks[1]	8 0 (1 80)	0 541	2 0 (3 16)	(-5 4, 9 5)
	2	Eteplirsen for 144 Weeks[2]	6 0 (2 16)			
		Eteplirsen for 168 weeks[2]	6 7 (1 35)	0 822	0 7 (2 92)	(-6 1, 7 4)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L.16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	5 0 (2 12)			
		30 mg/kg for 168 weeks[1]	5 6 (1 74)	0 868	0 5 (3 06)	(-6 7, 7 8)
		50 mg/kg for 168 weeks[1]	8 9 (1 78)	0 252	3 9 (3 12)	(-3 5, 11 3)
	2	Eteplirsen for 144 Weeks[2]	5 1 (2 26)			
		Eteplirsen for 168 weeks[2]	7 2 (1 41)	0 516	2 1 (3 06)	(-5 0, 9 1)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	7 7 (0 80)			
		30 mg/kg for 168 weeks[1]	6 8 (0 66)	0 427	-1 0 (1 15)	(-3 7, 1 8)
		50 mg/kg for 168 weeks[1]	5 0 (0 67)	0 053	-2 7 (1 18)	(-5 5, 0 0)
	2	Eteplirsen for 144 Weeks[2]	7 7 (0 94)			
		Eteplirsen for 168 weeks[2]	5 9 (0 59)	0 198	-1 8 (1 27)	(-4 7, 1 1)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13.56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	3 9 (1 75)			
		30 mg/kg for 168 weeks[1]	7 1 (1 43)	0 247	3 2 (2 52)	(-2 8, 9 1)
		50 mg/kg for 168 weeks[1]	8 6 (1 47)	0 110	4 7 (2 57)	(-1 4, 10 8)
	2	Eteplirsen for 144 Weeks[2]	3 9 (1 71)			
		Eteplirsen for 168 weeks[2]	7 8 (1 07)	0 131	3 9 (2 31)	(-1 4, 9 2)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	5 4 (2 81)			
		30 mg/kg for 168 weeks[1]	7 7 (2 31)	0 588	2 3 (4 05)	(-7 3, 11 9)
		50 mg/kg for 168 weeks[1]	6 3 (2 36)	0 830	0 9 (4 14)	(-8 9, 10 7)
	2	Eteplirsen for 144 Weeks[2]	5 4 (2 67)			
		Eteplirsen for 168 weeks[2]	7 1 (1 67)	0 656	1 7 (3 61)	(-6 6, 10 0)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	4 1 (2 22)			
		30 mg/kg for 168 weeks[1]	6 0 (1 82)	0 578	1 9 (3 21)	(-5 7, 9 5)
		50 mg/kg for 168 weeks[1]	9 4 (1 87)	0 152	5 3 (3 28)	(-2.5, 13 0)
	2	Eteplirsen for 144 Weeks[2]	4 2 (2 35)			
		Eteplirsen for 168 weeks[2]	7 6 (1 47)	0 311	3 4 (3 18)	(-3 9, 10 8)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	7 3 (2 80)			
		30 mg/kg for 168 weeks[1]	6 5 (2 30)	0 847	-0 8 (4 04)	(-10.4, 8 8)
		50 mg/kg for 168 weeks[1]	5 6 (2 35)	0 686	-1 7 (4 13)	(-11 5, 8 0)
	2	Eteplirsen for 144 Weeks[2]	7 3 (2 64)			
		Eteplirsen for 168 weeks[2]	6 1 (1 65)	0 738	-1 2 (3 57)	(-9 5, 7 0)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	6 6 (2 57)			
		30 mg/kg for 168 weeks[1]	5 3 (2 11)	0 736	-1 3 (3 70)	(-10 1, 7 5)
		50 mg/kg for 168 weeks[1]	7 6 (2 15)	0 790	1 0 (3 78)	(-7 9, 10 0)
	2	Eteplirsen for 144 Weeks[2]	6 6 (2 52)			
		Eteplirsen for 168 weeks[2]	6 4 (1 57)	0 950	-0 2 (3 41)	(-8 1, 7 6)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	6 238 (2.0128)			
		30 mg/kg for 168 weeks[1]	8 345 (2 1226)	0 478	2 108 (2 8112)	(-4 540, 8 755)
		50 mg/kg for 168 weeks[1]	4 917 (2 3158)	0 695	-1 320 (3 2366)	(-8 974, 6 333)
	2	Eteplirsen for 144 Weeks[2]	5 989 (1 9964)			
		Eteplirsen for 168 weeks[2]	6 755 (1 3991)	0 764	0.766 (2.4667)	(-4 922, 6 454)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	4.913 (2 0563)			
		30 mg/kg for 168 weeks[1]	6 531 (2 1685)	0 591	1 618 (2 8720)	(-5 173, 8 409)
		50 mg/kg for 168 weeks[1]	8 056 (2 3658)	0 374	3 143 (3 3065)	(-4 676, 10 962)
	2	Eteplirsen for 144 Weeks[2]	5 024 (1 9342)			
		Eteplirsen for 168 weeks[2]	7 238 (1 3555)	0 381	2 215 (2 3898)	(-3 296, 7 726)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015.13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	3 648 (1 6761)			
		30 mg/kg for 168 weeks[1]	9 243 (1 7675)	0 048	5.595 (2 3409)	(0 060, 11 131)
		50 mg/kg for 168 weeks[1]	6 610 (1 9284)	0 308	2 962 (2 6951)	(-3 411, 9 335)
	2	Eteplirsen for 144 Weeks[2]	3 457 (1 6470)			
		Eteplirsen for 168 weeks[2]	8 022 (1 1542)	0 055	4 565 (2 0349)	(-0 128, 9 257)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	3 039 (1 6194)			
		30 mg/kg for 168 weeks[1]	7 427 (1 7078)	0 094	4.388 (2 2618)	(-0 961, 9 736)
		50 mg/kg for 168 weeks[1]	9 033 (1 8632)	0 055	5 994 (2 6040)	(-0 164, 12 151)
	2	Eteplirsen for 144 Weeks[2]	3 156 (1 5389)			
		Eteplirsen for 168 weeks[2]	8 172 (1 0785)	0 030	5 016 (1 9014)	(0 632, 9 401)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	3 544 (1 4019)			
		30 mg/kg for 168 weeks[1]	7 444 (1 4784)	0 087	3 900 (1 9580)	(-0 730, 8 530)
		50 mg/kg for 168 weeks[1]	8 512 (1 6129)	0 063	4 968 (2 2543)	(-0 363, 10 298)
	2	Eteplirsen for 144 Weeks[2]	3 622 (1 3196)			
		Eteplirsen for 168 weeks[2]	7 939 (0 9248)	0 029	4 318 (1 6305)	(0 558 8 077)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	3 547 (1 5589)			
		30 mg/kg for 168 weeks[1]	7 875 (1 6439)	0 087	4 327 (2 1772)	(-0 821, 9 476)
		50 mg/kg for 168 weeks[1]	8 078 (1 7935)	0 114	4 530 (2 5067)	(-1 397, 10 458)
	2	Eteplirsen for 144 Weeks[2]	3 562 (1 4476)			
		Eteplirsen for 168 weeks[2]	7 969 (1 0145)	0.039	4 407 (1 7886)	(0 282, 8 531)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	3 495 (1 5200)			
		30 mg/kg for 168 weeks[1]	7 265 (1 6029)	0 119	3 770 (2 1229)	(-1 250, 8 790)
		50 mg/kg for 168 weeks[1]	8 739 (1 7488)	0 069	5 244 (2 4441)	(-0 535, 11 024)
	2	Eteplirsen for 144 Weeks[2]	3 602 (1 4430)			
		Eteplirsen for 168 weeks[2]	7 949 (1 0112)	0 041	4 347 (1 7829)	(0 236, 8 458)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	3 695 (1 3254)			
		30 mg/kg for 168 weeks[1]	8 622 (1 3977)	0 032	4 927 (1 8511)	(0 550, 9 305)
		50 mg/kg for 168 weeks[1]	7 183 (1 5249)	0 146	3 488 (2 1312)	(-1 551, 8 528)
	2	Eteplirsen for 144 Weeks[2]	3 59C (1 2652)			
		Eteplirsen for 168 weeks[2]	7 955 (0 8867)	0 023	4 364 (1 5632)	(0 760, 7 969)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	2 689 (1 3663)			
		30 mg/kg for 168 weeks[1]	6 524 (1 4408)	0 084	3 835 (1 9082)	(-0 677, 8 347)
		50 mg/kg for 168 weeks[1]	10 286 (1 5719)	0 011	7 597 (2 1970)	(2 402, 12 792)
	2	Eteplirsen for 144 Weeks[2]	2 962 (1 4848)			
		Eteplirsen for 168 weeks[2]	8 269 (1 0405)	0 020	5 307 (1 8345)	(1 077, 9 538)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	3 354 (1 3630)			
		30 mg/kg for 168 weeks[1]	8 446 (1.4374)	0 032	5 092 (1 9037)	(0 591, 9 594)
		50 mg/kg for 168 weeks[1]	7 700 (1 5682)	0 088	4 346 (2 1917)	(-0 836, 9 529)
	2	Eteplirsen for 144 Weeks[2]	3 300 (1 2744)			
		Eteplirsen for 168 weeks[2]	8.100 (0 8931)	0 016	4 800 (1 5746)	(1 169, 8 431)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
FVC (L)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	3 272 (1 7754)			
		30 mg/kg for 168 weeks[1]	7 304 (1 8722)	0 148	4 032 (2 4796)	(-1 832, 9 895)
		50 mg/kg for 168 weeks[1]	8 924 (2 0426)	0 088	5 652 (2 8548)	(-1.098, 12 403)
	2	Eteplirsen for 144 Weeks[2]	3 389 (1 6812)			
		Eteplirsen for 168 weeks[2]	8 055 (1 1782)	0 055	4 666 (2 0772)	(-0 124, 9 456)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	5 9 (1 49)			
		30 mg/kg for 168 weeks[1]	7 2 (1 75)	0 624	1 2 (2 42)	(-4 5, 7 0)
		50 mg/kg for 168 weeks[1]	6 4 (1 63)	0 850	0 4 (2 14)	(-4 6, 5 5)
	2	Eteplirsen for 144 Weeks[2]	6.0 (1 39)			
		Eteplirsen for 168 weeks[2]	6 8 (0 97)	0 673	0 8 (1 73)	(-3 2, 4 7)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	7 4 (0 95)			
		30 mg/kg for 168 weeks[1]	5 3 (1 12)	0 233	-2 0 (1 54)	(-5 7, 1 6)
		50 mg/kg for 168 weeks[1]	6 8 (1 04)	0 685	-0 6 (1 36)	(-3 8, 2 6)
	2	Eteplirsen for 144 Weeks[2]	7 3 (0 92)			
		Eteplirsen for 168 weeks[2]	6 1 (0 65)	0 340	-1 2 (1 15)	(-3 8, 1 5)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2.6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	6 4 (1 06)			
		30 mg/kg for 168 weeks[1]	6 8 (1 25)	0 827	0 4 (1 73)	(-3 7, 4 5)
		50 mg/kg for 168 weeks[1]	6 3 (1 16)	0 936	-0 1 (1 53)	(-3 7, 3 5)
	2	Eteplirsen for 144 Weeks[2]	6 4 (0 99)			
		Eteplirsen for 168 weeks[2]	6 5 (0 69)	0 947	0 1 (1 23)	(-2 8, 2 9)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	4 6 (1 37)			
		30 mg/kg for 168 weeks[1]	8 0 (1 61)	0 174	3 4 (2 23)	(-1 9, 8 6)
		50 mg/kg for 168 weeks[1]	6 8 (1 50)	0 303	2 2 (1 97)	(-2 5, 6 8)
	2	Eteplirsen for 144 Weeks[2]	4 7 (1 29)			
		Eteplirsen for 168 weeks[2]	7 4 (0 90)	0 135	2 7 (1 61)	(-1 0, 6 4)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	5 1 (1 21)			
		30 mg/kg for 168 weeks[1]	8 1 (1 42)	0 177	3 0 (1 97)	(-1 7, 7 6)
		50 mg/kg for 168 weeks[1]	6 3 (1 32)	0 539	1 1 (1 74)	(-3 0, 5 2)
	2	Eteplirsen for 144 Weeks[2]	5 3 (1 18)			
		Eteplirsen for 168 weeks[2]	7 1 (0 82)	0 236	1 9 (1 46)	(-1 5, 5 2)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	6 3 (1 59)			
		30 mg/kg for 168 weeks[1]	6 0 (1 88)	0 904	-0 3 (2 59)	(-6 5, 5 8)
		50 mg/kg for 168 weeks[1]	7 2 (1 74)	0 691	1 0 (2 29)	(-4 5, 6 4)
	2	Eteplirsen for 144 Weeks[2]	6 2 (1 50)			
		Eteplirsen for 168 weeks[2]	6 6 (1 05)	0 825	0 4 (1 87)	(-3 9, 4 7)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6.8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	6 3 (1 18)			
		30 mg/kg for 168 weeks[1]	3 7 (1 39)	0 221	-2 6 (1 92)	(-7 1, 2 0)
		50 mg/kg for 168 weeks[1]	9 6 (1 29)	0.093	3 3 (1 70)	(-0 7, 7 3)
	2	Eteplirsen for 144 Weeks[2]	5 9 (1 59)			
		Eteplirsen for 168 weeks[2]	6 8 (1 11)	0 666	0 9 (1 97)	(-3.7, 5 4)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	6 6 (1 27)			
		30 mg/kg for 168 weeks[1]	8 1 (1 50)	0 507	1 4 (2 07)	(-3 4, 6 3)
		50 mg/kg for 168 weeks[1]	4 8 (1 39)	0 346	-1 8 (1 83)	(-6 2, 2 5)
	2	Eteplirsen for 144 Weeks[2]	6.8 (1 34)			
		Eteplirsen for 168 weeks[2]	6 3 (0 94)	0 774	-0 5 (1 67)	(-4 3, 3 4)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean standaid error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	6 2 (1 08)			
		30 mg/kg for 168 weeks[1]	7 3 (1 27)	0.550	1 1 (1 75)	(-3.0, 5 2)
		50 mg/kg for 168 weeks[1]	6 1 (1 18)	0 949	-0 1 (1 55)	(-3 8, 3 6)
	2	Eteplirsen for 144 Weeks[2]	6 2 (1 03)			
		Eteplirsen for 168 weeks[2]	6 6 (0 72)	0 767	0 4 (1 28)	(-2 6, 3 3)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	6 2 (1 46)			
		30 mg/kg for 168 weeks[1]	8 0 (1 73)	0 493	1 7 (2 38)	(-3 9, 7 4)
		50 mg/kg for 168 weeks[1]	5 3 (1 60)	0 671	-0 9 (2 11)	(-5 9, 4 0)
	2	Eteplirsen for 144 Weeks[2]	6 4 (1 46)			
		Eteplirsen for 168 weeks[2]	6 6 (1 02)	0 933	0 2 (1.81)	(-4 0, 4 3)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	6 6 (1 39)			
		30 mg/kg for 168 weeks[1]	7 3 (1 63)	0 758	0 7 (2 25)	(-4 6, 6 1)
		50 mg/kg for 168 weeks[1]	5 6 (1 51)	0 627	-1 0 (1 99)	(-5 7, 3 7)
	2	Eteplirser for 144 Weeks[2]	6 7 (1 33)			
		Eteplirsen for 168 weeks[2]	6 4 (0 93)	0 860	-0 3 (1 65)	(-4 1, 3.5)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13:56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	5 7 (1 36)			
		30 mg/kg for 168 weeks[1]	7 9 (1 67)	0 376	2 2 (2 31)	(-3 3, 7 6)
		50 mg/kg for 168 weeks[1]	6 0 (1 50)	0 879	0 3 (1 94)	(-4 3, 4 9)
	2	Eteplirsen for 144 Weeks[2]	5 8 (1 31)			
		Eteplirsen for 168 weeks[2]	6 8 (0.91)	0 549	1 0 (1 63)	(-2 7, 4 8)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	7 3 (1 02)			
		30 mg/kg for 168 weeks[1]	5 6 (1 26)	0 346	-1.8 (1 73)	(-5 9, 2 3)
		50 mg/kg for 168 weeks[1]	6 6 (1 13)	0 617	-0 8 (1 46)	(-4 2, 2 7)
	2	Eteplirsen for 144 Weeks[2]	7 3 (0 96)			
		Eteplirsen for 168 weeks[2]	6 1 (0 67)	0 371	-1 1 (1 20)	(-3 9, 1 6)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H20)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	6 2 (1 10)			
		30 mg/kg for 168 weeks[1]	7 2 (1 34)	0.605	1 0 (1 86)	(-3 4, 5 4)
		50 mg/kg for 168 weeks[1]	6 1 (1 21)	0 926	-0 1 (1 56)	(-3 8, 3.5)
	2	Eteplirsen for 144 Weeks[2]	6 3 (1 04)			
		Eteplirsen for 168 weeks[2]	6 6 (0 72)	0 828	0 3 (1 29)	(-2 7, 3.3)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	4 6 (1 91)			
		30 mg/kg for 168 weeks[1]	7 2 (2 34)	0 446	2 6 (3 23)	(-5 0, 10.2)
		50 mg/kg for 168 weeks[1]	7 7 (2 11)	0 300	3 0 (2 71)	(-3 4, 9 5)
	2	Eteplirsen for 144 Weeks[2]	4 6 (1 77)			
		Eteplirsen for 168 weeks[2]	7 5 (1 23)	0 227	2.9 (2 20)	(-2 2, 7 9)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing: L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	5.2 (1 45)			
		30 mg/kg for 168 weeks[1]	7 8 (1 78)	0.329	2 6 (2 45)	(-3 2, 8 4)
		50 mg/kg for 168 weeks[1]	6 5 (1 60)	0 560	1 3 (2 06)	(-3 6, 6 1)
	2	Eteplirsen for 144 Weeks[2]	5 3 (1 36)			
		Eteplirsen for 168 weeks[2]	7 1 (0 95)	0 329	1 8 (1 70)	(-2 1, 5 7)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	5 8 (1 85)			
		30 mg/kg for 168 weeks[1]	6.0 (2 27)	0 949	0 2 (3 14)	(-7 2, 7 6)
		50 mg/kg for 168 weeks[1]	7 7 (2 05)	0 485	1 9 (2 64)	(-4 3, 8 2)
	2	Eteplirsen for 144 Weeks[2]	5 6 (1 74)			
		Eteplirsen for 168 weeks[2]	6 9 (1 21)	0 571	1 3 (2 17)	(-3 7, 6 3)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	6 0 (1 34)			
		30 mg/kg for 168 weeks[1]	4 1 (1 64)	0 420	-1 9 (2.27)	(-7 3, 3 4)
		50 mg/kg for 168 weeks[1]	9 4 (1 48)	0 121	3 4 (1 91)	(-1 1, 7 9)
	2	Eteplirsen for 144 Weeks[2]	5 6 (1 59)			
		Eteplirsen for 168 weeks[2]	6 9 (1 10)	0 514	1 3 (1 97)	(-3 2, 5 9)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	6 7 (1 45)			
		30 mg/kg for 168 weeks[1]	8 7 (1 78)	0 435	2 0 (2 46)	(-3 8, 7 9)
		50 mg/kg for 168 weeks[1]	4 0 (1 61)	0 237	-2 7 (2 07)	(-7 6, 2 2)
	2	Eteplirsen for 144 Weeks[2]	7 1 (1 60)			
		Eteplirsen for 168 weeks[2]	6 2 (1 12)	0 671	-0 9 (2 00)	(-5 5, 3 7)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	6 2 (1 31)			
		30 mg/kg for 168 weeks[1]	7 8 (1 60)	0 504	1 6 (2 22)	(-3 7, 6 8)
		50 mg/kg for 168 weeks[1]	5 5 (1 45)	0 714	-0 7 (1 86)	(-5 1, 3 7)
	2	Eteplirsen for 144 Weeks[2]	6 4 (1 28)			
		Eteplirsen for 168 weeks[2]	6 6 (0 89)	0 926	0.2 (1 60)	(-3 5, 3 8)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	6 0 (1.79)			
		30 mg/kg for 168 weeks[1]	8 6 (2 19)	0 410	2 7 (3 03)	(-4 5, 9 8)
		50 mg/kg for 168 weeks[1]	4 9 (1 97)	0 684	-1 1 (2 54)	(-7 1, 4 9)
	2	Eteplirsen for 144 Weeks[2]	6 3 (1 79)			
		Eteplirsen for 168 weeks[2]	6 6 (1 25)	0 883	0 3 (2 23)	(-4 8, 5 5)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MEP (cm H2O)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	6 7 (1 47)			
		30 mg/kg for 168 weeks[1]	7 6 (1 80)	0 708	1 0 (2 49)	(-4.9, 6 9)
		50 mg/kg for 168 weeks[1]	5 2 (1 63)	0 507	-1 5 (2 09)	(-6 4, 3 5)
	2	Eteplirsen for 144 Weeks[2]	6 9 (1 43)			
		Eteplirsen for 168 weeks[2]	6 3 (1 00)	0 771	-0 5 (1 78)	(-4 7, 3 6)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	6 5 (1 42)			
		30 mg/kg for 168 weeks[1]	7 9 (1 46)	0 511	1 5 (2 11)	(-3 5, 6 5)
		50 mg/kg for 168 weeks[1]	5 1 (1 40)	0 522	-1 3 (2 00)	(-6 1, 3 4)
	2	Eteplirsen for 144 Weeks[2]	6 6 (1 49)			
		Eteplirsen for 168 weeks[2]	6 5 (1 03)	0 960	-0 1 (1 86)	(-4.4, 4 2)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	7 2 (0 90)			
		30 mg/kg for 168 weeks[1]	7 7 (0 93)	0 736	0 5 (1 34)	(-2 7, 3 7)
		50 mg/kg for 168 weeks[1]	4 7 (0 89)	0 086	-2 5 (1 27)	(-5 5, 0 5)
	2	Eteplirsen for 144 Weeks[2]	7 3 (1 11)			
		Eteplirsen for 168 weeks[2]	6 1 (0 77)	0.415	-1 2 (1 39)	(-4 4, 2 0)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	6 0 (1 67)			
		30 mg/kg for 168 weeks[1]	7 9 (1 71)	0 460	1 9 (2 48)	(-3 9, 7 8)
		50 mg/kg for 168 weeks[1]	5 6 (1 65)	0 862	-0 4 (2.35)	(-6 0, 5 1)
	2	Eteplirsen for 144 Weeks[2]	6 1 (1 66)			
		Eteplirsen for 168 weeks[2]	6 7 (1 15)	0 769	0 6 (2 07)	(-4 2, 5 4)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	6 2 (0 47)			
		30 mg/kg for 168 weeks[1]	6 4 (0 48)	0 754	0 2 (0.69)	(-1 4, 1 9)
		50 mg/kg for 168 weeks[1]	6 9 (0 46)	0.299	0 7 (0 66)	(-0 8, 2.3)
	2	Eteplirsen for 144 Weeks[2]	6 2 (0 45)			
		Eteplirsen for 168 weeks[2]	6 7 (0 31)	0 394	0 5 (0 57)	(-0 8, 1 8)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	5 0 (1 64)			
		30 mg/kg for 168 weeks[1]	6 6 (1 68)	0 550	1 5 (2 44)	(-4 2, 7 3)
		50 mg/kg for 168 weeks[1]	7 9 (1 62)	0 247	2 9 (2 31)	(-2 5, 8 4)
	2	Eteplirsen for 144 Weeks[2]	5 0 (1 57)			
		Eteplirsen for 168 weeks[2]	7 3 (1 08)	0 274	2 3 (1.96)	(-2 2, 6 8)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	3 9 (1 37)			
		30 mg/kg for 168 weeks[1]	7 7 (1 41)	0 105	3 8 (2 05)	(-1 0, 8 6)
		50 mg/kg for 168 weeks[1]	8 0 (1 36)	0 070	4 1 (1 94)	(-0 4, 8 7)
	2	Eteplirsen for 144 Weeks[2]	3 8 (1 29)			
		Eteplirsen for 168 weeks[2]	7 8 (0 89)	0 038	4 0 (1 61)	(0 3, 7 7)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	4 3 (1 37)			
		30 mg/kg for 168 weeks[1]	7 1 (1 40)	0.215	2 8 (2 03)	(-2 0, 7 6)
		50 mg/kg for 168 weeks[1]	8 0 (1 35)	0 097	3 7 (1 92)	(-0 9, 8 2)
	2	Eteplirsen for 144 Weeks[2]	4 3 (1 29)			
		Eteplirsen for 168 weeks[2]	7 6 (0 90)	0 077	3 3 (1 62)	(-0 5, 7 0)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	6 1 (1 31)			
		30 mg/kg for 168 weeks[1]	7 5 (1 34)	0 490	1 4 (1 95)	(-3 2, 6 0)
		50 mg/kg for 168 weeks[1]	5 9 (1 29)	0 896	-0 2 (1 84)	(-4 6, 4 1)
	2	Eteplirsen for 144 Weeks[2]	6 2 (1 28)			
		Eteplirsen for 168 weeks[2]	6 7 (0 89)	0 766	0 5 (1 61)	(-3 2, 4 2)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	5 5 (1 26)			
		30 mg/kg for 168 weeks[1]	7 4 (1 29)	0 346	1 9 (1 87)	(-2 5, 6 3)
		50 mg/kg for 168 weeks[1]	6 7 (1 24)	0 515	1 2 (1 77)	(-3 0, 5 4)
	2	Eteplirsen for 144 Weeks[2]	5 5 (1 18)			
		Eteplirsen for 168 weeks[2]	7 0 (0 82)	0 336	1 5 (1 48)	(-1 9, 4 9)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	6 2 (1 49)			
		30 mg/kg for 168 weeks[1]	7 2 (1 53)	0 693	0 9 (2 22)	(-4 3, 6 2)
		50 mg/kg for 168 weeks[1]	6 1 (1 47)	0 952	-0 1 (2 10)	(-5 1, 4 8)
	2	Eteplirsen for 144 Weeks[2]	6 3 (1 41)			
		Eteplirsen for 168 weeks[2]	6 6 (0 98)	0 855	0 3 (1 77)	(-3 7, 4 4)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP % of Predicted Value (%)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	6 8 (1 83)			
		30 mg/kg for 168 weeks[1]	6 5 (1 88)	0 918	-0 3 (2 72)	(-6 7, 6 1)
		50 mg/kg for 168 weeks[1]	6 2 (1 81)	0 821	-0 6 (2 58)	(-6 7, 5 5)
	2	Eteplirsen for 144 Weeks[2]	6.8 (1 71)			
		Eteplirsen for 168 weeks[2]	6 3 (1 18)	0 834	-0 5 (2 14)	(-5 4, 4 5)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 12	1	Placebo to Eteplirsen [1]	6.7 (1 50)			
		30 mg/kg for 168 weeks[1]	8 0 (1 57)	0 578	1 3 (2 25)	(-4 0, 6 6)
		50 mg/kg for 168 weeks[1]	4 8 (1 51)	0 411	-1 9 (2 12)	(-6 9, 3 2)
	2	Eteplirsen for 144 Weeks[2]	6 8 (1 59)			
		Eteplirsen for 168 weeks[2]	6 4 (1 10)	0 826	-0 4 (1 98)	(-5 0, 4 1)
Change from Baseline to Week 24	1	Placebo to Eteplirsen [1]	7 4 (1 02)			
		30 mg/kg for 168 weeks[1]	7 5 (1 07)	0 957	0 1 (1 53)	(-3 5, 3 7)
		50 mg/kg for 168 weeks[1]	4 6 (1 03)	0 088	-2 9 (1 44)	(-6 3, 0 6)
	2	Eteplirsen for 144 Weeks[2]	7 5 (1 18)			
		Eteplirsen for 168 weeks[2]	6 0 (0 82)	0 322	-1 6 (1 47)	(-4 9, 1 8)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 36	1	Placebo to Eteplirsen [1]	5 9 (1 61)			
		30 mg/kg for 168 weeks[1]	8 2 (1 68)	0 381	2 3 (2 42)	(-3 5, 8 0)
		50 mg/kg for 168 weeks[1]	5 3 (1 62)	0 796	-0 6 (2 28)	(-6 0, 4 8)
	2	Eteplirsen for 144 Weeks[2]	6 1 (1 65)			
		Eteplirsen for 168 weeks[2]	6 7 (1 15)	0 755	0 7 (2 06)	(-4 1, 5 4)
Change from Baseline to Week 48	1	Placebo to Eteplirsen [1]	6 0 (0 78)			
		30 mg/kg for 168 weeks[1]	7 1 (0 81)	0 375	1 1 (1 17)	(-1 7, 3 9)
		50 mg/kg for 168 weeks[1]	6 4 (0 78)	0 739	0 4 (1 10)	(-2 2, 3 0)
	2	Eteplirsen for 144 Weeks[2]	6 0 (0 75)			
		Eteplirsen for 168 weeks[2]	6 7 (0 52)	0 471	0 7 (0 93)	(-1 4, 2 8)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H20)						
Change from Baseline to Week 62	1	Placebo to Eteplirsen [1]	4 8 (1 80)			
		30 mg/kg for 168 weeks[1]	6 8 (1 88)	0 471	2 1 (2 69)	(-4 3, 8 4)
		50 mg/kg for 168 weeks[1]	7 9 (1 80)	0 267	3 1 (2 54)	(-2 9, 9 1)
	2	Eteplirsen for 144 Weeks[2]	4 8 (1 69)			
		Eteplirsen for 168 weeks[2]	7 4 (1 18)	0 251	2 6 (2.11)	(-2 3, 7 5)
Change from Baseline to Week 74	1	Placebo to Eteplirsen [1]	3 8 (1 52)			
		30 mg/kg for 168 weeks[1]	8 4 (1 59)	0 084	4 6 (2 28)	(-0 8, 10 0)
		50 mg/kg for 168 weeks[1]	7 3 (1 52)	0 152	3 4 (2.15)	(-1 6, 8 5)
	2	Eteplirsen for 144 Weeks[2]	3 9 (1 44)			
		Eteplirsen for 168 weeks[2]	7 8 (1 00)	0 059	4 0 (1 80)	(-0 2, 8 1)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates.
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Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 84	1	Placebo to Eteplirsen [1]	4 0 (1 28)			
		30 mg/kg for 168 weeks[1]	7 1 (1 33)	0.144	3 2 (1 92)	(-1 4, 7 7)
		50 mg/kg for 168 weeks[1]	8 4 (1 28)	0 046	4 4 (1 80)	(0 1, 8 6)
	2	Eteplirsen for 144 Weeks[2]	4 0 (1 23)			
		Eteplirsen for 168 weeks[2]	7 8 (0 85)	0 037	3 8 (1 53)	(0 3, 7.3)
Change from Baseline to Week 96	1	Placebo to Eteplirsen [1]	6 1 (1 61)			
		30 mg/kg for 168 weeks[1]	7 6 (1 68)	0 547	1 5 (2 41)	(-4 2, 7 2)
		50 mg/kg for 168 weeks[1]	5 8 (1 61)	0 878	-0 4 (2 27)	(-5 7, 5 0)
	2	Eteplirsen for 144 Weeks[2]	6 2 (1 57)			
		Eteplirsen for 168 weeks[2]	6 7 (1 09)	0 814	0 5 (1 95)	(-4 0, 5 0)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H2O)						
Change from Baseline to Week 120	1	Placebo to Eteplirsen [1]	5 5 (1 32)			
		30 mg/kg for 168 weeks[1]	8 1 (1 38)	0 237	2.6 (1 98)	(-2 1, 7 3)
		50 mg/kg for 168 weeks[1]	5 9 (1 32)	0 819	0.4 (1 87)	(-4 0, 4 9)
	2	Eteplirsen for 144 Weeks[2]	5 6 (1 33)			
		Eteplirsen for 168 weeks[2]	7 0 (0 92)	0 428	1 4 (1 66)	(-2 4, 5 2)
Change from Baseline to Week 144	1	Placebo to Eteplirsen [1]	5 9 (1 44)			
		30 mg/kg for 168 weeks[1]	7 6 (1 50)	0 438	1.8 (2 16)	(-3 3, 6 9)
		50 mg/kg for 168 weeks[1]	6 0 (1 44)	0 960	0 1 (2 03)	(-4 7, 4 9)
	2	Eteplirsen for 144 Weeks[2]	5 9 (1 40)			
		Eteplirsen for 168 weeks[2]	6 8 (0 97)	0 641	0 8 (1 74)	(-3 2, 4 9)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run. 06MAR2015·13 56
Reference listing L 16 2 6 8

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 2 8 1 3
Analysis of Change from Baseline for Pulmonary Function Test using Ranked Data
ITT Population

Time Point	Model	Treatment	Adjusted Mean Change from Baseline (SEM)	P-value for Treatment Comparison versus Placebo	Estimated Treatment Effect (SEM)	95% CI for Treatment Effect
MIP (cm H20)						
Change from Baseline to Week 168	1	Placebo to Eteplirsen [1]	5 9 (1 74)			
		30 mg/kg for 168 weeks[1]	7 5 (1.81)	0 553	1.6 (2 61)	(-4 5, 7 8)
		50 mg/kg for 168 weeks[1]	6 1 (1 74)	0 950	0.2 (2.45)	(-5 6, 6 0)
	2	Eteplirsen for 144 Weeks[2]	6 0 (1 66)			
		Eteplirsen for 168 weeks[2]	6 8 (1 15)	0 706	0 8 (2 07)	(-4 0, 5 6)

Note Change from baseline values are ranked at each time point prior to performing the analysis LS Means are based on the ranked data
[1] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, 30 0 mg/kg, 50 0 mg/kg) as a fixed effect and baseline value and time since DMD diagnosis as covariates
[2] LS Mean, standard error and p-value are from an ANCOVA model for ranked data with treatment (placebo to Eteplirsen, Eteplirsen for 168 Weeks) as a fixed effect and baseline value and time since DMD diagnosis as covariates
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_cfb2 sas, Date/time of run 06MAR2015 13 56
Reference listing L 16 2 6 8

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
At Least One TEAE		2 (100.0%)	2 (100.0%)	4 (100.0%)	4 (100.0%)	4 (100.0%)	8 (100.0%)
Injury, poisoning and procedural complications	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	4 (100.0%)	4 (100.0%)	8 (100.0%)
	Procedural pain	1 (50.0%)	1 (50.0%)	2 (50.0%)	2 (50.0%)	4 (100.0%)	6 (75.0%)
	Contusion	1 (50.0%)	2 (100.0%)	3 (75.0%)	2 (50.0%)	1 (25.0%)	3 (37.5%)
	Arthropod bite	1 (50.0%)	1 (50.0%)	2 (50.0%)	1 (25.0%)	0	1 (12.5%)
	Excoriation	0	1 (50.0%)	1 (25.0%)	1 (25.0%)	1 (25.0%)	2 (25.0%)
	Joint injury	0	1 (50.0%)	1 (25.0%)	2 (50.0%)	0	2 (25.0%)
	Foot fracture	1 (50.0%)	0	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Incision site haemorrhage	1 (50.0%)	0	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Muscle strain	0	0	0	1 (25.0%)	1 (25.0%)	2 (25.0%)
	Back injury	0	0	0	1 (25.0%)	0	1 (12.5%)
	Burns first degree	0	1 (50.0%)	1 (25.0%)	0	0	0
	Fall	0	0	0	1 (25.0%)	0	1 (12.5%)
	Femur fracture	0	0	0	1 (25.0%)	0	1 (12.5%)
	Head injury	0	0	0	0	1 (25.0%)	1 (12.5%)
	Incision site pain	0	0	0	1 (25.0%)	0	1 (12.5%)
	Joint sprain	0	0	0	0	1 (25.0%)	1 (12.5%)
	Laceration	0	0	0	1 (25.0%)	0	1 (12.5%)
	Limb injury	0	0	0	1 (25.0%)	0	1 (12.5%)
	Lower limb fracture	1 (50.0%)	0	1 (25.0%)	0	0	0
	Nail injury	0	0	0	1 (25.0%)	0	1 (12.5%)
	Radius fracture	1 (50.0%)	0	1 (25.0%)	0	0	0
	Thermal burn	0	0	0	0	1 (25.0%)	1 (12.5%)

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumae2.sas, Date/time of run: 06MAR2015:14:07
Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
Respiratory, thoracic and mediastinal disorders	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	4 (100.0%)	4 (100.0%)	8 (100.0%)
	Oropharyngeal pain	1 (50.0%)	2 (100.0%)	3 (75.0%)	3 (75.0%)	2 (50.0%)	5 (62.5%)
	Nasal congestion	1 (50.0%)	1 (50.0%)	2 (50.0%)	3 (75.0%)	1 (25.0%)	4 (50.0%)
	Cough	0	1 (50.0%)	1 (25.0%)	1 (25.0%)	2 (50.0%)	3 (37.5%)
	Epistaxis	1 (50.0%)	1 (50.0%)	2 (50.0%)	0	0	0
	Pharyngeal erythema	0	0	0	1 (25.0%)	1 (25.0%)	2 (25.0%)
	Rhinorrhoea	0	1 (50.0%)	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Upper respiratory tract congestion	0	0	0	2 (50.0%)	0	2 (25.0%)
	Productive cough	0	0	0	0	1 (25.0%)	1 (12.5%)
	Respiratory disorder	0	0	0	1 (25.0%)	0	1 (12.5%)
	Sinus congestion	0	0	0	1 (25.0%)	0	1 (12.5%)
	Sneezing	1 (50.0%)	0	1 (25.0%)	0	0	0
	Upper-airway cough syndrome	0	0	0	0	1 (25.0%)	1 (12.5%)
General disorders and administration site conditions	Overall	2 (100.0%)	1 (50.0%)	3 (75.0%)	4 (100.0%)	4 (100.0%)	8 (100.0%)
	Catheter site pain	0	0	0	2 (50.0%)	2 (50.0%)	4 (50.0%)
	Infusion site extravasation	1 (50.0%)	0	1 (25.0%)	1 (25.0%)	2 (50.0%)	3 (37.5%)
	Infusion site haematoma	1 (50.0%)	1 (50.0%)	2 (50.0%)	0	0	0
	Oedema peripheral	0	0	0	2 (50.0%)	0	2 (25.0%)

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumae2.sas, Date/time of run: 06MAR2015:14:07
Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
	Thrombosis in device	0	0	0	2 (50.0%)	0	2 (25.0%)
	Catheter site related reaction	0	1 (50.0%)	1 (25.0%)	0	0	0
	Chest pain	0	0	0	1 (25.0%)	0	1 (12.5%)
	Device occlusion	0	0	0	1 (25.0%)	0	1 (12.5%)
	Influenza like illness	1 (50.0%)	0	1 (25.0%)	0	0	0
	Infusion site rash	0	0	0	1 (25.0%)	0	1 (12.5%)
	Injection site pain	0	0	0	0	1 (25.0%)	1 (12.5%)
	Irritability	1 (50.0%)	0	1 (25.0%)	0	0	0
	Malaise	0	0	0	0	1 (25.0%)	1 (12.5%)
	Non-cardiac chest pain	0	0	0	1 (25.0%)	0	1 (12.5%)
	Pain	0	0	0	0	1 (25.0%)	1 (12.5%)
	Pyrexia	0	0	0	1 (25.0%)	0	1 (12.5%)
Nervous system disorders	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	4 (100.0%)	3 (75.0%)	7 (87.5%)
	Headache	2 (100.0%)	2 (100.0%)	4 (100.0%)	2 (50.0%)	2 (50.0%)	4 (50.0%)
	Balance disorder	0	0	0	2 (50.0%)	2 (50.0%)	4 (50.0%)
	Psychomotor hyperactivity	0	0	0	0	1 (25.0%)	1 (12.5%)
	Somnolence	0	0	0	1 (25.0%)	0	1 (12.5%)

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumae2.sas, Date/time of run: 06MAR2015:14:07
Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
Infections and infestations	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	2 (50.0%)	4 (100.0%)	6 (75.0%)
	Nasopharyngitis	1 (50.0%)	2 (100.0%)	3 (75.0%)	2 (50.0%)	1 (25.0%)	3 (37.5%)
	Upper respiratory tract infection	1 (50.0%)	1 (50.0%)	2 (50.0%)	1 (25.0%)	3 (75.0%)	4 (50.0%)
	Hordeolum	0	1 (50.0%)	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Influenza	0	1 (50.0%)	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Gastroenteritis viral	0	1 (50.0%)	1 (25.0%)	0	0	0
	Incision site infection	0	0	0	0	1 (25.0%)	1 (12.5%)
	Pharyngitis streptococcal	1 (50.0%)	0	1 (25.0%)	0	0	0
	Rhinitis	0	0	0	0	1 (25.0%)	1 (12.5%)
	Tinea pedis	1 (50.0%)	0	1 (25.0%)	0	0	0
	Viral infection	1 (50.0%)	0	1 (25.0%)	0	0	0
Investigations	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	2 (50.0%)	4 (100.0%)	6 (75.0%)
	C-reactive protein increased	2 (100.0%)	0	2 (50.0%)	0	2 (50.0%)	2 (25.0%)
	Activated partial thromboplastin time prolonged	0	1 (50.0%)	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Blood creatine phosphokinase increased	0	0	0	2 (50.0%)	0	2 (25.0%)
	Blood glucose increased	1 (50.0%)	0	1 (25.0%)	0	1 (25.0%)	1 (12.5%)

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumae2.sas, Date/time of run: 06MAR2015:14:07
Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
	Blood creatinine increased	0	0	0	0	1 (25.0%)	1 (12.5%)
	Blood urea increased	0	0	0	0	1 (25.0%)	1 (12.5%)
	Lymphocyte count decreased	0	0	0	0	1 (25.0%)	1 (12.5%)
	Neutrophil count increased	0	0	0	0	1 (25.0%)	1 (12.5%)
	Red blood cells urine positive	0	0	0	0	1 (25.0%)	1 (12.5%)
	White blood cell count decreased	0	0	0	0	1 (25.0%)	1 (12.5%)
	Wound healing normal	0	1 (50.0%)	1 (25.0%)	0	0	0
Musculoskeletal and connective tissue disorders	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	3 (75.0%)	3 (75.0%)	6 (75.0%)
	Pain in extremity	1 (50.0%)	0	1 (25.0%)	2 (50.0%)	3 (75.0%)	5 (62.5%)
	Arthralgia	1 (50.0%)	1 (50.0%)	2 (50.0%)	1 (25.0%)	2 (50.0%)	3 (37.5%)
	Back pain	0	1 (50.0%)	1 (25.0%)	3 (75.0%)	1 (25.0%)	4 (50.0%)
	Muscle spasms	1 (50.0%)	1 (50.0%)	2 (50.0%)	0	1 (25.0%)	1 (12.5%)
	Muscular weakness	0	2 (100.0%)	2 (50.0%)	0	0	0
	Musculoskeletal pain	0	1 (50.0%)	1 (25.0%)	1 (25.0%)	0	1 (12.5%)
	Bone pain	0	0	0	1 (25.0%)	0	1 (12.5%)
	Myalgia	0	1 (50.0%)	1 (25.0%)	0	0	0
	Neck pain	0	1 (50.0%)	1 (25.0%)	0	0	0
	Tendon disorder	0	0	0	0	1 (25.0%)	1 (12.5%)

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumae2.sas, Date/time of run: 06MAR2015:14:07
Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
	Tendonitis	0	0	0	0	1 (25.0%)	1 (12.5%)
Skin and subcutaneous tissue disorders	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	3 (75.0%)	3 (75.0%)	6 (75.0%)
	Dermatitis contact	0	0	0	2 (50.0%)	1 (25.0%)	3 (37.5%)
	Rash	1 (50.0%)	1 (50.0%)	2 (50.0%)	1 (25.0%)	0	1 (12.5%)
	Erythema	0	0	0	1 (25.0%)	1 (25.0%)	2 (25.0%)
	Ecchymosis	0	1 (50.0%)	1 (25.0%)	0	0	0
	Intertrigo	1 (50.0%)	0	1 (25.0%)	0	0	0
	Keloid scar	0	1 (50.0%)	1 (25.0%)	0	0	0
	Nail discolouration	1 (50.0%)	0	1 (25.0%)	0	0	0
	Nail dystrophy	1 (50.0%)	0	1 (25.0%)	0	0	0
	Papule	0	0	0	1 (25.0%)	0	1 (12.5%)
	Petechiae	0	0	0	1 (25.0%)	0	1 (12.5%)
	Pruritus	0	0	0	0	1 (25.0%)	1 (12.5%)
	Rash papular	1 (50.0%)	0	1 (25.0%)	0	0	0
	Urticaria	0	0	0	0	1 (25.0%)	1 (12.5%)
	Urticaria thermal	0	0	0	1 (25.0%)	0	1 (12.5%)
Gastrointestinal disorders	Overall	2 (100.0%)	2 (100.0%)	4 (100.0%)	3 (75.0%)	2 (50.0%)	5 (62.5%)
	Vomiting	1 (50.0%)	1 (50.0%)	2 (50.0%)	2 (50.0%)	2 (50.0%)	4 (50.0%)
	Diarrhoea	0	1 (50.0%)	1 (25.0%)	1 (25.0%)	1 (25.0%)	2 (25.0%)
	Dyspepsia	1 (50.0%)	1 (50.0%)	2 (50.0%)	1 (25.0%)	0	1 (12.5%)
	Nausea	1 (50.0%)	1 (50.0%)	2 (50.0%)	0	1 (25.0%)	1 (12.5%)
	Abdominal pain	0	1 (50.0%)	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Abdominal pain upper	1 (50.0%)	1 (50.0%)	2 (50.0%)	0	0	0

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumae2.sas, Date/time of run: 06MAR2015:14:07
Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
	Abdominal discomfort	1 (50.0%)	0	1 (25.0%)	0	0	0
	Dental caries	1 (50.0%)	0	1 (25.0%)	0	0	0
	Dysphagia	1 (50.0%)	0	1 (25.0%)	0	0	0
	Flatulence	1 (50.0%)	0	1 (25.0%)	0	0	0
	Food poisoning	0	1 (50.0%)	1 (25.0%)	0	0	0
	Lip swelling	0	0	0	0	1 (25.0%)	1 (12.5%)
	Oral pain	1 (50.0%)	0	1 (25.0%)	0	0	0
	Retained deciduous tooth	0	0	0	1 (25.0%)	0	1 (12.5%)
	Tooth impacted	0	0	0	0	1 (25.0%)	1 (12.5%)
	Toothache	1 (50.0%)	0	1 (25.0%)	0	0	0
Renal and urinary disorders	Overall	0	1 (50.0%)	1 (25.0%)	2 (50.0%)	3 (75.0%)	5 (62.5%)
	Proteinuria	0	1 (50.0%)	1 (25.0%)	2 (50.0%)	3 (75.0%)	5 (62.5%)
	Glycosuria	0	1 (50.0%)	1 (25.0%)	0	0	0
	Polyuria	0	0	0	1 (25.0%)	0	1 (12.5%)
Metabolism and nutrition disorders	Overall	0	0	0	3 (75.0%)	2 (50.0%)	5 (62.5%)
	Hypokalaemia	0	0	0	2 (50.0%)	2 (50.0%)	4 (50.0%)
	Obesity	0	0	0	2 (50.0%)	0	2 (25.0%)
	Vitamin D deficiency	0	0	0	2 (50.0%)	0	2 (25.0%)

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
Ear and labyrinth disorders	Overall	0	0	0	0	2 (50.0%)	2 (25.0%)
	Motion sickness	0	0	0	0	1 (25.0%)	1 (12.5%)
	Tympanic membrane disorder	0	0	0	0	1 (25.0%)	1 (12.5%)
Eye disorders	Overall	1 (50.0%)	0	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Cataract	0	0	0	0	1 (25.0%)	1 (12.5%)
	Conjunctivitis	1 (50.0%)	0	1 (25.0%)	0	0	0
Psychiatric disorders	Overall	1 (50.0%)	0	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Anxiety disorder	1 (50.0%)	0	1 (25.0%)	0	0	0
	Insomnia	0	0	0	0	1 (25.0%)	1 (12.5%)
Unknown	Overall	1 (50.0%)	0	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
	Unknown	1 (50.0%)	0	1 (25.0%)	0	1 (25.0%)	1 (12.5%)
Vascular disorders	Overall	0	0	0	1 (25.0%)	1 (25.0%)	2 (25.0%)
	Haematoma	0	0	0	1 (25.0%)	1 (25.0%)	2 (25.0%)
Cardiac disorders	Overall	0	0	0	1 (25.0%)	0	1 (12.5%)
	Tachycardia	0	0	0	1 (25.0%)	0	1 (12.5%)
Immune system disorders	Overall	0	1 (50.0%)	1 (25.0%)	0	0	0
	Hypersensitivity	0	1 (50.0%)	1 (25.0%)	0	0	0

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1.A
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo to Eteplirsen			Eteplirsen		
		30 mg/kg (N=2)	50 mg/kg (N=2)	Eteplirsen for 144 Weeks (N=4)	30 mg/kg (N=4)	50 mg/kg (N=4)	Eteplirsen for 168 Weeks (N=8)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)	Overall	0	1 (50.0%)	1 (25.0%)	0	0	0
	Skin papilloma	0	1 (50.0%)	1 (25.0%)	0	0	0

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen[1] (N=12)	[redacted]
At Least One TEAE		4 (100.0%)	6 (100.0%)	6 (100.0%)	12 (100.0%)	
Injury, poisoning and procedural complications	Overall	4 (100.0%)	6 (100.0%)	6 (100.0%)	12 (100.0%)	
	Procedural pain	3 (75.0%)	3 (50.0%)	5 (83.3%)	8 (66.7%)	
	Contusion	0	3 (50.0%)	3 (50.0%)	6 (50.0%)	
	Arthropod bite	0	2 (33.3%)	1 (16.7%)	3 (25.0%)	
	Excoriation	0	1 (16.7%)	2 (33.3%)	3 (25.0%)	
	Joint injury	0	2 (33.3%)	1 (16.7%)	3 (25.0%)	
	Fall	1 (25.0%)	1 (16.7%)	0	1 (8.3%)	
	Foot fracture	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Incision site haemorrhage	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Incision site pain	1 (25.0%)	1 (16.7%)	0	1 (8.3%)	
	Muscle strain	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Back injury	0	1 (16.7%)	0	1 (8.3%)	
	Burns first degree	0	0	1 (16.7%)	1 (8.3%)	
	Femur fracture	0	1 (16.7%)	0	1 (8.3%)	
	Head injury	0	0	1 (16.7%)	1 (8.3%)	
	Joint sprain	0	0	1 (16.7%)	1 (8.3%)	
	Laceration	0	1 (16.7%)	0	1 (8.3%)	
	Limb injury	0	1 (16.7%)	0	1 (8.3%)	
	Lower limb fracture	0	1 (16.7%)	0	1 (8.3%)	
	Nail injury	0	1 (16.7%)	0	1 (8.3%)	
	Radius fracture	0	1 (16.7%)	0	1 (8.3%)	
	Thermal burn	0	0	1 (16.7%)	1 (8.3%)	
	Wound dehiscence	1 (25.0%)	0	0	0	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
[2]Placebo is from Week 1 to Week 24 of the 201 Study.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
Respiratory, thoracic and mediastinal disorders	Overall	3 (75.0%)	6 (100.0%)	6 (100.0%)	12 (100.0%)	
	Oropharyngeal pain	3 (75.0%)	4 (66.7%)	4 (66.7%)	8 (66.7%)	
	Nasal congestion	1 (25.0%)	4 (66.7%)	2 (33.3%)	6 (50.0%)	
	Cough	2 (50.0%)	1 (16.7%)	3 (50.0%)	4 (33.3%)	
	Epistaxis	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Pharyngeal erythema	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Rhinorrhoea	0	0	2 (33.3%)	2 (16.7%)	
	Upper respiratory tract congestion	0	2 (33.3%)	0	2 (16.7%)	
	Productive cough	0	0	1 (16.7%)	1 (8.3%)	
	Respiratory disorder	0	1 (16.7%)	0	1 (8.3%)	
	Sinus congestion	0	1 (16.7%)	0	1 (8.3%)	
	Sneezing	0	1 (16.7%)	0	1 (8.3%)	
	Upper-airway cough syndrome	0	0	1 (16.7%)	1 (8.3%)	
General disorders and administration site conditions	Overall	2 (50.0%)	6 (100.0%)	5 (83.3%)	11 (91.7%)	
	Catheter site pain	0	2 (33.3%)	2 (33.3%)	4 (33.3%)	
	Infusion site extravasation	0	2 (33.3%)	2 (33.3%)	4 (33.3%)	
	Pyrexia	2 (50.0%)	1 (16.7%)	0	1 (8.3%)	
	Infusion site haematoma	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Oedema peripheral	0	2 (33.3%)	0	2 (16.7%)	
	Thrombosis in device	0	2 (33.3%)	0	2 (16.7%)	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
[2]Placebo is from Week 1 to Week 24 of the 201 Study.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	All Eteplirsen (N=12)	[redacted]
	Catheter site related reaction	0	0	1 (16.7%)	1 (8.3%)	
	Chest pain	0	1 (16.7%)	0	1 (8.3%)	
	Device occlusion	0	1 (16.7%)	0	1 (8.3%)	
	Influenza like illness	0	1 (16.7%)	0	1 (8.3%)	
	Infusion site rash	0	1 (16.7%)	0	1 (8.3%)	
	Injection site pain	0	0	1 (16.7%)	1 (8.3%)	
	Irritability	0	1 (16.7%)	0	1 (8.3%)	
	Malaise	0	0	1 (16.7%)	1 (8.3%)	
	Non-cardiac chest pain	0	1 (16.7%)	0	1 (8.3%)	
	Pain	0	0	1 (16.7%)	1 (8.3%)	
Nervous system disorders	Overall	2 (50.0%)	6 (100.0%)	5 (83.3%)	11 (91.7%)	
	Headache	2 (50.0%)	4 (66.7%)	4 (66.7%)	8 (66.7%)	
	Balance disorder	0	2 (33.3%)	2 (33.3%)	4 (33.3%)	
	Dizziness	1 (25.0%)	0	0	0	
	Psychomotor hyperactivity	0	0	1 (16.7%)	1 (8.3%)	
	Somnolence	0	1 (16.7%)	0	1 (8.3%)	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
[2]Placebo is from Week 1 to Week 24 of the 201 Study.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	All Eteplirsen (N=12)	[redacted]
Infections and infestations	Overall	3 (75.0%)	4 (66.7%)	6 (100.0%)	10 (83.3%)	
	Nasopharyngitis	1 (25.0%)	3 (50.0%)	3 (50.0%)	6 (50.0%)	
	Upper respiratory tract infection	0	2 (33.3%)	4 (66.7%)	6 (50.0%)	
	Hordeolum	0	0	2 (33.3%)	2 (16.7%)	
	Influenza	0	0	2 (33.3%)	2 (16.7%)	
	Rhinitis	1 (25.0%)	0	1 (16.7%)	1 (8.3%)	
	Enterobiasis	1 (25.0%)	0	0	0	
	Gastroenteritis viral	0	0	1 (16.7%)	1 (8.3%)	
	Incision site infection	0	0	1 (16.7%)	1 (8.3%)	
	Pharyngitis streptococcal	0	1 (16.7%)	0	1 (8.3%)	
	Soft tissue infection	1 (25.0%)	0	0	0	
	Tinea pedis	0	1 (16.7%)	0	1 (8.3%)	
	Viral infection	0	1 (16.7%)	0	1 (8.3%)	
Investigations	Overall	0	4 (66.7%)	6 (100.0%)	10 (83.3%)	
	C-reactive protein increased	0	2 (33.3%)	2 (33.3%)	4 (33.3%)	
	Activated partial thromboplastin time prolonged	0	0	2 (33.3%)	2 (16.7%)	
	Blood creatine phosphokinase increased	0	2 (33.3%)	0	2 (16.7%)	
	Blood glucose increased	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
	Blood creatinine increased	0	0	1 (16.7%)	1 (8.3%)	
	Blood urea increased	0	0	1 (16.7%)	1 (8.3%)	
	Lymphocyte count decreased	0	0	1 (16.7%)	1 (8.3%)	
	Neutrophil count increased	0	0	1 (16.7%)	1 (8.3%)	
	Red blood cells urine positive	0	0	1 (16.7%)	1 (8.3%)	
	White blood cell count decreased	0	0	1 (16.7%)	1 (8.3%)	
	Wound healing normal	0	0	1 (16.7%)	1 (8.3%)	
Musculoskeletal and connective tissue disorders	Overall	4 (100.0%)	5 (83.3%)	5 (83.3%)	10 (83.3%)	
	Pain in extremity	4 (100.0%)	3 (50.0%)	3 (50.0%)	6 (50.0%)	
	Back pain	2 (50.0%)	3 (50.0%)	2 (33.3%)	5 (41.7%)	
	Arthralgia	0	2 (33.3%)	3 (50.0%)	5 (41.7%)	
	Muscle spasms	0	1 (16.7%)	2 (33.3%)	3 (25.0%)	
	Muscular weakness	0	0	2 (33.3%)	2 (16.7%)	
	Musculoskeletal pain	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Bone pain	0	1 (16.7%)	0	1 (8.3%)	
	Myalgia	0	0	1 (16.7%)	1 (8.3%)	
	Neck pain	0	0	1 (16.7%)	1 (8.3%)	
	Tendon disorder	0	0	1 (16.7%)	1 (8.3%)	
	Tendonitis	0	0	1 (16.7%)	1 (8.3%)	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	All Eteplirsen (N=12)	[redacted]
Skin and subcutaneous tissue disorders	Overall	0	5 (83.3%)	5 (83.3%)	10 (83.3%)	
	Dermatitis contact	0	2 (33.3%)	1 (16.7%)	3 (25.0%)	
	Rash	0	2 (33.3%)	1 (16.7%)	3 (25.0%)	
	Erythema	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Ecchymosis	0	0	1 (16.7%)	1 (8.3%)	
	Intertrigo	0	1 (16.7%)	0	1 (8.3%)	
	Keloid scar	0	0	1 (16.7%)	1 (8.3%)	
	Nail discolouration	0	1 (16.7%)	0	1 (8.3%)	
	Nail dystrophy	0	1 (16.7%)	0	1 (8.3%)	
	Papule	0	1 (16.7%)	0	1 (8.3%)	
	Petechiae	0	1 (16.7%)	0	1 (8.3%)	
	Pruritus	0	0	1 (16.7%)	1 (8.3%)	
	Rash papular	0	1 (16.7%)	0	1 (8.3%)	
	Urticaria	0	0	1 (16.7%)	1 (8.3%)	
	Urticaria thermal	0	1 (16.7%)	0	1 (8.3%)	
Gastrointestinal disorders	Overall	1 (25.0%)	5 (83.3%)	4 (66.7%)	9 (75.0%)	
	Vomiting	0	3 (50.0%)	3 (50.0%)	6 (50.0%)	
	Abdominal pain	1 (25.0%)	0	2 (33.3%)	2 (16.7%)	
	Diarrhoea	0	1 (16.7%)	2 (33.3%)	3 (25.0%)	
	Dyspepsia	0	2 (33.3%)	1 (16.7%)	3 (25.0%)	
	Nausea	1 (25.0%)	1 (16.7%)	2 (33.3%)	3 (25.0%)	
	Abdominal pain upper	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Abdominal discomfort	0	1 (16.7%)	0	1 (8.3%)	
	Dental caries	0	1 (16.7%)	0	1 (8.3%)	
	Dysphagia	0	1 (16.7%)	0	1 (8.3%)	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
[2]Placebo is from Week 1 to Week 24 of the 201 Study.
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Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	All Eteplirsen (N=12)
	Flatulence	0	1 (16.7%)	0	1 (8.3%)
	Food poisoning	0	0	1 (16.7%)	1 (8.3%)
	Lip swelling	0	0	1 (16.7%)	1 (8.3%)
	Oral pain	0	1 (16.7%)	0	1 (8.3%)
	Retained deciduous tooth	0	1 (16.7%)	0	1 (8.3%)
	Tooth impacted	0	0	1 (16.7%)	1 (8.3%)
	Toothache	0	1 (16.7%)	0	1 (8.3%)
Metabolism and nutrition disorders	Overall	2 (50.0%)	3 (50.0%)	2 (33.3%)	5 (41.7%)
	Hypokalaemia	2 (50.0%)	2 (33.3%)	2 (33.3%)	4 (33.3%)
	Obesity	0	2 (33.3%)	0	2 (16.7%)
	Vitamin D deficiency	0	2 (33.3%)	0	2 (16.7%)
Renal and urinary disorders	Overall	1 (25.0%)	2 (33.3%)	4 (66.7%)	6 (50.0%)
	Proteinuria	1 (25.0%)	2 (33.3%)	4 (66.7%)	6 (50.0%)
	Glycosuria	0	0	1 (16.7%)	1 (8.3%)
	Polyuria	0	1 (16.7%)	0	1 (8.3%)
Vascular disorders	Overall	1 (25.0%)	1 (16.7%)	1 (16.7%)	2 (16.7%)
	Haematoma	1 (25.0%)	1 (16.7%)	1 (16.7%)	2 (16.7%)
Ear and labyrinth disorders	Overall	0	0	2 (33.3%)	2 (16.7%)
	Motion sickness	0	0	1 (16.7%)	1 (8.3%)
	Tympanic membrane disorder	0	0	1 (16.7%)	1 (8.3%)

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
[2]Placebo is from Week 1 to Week 24 of the 201 Study.
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Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.1
Summary of Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	All Eteplirsen (N=12)	[redacted]
Eye disorders	Overall	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Cataract	0	0	1 (16.7%)	1 (8.3%)	
	Conjunctivitis	0	1 (16.7%)	0	1 (8.3%)	
Psychiatric disorders	Overall	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Anxiety disorder	0	1 (16.7%)	0	1 (8.3%)	
	Insomnia	0	0	1 (16.7%)	1 (8.3%)	
Unknown	Overall	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Unknown	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
Cardiac disorders	Overall	0	1 (16.7%)	0	1 (8.3%)	
	Tachycardia	0	1 (16.7%)	0	1 (8.3%)	
Immune system disorders	Overall	0	0	1 (16.7%)	1 (8.3%)	
	Hypersensitivity	0	0	1 (16.7%)	1 (8.3%)	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)	Overall	0	0	1 (16.7%)	1 (8.3%)	
	Skin papilloma	0	0	1 (16.7%)	1 (8.3%)	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
[2]Placebo is from Week 1 to Week 24 of the 201 Study.
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Reference listing: L.16.2.7.2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.1.2
Summary of Treatment Related Treatment Emergent Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
At Least One Treatment Related TEAE		1 (25.0%)	3 (50.0%)	3 (50.0%)	6 (50.0%)	
General disorders and administration site conditions	Overall	0	2 (33.3%)	0	2 (16.7%)	
	Thrombosis in device	0	2 (33.3%)	0	2 (16.7%)	
	Device occlusion	0	1 (16.7%)	0	1 (8.3%)	
Renal and urinary disorders	Overall	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
	Proteinuria	0	1 (16.7%)	1 (16.7%)	2 (16.7%)	
Gastrointestinal disorders	Overall	1 (25.0%)	0	0	0	
	Nausea	1 (25.0%)	0	0	0	
Investigations	Overall	0	0	1 (16.7%)	1 (8.3%)	
	White blood cell count decreased	0	0	1 (16.7%)	1 (8.3%)	
Skin and subcutaneous tissue disorders	Overall	0	0	1 (16.7%)	1 (8.3%)	
	Erythema	0	0	1 (16.7%)	1 (8.3%)	

Note: Adverse events were attributed to the treatment being received at start of adverse event.
Note: Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term.
[2]Placebo is from Week 1 to Week 24 of the 201 Study.
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Reference listing: L.16.2.7.2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
At Least One TEAE	Overall		4 (100 0%)	6 (100.0%)	6 (100 0%)	12 (100 0%)	
		Mild	3 (75 0%)	0	2 (33.3%)	2 (16 7%)	
		Moderate	1 (25 0%)	4 (66 7%)	4 (66 7%)	8 (66 7%)	
		Severe	0	2 (33 3%)	0	2 (16 7%)	
Injury, poisoning and procedural complications	Overall		4 (100 0%)	6 (100 0%)	6 (100 0%)	12 (100.0%)	
	Procedural pain	Mild	2 (50 0%)	2 (33 3%)	5 (83 3%)	7 (58 3%)	
		Moderate	1 (25 0%)	1 (16.7%)	0	1 (8 3%)	
		Severe	0	0	0	0	
	Contusion	Mild	0	2 (33.3%)	2 (33 3%)	4 (33 3%)	
		Moderate	0	1 (16 7%)	1 (16 7%)	2 (16 7%)	
		Severe	0	0	0	0	
	Arthropod bite	Mild	0	2 (33 3%)	1 (16.7%)	3 (25 0%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Excoriation	Mild	0	1 (16 7%)	2 (33 3%)	3 (25 0%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Joint injury	Mild	0	2 (33 3%)	1 (16.7%)	3 (25 0%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Fall	Mild	1 (25 0%)	1 (16.7%)	0	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	

Note. Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Reference listing: L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Foot fracture	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
	Incision site haemorrhage	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	1 (16 7%)	2 (16 7%)
		Severe	0	0	0	0
	Incision site pain	Mild	1 (25 0%)	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8.3%)
		Severe	0	0	0	0
	Muscle strain	Mild	0	1 (16 7%)	1 (16 7%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Back injury	Mild	0	1 (16.7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Burns first degree	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Femur fracture	Mild	0	0	0	0
		Moderate	0	0	0	0
		Severe	0	1 (16.7%)	0	1 (8 3%)
	Head injury	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study.
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Joint sprain	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Laceration	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
	Limb injury	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Lower limb fracture	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
	Nail injury	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Radius fracture	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
	Thermal burn	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Wound dehiscence	Mild	1 (25.0%)	0	0	0
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
Respiratory, thoracic and mediastinal disorders	Overall		3 (75 0%)	6 (100.0%)	6 (100 0%)	12 (100 0%)	
	Oropharyngeal pain	Mild	3 (75.0%)	4 (66 7%)	3 (50 0%)	7 (58 3%)	
		Moderate	0	0	1 (16 7%)	1 (8 3%)	
		Severe	0	0	0	0	
	Nasal congestion	Mild	1 (25.0%)	3 (50 0%)	2 (33 3%)	5 (41 7%)	
		Moderate	0	0	0	0	
		Severe	0	1 (16 7%)	0	1 (8 3%)	
	Cough	Mild	2 (50 0%)	1 (16 7%)	2 (33 3%)	3 (25 0%)	
		Moderate	0	0	1 (16.7%)	1 (8.3%)	
		Severe	0	0	0	0	
	Epistaxis	Mild	0	1 (16 7%)	1 (16 7%)	2 (16 7%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Pharyngeal erythema	Mild	0	1 (16 7%)	1 (16 7%)	2 (16 7%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Rhinorrhoea	Mild	0	0	1 (16 7%)	1 (8 3%)	
		Moderate	0	0	1 (16 7%)	1 (8 3%)	
		Severe	0	0	0	0	
	Upper respiratory tract congestion	Mild	0	2 (33 3%)	0	2 (16 7%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	

Note Adverse events were attributed to the treatment being received at start of adverse event.
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Productive cough	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Respiratory disorder	Mild	0	1 (16.7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Sinus congestion	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Sneezing	Mild	0	1 (16.7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Upper-airway cough syndrome	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
General disorders and administration site conditions	Overall		2 (50 0%)	6 (100.0%)	5 (83 3%)	11 (91 7%)
	Catheter site pain	Mild	0	1 (16 7%)	2 (33 3%)	3 (25 0%)
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Infusion site extravasation	Mild	0	2 (33 3%)	2 (33 3%)	4 (33.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Pyrexia	Mild	2 (50 0%)	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Infusion site haematoma	Mild	0	1 (16 7%)	1 (16 7%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Oedema peripheral	Mild	0	0	0	0
		Moderate	0	2 (33 3%)	0	2 (16.7%)
		Severe	0	0	0	0
	Thrombosis in device	Mild	0	0	0	0
		Moderate	0	2 (33 3%)	0	2 (16 7%)
		Severe	0	0	0	0
	Catheter site related reaction	Mild	0	0	1 (16 7%)	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Chest pain	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event.
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Reference listing. L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1.3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Device occlusion	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
	Influenza like illness	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Infusion site rash	Mild	0	1 (16 7%)	0	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Injection site pain	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Irritability	Mild	0	1 (16 7%)	0	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Malaise	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Non-cardiac chest pain	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Pain	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included.
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once). Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Reference listing L.16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
Nervous system disorders	Overall		2 (50 0%)	6 (100 0%)	5 (83 3%)	11 (91 7%)	
	Headache	Mild	2 (50 0%)	3 (50.0%)	4 (66 7%)	7 (58 3%)	
		Moderate	0	1 (16 7%)	0	1 (8.3%)	
		Severe	0	0	0	0	
	Balance disorder	Mild	0	1 (16 7%)	2 (33 3%)	3 (25 0%)	
		Moderate	0	0	0	0	
		Severe	0	1 (16 7%)	0	1 (8 3%)	
	Dizziness	Mild	1 (25.0%)	0	0	0	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Psychomotor hyperactivity	Mild	0	0	1 (16.7%)	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Somnolence	Mild	0	1 (16 7%)	0	1 (8.3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
Infections and infestations	Overall		3 (75.0%)	4 (66 7%)	6 (100 0%)	10 (83 3%)	
	Nasopharyngitis	Mild	1 (25 0%)	3 (50 0%)	3 (50 0%)	6 (50 0%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	

Note. Adverse events were attributed to the treatment being received at start of adverse event.
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumaesev sas, Date/time of run 06MAR2015 14 07
Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Upper respiratory tract infection	Mild	0	2 (33 3%)	3 (50 0%)	5 (41 7%)
		Moderate	0	0	1 (16.7%)	1 (8 3%)
		Severe	0	0	0	0
	Hordeolum	Mild	0	0	2 (33 3%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Influenza	Mild	0	0	1 (16 7%)	1 (8.3%)
		Moderate	0	0	1 (16 7%)	1 (8 3%)
		Severe	0	0	0	0
	Rhinitis	Mild	1 (25.0%)	0	1 (16 7%)	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Enterobiasis	Mild	1 (25 0%)	0	0	0
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Gastroenteritis viral	Mild	0	0	1 (16 7%)	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Incision site infection	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included.
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Pharyngitis streptococcal	Mild	0	1 (16.7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Soft tissue infection	Mild	1 (25 0%)	0	0	0
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Tinea pedis	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Viral infection	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Investigations	Overall		0	4 (66 7%)	6 (100 0%)	10 (83.3%)
	C-reactive protein increased	Mild	0	2 (33 3%)	1 (16 7%)	3 (25 0%)
		Moderate	0	0	1 (16 7%)	1 (8 3%)
		Severe	0	0	0	0
	Activated partial thromboplastin time prolonged	Mild	0	0	2 (33.3%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Blood creatine phosphokinase increased	Mild	0	2 (33 3%)	0	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Blood glucose increased	Mild	0	1 (16 7%)	1 (16.7%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Blood creatinine increased	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Blood urea increased	Mild	0	0	1 (16.7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Lymphocyte count decreased	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Neutrophil count increased	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note: Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term.
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Reference listing L 16 2 7 2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Red blood cells urine positive	Mild	0	0	1 (16 7%)	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	White blood cell count decreased	Mild	0	0	1 (16.7%)	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Wound healing normal	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Musculoskeletal and connective tissue disorders	Overall		4 (100 0%)	5 (83 3%)	5 (83 3%)	10 (83 3%)
	Pain in extremity	Mild	4 (100 0%)	2 (33 3%)	2 (33 3%)	4 (33 3%)
		Moderate	0	1 (16.7%)	1 (16.7%)	2 (16 7%)
		Severe	0	0	0	0
	Back pain	Mild	2 (50 0%)	1 (16.7%)	2 (33 3%)	3 (25 0%)
		Moderate	0	2 (33 3%)	0	2 (16 7%)
		Severe	0	0	0	0
	Arthralgia	Mild	0	1 (16 7%)	3 (50 0%)	4 (33 3%)
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0

Note Adverse events were attributed to the treatment being received at start of adverse event
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Muscle spasms	Mild	0	1 (16 7%)	2 (33 3%)	3 (25 0%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Muscular weakness	Mild	0	0	2 (33 3%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Musculoskeletal pain	Mild	0	1 (16 7%)	1 (16 7%)	2 (16.7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Bone pain	Mild	0	0	0	0
		Moderate	0	0	0	0
		Severe	0	1 (16 7%)	0	1 (8 3%)
	Myalgia	Mild	0	0	0	0
		Moderate	0	0	1 (16 7%)	1 (8' 3%)
		Severe	0	0	0	0
	Neck pain	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Tendon disorder	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Tendonitis	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note· Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
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[3]Placebo is from Week 1 to Week 24 of the 201 Study
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Reference listing L 16 2 7 2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
Skin and subcutaneous tissue disorders	Overall		0	5 (83.3%)	5 (83.3%)	10 (83 3%)
	Dermatitis contact	Mild	0	1 (16 7%)	1 (16 7%)	2 (16 7%)
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
	Rash	Mild	0	2 (33 3%)	1 (16 7%)	3 (25 0%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Erythema	Mild	0	1 (16 7%)	1 (16 7%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Ecchymosis	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Intertrigo	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Keloid scar	Mild	0	0	0	0
		Moderate	0	0	1 (16 7%)	1 (8.3%)
		Severe	0	0	0	0
	Nail discolouration	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Nail dystrophy	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Papule	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Petechiae	Mild	0	1 (16 7%)	0	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Pruritus	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Rash papular	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Urticaria	Mild	0	0	1 (16.7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Urticaria thermal	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Gastrointestinal disorders	Overall		1 (25.0%)	5 (83 3%)	4 (66 7%)	9 (75 0%)

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Vomiting	Mild	0	3 (50 0%)	3 (50.0%)	6 (50 0%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Abdominal pain	Mild	1 (25 0%)	0	2 (33.3%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Diarrhoea	Mild	0	1 (16.7%)	1 (16 7%)	2 (16 7%)
		Moderate	0	0	1 (16 7%)	1 (8 3%)
		Severe	0	0	0	0
	Dyspepsia	Mild	0	2 (33 3%)	1 (16 7%)	3 (25 0%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Nausea	Mild	1 (25.0%)	1 (16 7%)	2 (33 3%)	3 (25 0%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Abdominal pain upper	Mild	0	1 (16 7%)	1 (16.7%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Abdominal discomfort	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Dental caries	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
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Reference listing. L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Dysphagia	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8.3%)
		Severe	0	0	0	0
	Flatulence	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Food poisoning	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Lip swelling	Mild	0	0	1 (16.7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Oral pain	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Retained deciduous tooth	Mild	0	1 (16.7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Tooth impacted	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
	Toothache	Mild	0	1 (16 7%)	0	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
Metabolism and nutrition disorders	Overall		2 (50 0%)	3 (50 0%)	2 (33 3%)	5 (41 7%)	
	Hypokalaemia	Mild	2 (50 0%)	1 (16 7%)	2 (33.3%)	3 (25 0%)	
		Moderate	0	1 (16.7%)	0	1 (8.3%)	
		Severe	0	0	0	0	
	Obesity	Mild	0	0	0	0	
		Moderate	0	2 (33 3%)	0	2 (16 7%)	
		Severe	0	0	0	0	
	Vitamin D deficiency	Mild	0	2 (33 3%)	0	2 (16 7%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
Renal and urinary disorders	Overall		1 (25 0%)	2 (33 3%)	4 (66 7%)	6 (50 0%)	
	Proteinuria	Mild	1 (25 0%)	2 (33 3%)	4 (66 7%)	6 (50 0%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Glycosuria	Mild	0	0	1 (16 7%)	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Polyuria	Mild	0	1 (16 7%)	0	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
Vascular disorders	Overall		1 (25 0%)	1 (16 7%)	1 (16 7%)	2 (16 7%)	

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Reference listing: L 16 2 7 2

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 1.3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)	[redacted]
	Haematoma	Mild	1 (25.0%)	1 (16 7%)	1 (16 7%)	2 (16 7%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
Ear and labyrinth disorders	Overall		0	0	2 (33 3%)	2 (16 7%)	
	Motion sickness	Mild	0	0	1 (16 7%)	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Tympanic membrane disorder	Mild	0	0	1 (16 7%)	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
Eye disorders	Overall		0	1 (16.7%)	1 (16 7%)	2 (16 7%)	
	Cataract	Mild	0	0	1 (16 7%)	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
	Conjunctivitis	Mild	0	1 (16 7%)	0	1 (8 3%)	
		Moderate	0	0	0	0	
		Severe	0	0	0	0	
Psychiatric disorders	Overall		0	1 (16 7%)	1 (16 7%)	2 (16 7%)	
	Anxiety disorder	Mild	0	0	0	0	
		Moderate	0	1 (16 7%)	0	1 (8 3%)	
		Severe	0	0	0	0	

Note. Adverse events were attributed to the treatment being received at start of adverse event
Note. Adverse events through study day 1177 are included
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1.3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
	Insomnia	Mild	0	0	1 (16 7%)	1 (8.3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Unknown	Overall		0	1 (16 7%)	1 (16 7%)	2 (16 7%)
	Unknown	Mild	0	1 (16 7%)	1 (16 7%)	2 (16.7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Cardiac disorders	Overall		0	1 (16 7%)	0	1 (8 3%)
	Tachycardia	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
Immune system disorders	Overall		0	0	1 (16.7%)	1 (8 3%)
	Hypersensitivity	Mild	0	0	1 (16.7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Neoplasms benign, malignant and unspecified (incl cysts and polyps)	Overall		0	0	1 (16 7%)	1 (8 3%)
	Skin papilloma	Mild	0	0	1 (16.7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note: Adverse events were attributed to the treatment being received at start of adverse event
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.1 3
Summary of Treatment Emergent Adverse Events by Severity
Safety Population

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
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Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 1 4
Summary of Treatment Related Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
At Least One TEAE	Overall		1 (25 0%)	3 (50 0%)	3 (50 0%)	6 (50 0%)
		Mild	1 (25 0%)	1 (16.7%)	3 (50 0%)	4 (33 3%)
		Moderate	0	2 (33.3%)	0	2 (16 7%)
		Severe	0	0	0	0
General disoiders and administration site conditions	Overall		0	2 (33 3%)	0	2 (16.7%)
	Thrombosis in device	Mild	0	0	0	0
		Moderate	0	2 (33.3%)	0	2 (16.7%)
		Severe	0	0	0	0
	Device occlusion	Mild	0	0	0	0
		Moderate	0	1 (16 7%)	0	1 (8 3%)
		Severe	0	0	0	0
Renal and urinary disorders	Overall		0	1 (16 7%)	1 (16 7%)	2 (16 7%)
	Proteinuria	Mild	0	1 (16 7%)	1 (16 7%)	2 (16 7%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Gastrointestinal disorders	Overall		1 (25 0%)	0	0	0
	Nausea	Mild	1 (25 0%)	0	0	0
		Moderate	0	0	0	0
		Severe	0	0	0	0

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term.
[3]Placebo is from Week 1 to Week 24 of the 201 Study
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumaesev sas, Date/time of run: 06MAR2015 14 08
Reference listing L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 1.4
Summary of Treatment Related Treatment Emergent Adverse Events by Severity
Safety Population

Body System	Preferred Term	Severity[1]	Placebo[3] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
Investigations	Overall		0	0	1 (16 7%)	1 (8 3%)
	White blood cell count decreased	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0
Skin and subcutaneous tissue disorders	Overall		0	0	1 (16.7%)	1 (8 3%)
	Erythema	Mild	0	0	1 (16 7%)	1 (8 3%)
		Moderate	0	0	0	0
		Severe	0	0	0	0

[redacted]

Note Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included
[1]Worst severity in case of multiple occurences per preferred term
[2]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[3]Placebo is from Week 1 to Week 24 of the 201 Study
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumaesev sas, Date/time of run 06MAR2015 14 08
Reference listing. L 16 2 7 2

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.1 5
Summary of Serious Adverse Events
Safety Population

Body System	Preferred Term	Placebo[2] (N=4)	Eteplirsen 30 mg/kg (N=6)	Eteplirsen 50 mg/kg (N=6)	Eteplirsen All Eteplirsen (N=12)
At Least One Serious AE		0	1 (16.7%)	0	1 (8 3%)
Injury, poisoning and procedural complications	Overall	0	1 (16 7%)	0	1 (8 3%)
	Femur fracture	0	1 (16 7%)	0	1 (8 3%)

[redacted]

Note: Adverse events were attributed to the treatment being received at start of adverse event
Note Adverse events through study day 1177 are included.
[1]All Subjects = Subjects who took any study medication (counted only once) Subjects were counted once in each body system and preferred term
[2]Placebo is from Week 1 to Week 24 of the 201 Study
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_sumae sas, Date/time of run 06MAR2015 14 07
Reference listing L 16 2 7 4

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Sodium (mmol/L)							
Baseline [1]	n	4		4		8	
	Mean	141 0		140 0		140 5	
	Median	141 0		140 0		140 0	
	SD	1.15		0 82		1 07	
	(SE)	(0 58)		(0 41)		(0 38)	
	Min,	140,		139,		139,	
	Max	142		141		142	
Week 2	n	4	4	4	4	8	8
	Mean	140 5	-0 5	140 3	0 3	140 4	-0 1
	Median	141 0	-1 0	141 0	0 5	141 0	-0.5
	SD	1 00	1 00	1 50	0 96	1 19	0 99
	(SE)	(0 50)	(0 50)	(0 75)	(0 48)	(0 42)	(0 35)
	Min,	139,	-1,	138,	-1,	138,	-1,
	Max	141	1	141	1	141	1
Week 4	n	4	4	4	4	8	8
	Mean	138 5	-2 5	140 5	0.5	139 5	-1 0
	Median	139 0	-2 0	140 5	0 0	139 5	0 0
	SD	1 73	2 65	1 29	1.00	1 77	2 45
	(SE)	(0 87)	(1 32)	(0 65)	(0 50)	(0 63)	(0 87)
	Min,	136,	-6,	139,	0,	136,	-6,
	Max	140	0	142	2	142	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	142.0	1.0	142.5	2 5	142.3	1 8
	Median	142.0	1 0	141 5	1 5	142.0	1 5
	SD	1 63	1 15	3.11	2.38	2 31	1 91
	(SE)	(0.82)	(0 58)	(1 55)	(1 19)	(0 82)	(0 67)
	Min,	140,	0,	140,	1,	140,	0,
	Max	144	2	147	6	147	6
Week 8	n	4	4	4	4	8	8
	Mean	140 8	-0.3	141 3	1 3	141.0	0 5
	Median	140 0	-0.5	141 5	1 5	140 5	0 5
	SD	2 22	1.71	1 71	1.71	1 85	1 77
	(SE)	(1 11)	(0 85)	(0 85)	(0.85)	(0 65)	(0 63)
	Min,	139,	-2,	139,	-1,	139,	-2,
	Max	144	2	143	3	144	3
Week 10	n	4	4	4	4	8	8
	Mean	139 5	-1 5	140 5	0 5	140 0	-0 5
	Median	139 5	-2 0	140 5	0 5	140 0	-0 5
	SD	1 29	1 73	1 29	1 29	1 31	1 77
	(SE)	(0 65)	(0 87)	(0 65)	(0 65)	(0.46)	(0 63)
	Min,	138,	-3,	139,	-1,	138,	-3,
	Max	141	1	142	2	142	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015·14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	140 3	-0.8	139 0	-1 0	139 6	-0 9
	Median	140 5	-0 5	138 5	-2 0	139 5	-1 5
	SD	1.71	0.96	1 41	2 00	1 60	1 46
	(SE)	(0.85)	(0 48)	(0 71)	(1 00)	(0 56)	(0 52)
	Min,	138,	-2,	138,	-2,	138,	-2,
	Max	142	0	141	2	142	2
Week 15	n	4	4	4	4	8	8
	Mean	139 8	-1 3	140 0	0 0	139 9	-0 6
	Median	139.0	-1.0	140 0	0 0	139 5	-0 5
	SD	2 22	2 06	0 82	0 82	1 55	1 60
	(SE)	(1 11)	(1.03)	(0 41)	(0.41)	(0 55)	(0 56)
	Min,	138,	-4,	139,	-1,	138,	-4,
	Max	143	1	141	1	143	1
Week 18	n	4	4	4	4	8	8
	Mean	140.0	-1 0	139 0	-1 0	139.5	-1 0
	Median	140 5	-1 5	139 0	-1 0	139 5	-1 5
	SD	1 41	1 41	1 63	1 83	1.51	1 51
	(SE)	(0 71)	(0 71)	(0.82)	(0 91)	(0 53)	(0 53)
	Min,	138,	-2,	137,	-3,	137,	-3,
	Max	141	1	141	1	141	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 21	n	4	4	4	4	8	8
	Mean	142 3	1 3	141 3	1 3	141 8	1 3
	Median	142 0	1 5	141 0	1.0	142 0	1 0
	SD	0 50	0 96	1 26	0.50	1 04	0 71
	(SE)	(0 25)	(0.48)	(0 63)	(0.25)	(0 37)	(0 25)
	Min,	142,	0,	140,	1,	140,	0,
	Max	143	2	143	2	143	2
Week 24	n	4	4	4	4	8	8
	Mean	141 5	0 5	140.5	0 5	141.0	0 5
	Median	142 0	1 0	141 0	0 5	141 0	1 0
	SD	1 91	1 00	1 73	1 29	1.77	1 07
	(SE)	(0 96)	(0 50)	(0 87)	(0.65)	(0 63)	(0 38)
	Min,	139,	-1,	138,	-1,	138,	-1,
	Max	143	1	142	2	143	2
Week 25	n	4	4	4	4	8	8
	Mean	141 8	0 8	141.3	1 3	141 5	1 0
	Median	141 5	1 0	142.0	1 5	142.0	1 0
	SD	0 96	1 26	1 50	1 71	1 20	1 41
	(SE)	(0 48)	(0.63)	(0.75)	(0 85)	(0 42)	(0 50)
	Min,	141,	-1,	139,	-1,	139,	-1,
	Max	143	2	142	3	143	3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 28	n	4	4	4	4	8	8
	Mean	138 0	-3 0	139.8	-0.3	138.9	-1 6
	Median	138 0	-3 0	140 0	0.5	139 0	-2.0
	SD	2 58	1 63	3 77	3 59	3 14	2 97
	(SE)	(1 29)	(0 82)	(1.89)	(1 80)	(1 11)	(1 05)
	Min,	135,	-5,	135,	-5,	135,	-5,
	Max	141	-1	144	3	144	3
Week 32	n	4	4	3	3	7	7
	Mean	140 5	-0 5	140 0	-0 3	140.3	-0.4
	Median	140.5	-1 0	140 0	-1 0	140 0	-1 0
	SD	1 29	1.73	1 00	1 15	1 11	1 40
	(SE)	(0 65)	(0 87)	(0.58)	(0 67)	(0.42)	(0.53)
	Min,	139,	-2,	139,	-1,	139,	-2,
	Max	142	2	141	1	142	2
Week 36	n	4	4	4	4	8	8
	Mean	139 0	-2 0	139 0	-1.0	139 0	-1 5
	Median	139 0	-2 0	139 5	-0 5	139 5	-1 5
	SD	1 83	2 45	1 41	1 41	1 51	1 93
	(SE)	(0 91)	(1 22)	(0.71)	(0 71)	(0.53)	(0 68)
	Min,	137,	-5,	137,	-3,	137,	-5,
	Max	141	1	140	0	141	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 40	n	4	4	4	4	8	8
	Mean	140 5	-0 5	140 8	0 8	140 6	0 1
	Median	140 5	-0.5	141.0	1 0	141 0	0 0
	SD	0 58	0 58	1.26	0 50	0 92	0 83
	(SE)	(0.29)	(0.29)	(0 63)	(0.25)	(0.32)	(0 30)
	Min,	140,	-1,	139,	0,	139,	-1,
	Max	141	0	142	1	142	1
Week 44	n	4	4	4	4	8	8
	Mean	140 5	-0 5	139 3	-0.8	139 9	-0 6
	Median	141 5	0 5	139 0	-1 0	139 5	-1.0
	SD	3.11	4 04	0 50	1 26	2.17	2 77
	(SE)	(1 55)	(2 02)	(0.25)	(0 63)	(0 77)	(0.98)
	Min,	136,	-6,	139,	-2,	136,	-6,
	Max	143	3	140	1	143	3
Week 48	n	4	4	4	4	8	8
	Mean	139 5	-1 5	139 5	-0 5	139 5	-1 0
	Median	139 5	-1.5	139 5	-0 5	139 5	-0 5
	SD	1 29	2 38	1 29	1 29	1 20	1 85
	(SE)	(0 65)	(1 19)	(0 65)	(0 65)	(0.42)	(0.65)
	Min,	138,	-4,	138,	-2,	138,	-4,
	Max	141	1	141	1	141	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2 8.1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 52	n	4	4	4	4	8	8
	Mean	138 8	-2 3	138 5	-1.5	138.6	-1 9
	Median	138 5	-2 0	138 5	-1 5	138.5	-2.0
	SD	0 96	1 26	1 29	0.58	1 06	0 99
	(SE)	(0 48)	(0 63)	(0 65)	(0 29)	(0 38)	(0 35)
	Min,	138,	-4,	137,	-2,	137,	-4,
	Max	140	-1	140	-1	140	-1
Week 56	n	4	4	3	3	7	7
	Mean	138.8	-2 3	140.0	0 3	139 3	-1 1
	Median	138 5	-2 0	139 0	0 0	139 0	-1 0
	SD	0 96	1 26	1.73	1 53	1 38	1 86
	(SE)	(0 48)	(0 63)	(1 00)	(0 88)	(0 52)	(0.70)
	Min,	138,	-4,	139,	-1,	138,	-4,
	Max	140	-1	142	2	142	2
Week 60	n	4	4	4	4	8	8
	Mean	138 8	-2.3	139 5	-0.5	139.1	-1 4
	Median	138 5	-2 0	139 5	-1.0	139 0	-1 0
	SD	1 71	1 50	1 29	1 00	1 46	1 51
	(SE)	(0 85)	(0 75)	(0.65)	(0 50)	(0 52)	(0 53)
	Min,	137,	-4,	138,	-1,	137,	-4,
	Max	141	-1	141	1	141	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 64	n	4	4	4	4	8	8
	Mean	139 5	-1 5	139 3	-0 8	139.4	-1 1
	Median	140.5	-1.5	139 5	-0 5	140 0	-1 0
	SD	2 38	2 08	0.96	0.96	1.69	1 55
	(SE)	(1 19)	(1 04)	(0 48)	(0 48)	(0 60)	(0 55)
	Min,	136,	-4,	138,	-2,	136,	-4,
	Max	141	1	140	0	141	1
Week 68	n	4	4	4	4	8	8
	Mean	137 8	-3 3	139 5	-0 5	138.6	-1 9
	Median	138 0	-3 5	139 5	-1 0	139 0	-1.0
	SD	1 50	1.71	0 58	1 00	1 41	1 96
	(SE)	(0 75)	(0 85)	(0.29)	(0 50)	(0.50)	(0 69)
	Min,	136,	-5,	139,	-1,	136,	-5,
	Max	139	-1	140	1	140	1
Week 72	n	3	3	4	4	7	7
	Mean	138 3	-2 3	139 3	-0 8	138 9	-1 4
	Median	140 0	-2 0	139.5	-0 5	140 0	-1 0
	SD	2 89	2 52	1 71	0 96	2.12	1 81
	(SE)	(1 67)	(1 45)	(0 85)	(0.48)	(0 80)	(0 69)
	Min,	135,	-5,	137,	-2,	135,	-5,
	Max	140	0	141	0	141	0

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015:14 03
Reference listing L.16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 73	n	2	2	0	0	2	2
	Mean	139 0	-2.0			139 0	-2 0
	Median	139 0	-2 0			139.0	-2 0
	SD	0 00	1.41			0 00	1 41
	(SE)	(0 00)	(1 00)			(0 00)	(1 00)
	Min,	139,	-3,			139,	-3,
	Max	139	-1			139	-1
Week 76	n	4	4	4	4	8	8
	Mean	140 0	-1.0	139 3	-0.8	139 6	-0 9
	Median	139 5	-1 0	139 0	-1 0	139.0	-1 0
	SD	1 41	0 82	2 06	2 87	1 69	1 96
	(SE)	(0 71)	(0 41)	(1 03)	(1 44)	(0 60)	(0 69)
	Min,	139,	-2,	137,	-4,	137,	-4,
	Max	142	0	142	3	142	3
Week 80	n	4	4	4	4	8	8
	Mean	138 0	-3.0	139 0	-1 0	138 5	-2 0
	Median	137.5	-3 5	139 5	-0 5	138 0	-2 0
	SD	2 94	2 45	2 45	3 16	2.56	2 83
	(SE)	(1 47)	(1 22)	(1 22)	(1 58)	(0 91)	(1 00)
	Min,	135,	-5,	136,	-5,	135,	-5,
	Max	142	0	141	2	142	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 84	n	4	4	4	4	8	8
	Mean	140 0	-1.0	139 0	-1 0	139 5	-1 0
	Median	139 0	-1 0	139 0	-0 5	139 0	-1 0
	SD	2 00	1.63	0 82	1 41	1 51	1.41
	(SE)	(1.00)	(0 82)	(0 41)	(0 71)	(0 53)	(0.50)
	Min,	139,	-3,	138,	-3,	138,	-3,
	Max	143	1	140	0	143	1
Week 88	n	4	4	4	4	8	8
	Mean	139.3	-1.8	138.8	-1 3	139 0	-1 5
	Median	139 5	-1 5	139 0	-1 5	139 0	-1 5
	SD	3.50	2.50	2 22	1 71	2 73	2 00
	(SE)	(1 75)	(1 25)	(1 11)	(0.85)	(0 96)	(0 71)
	Min,	135,	-5,	136,	-3,	135,	-5,
	Max	143	1	141	1	143	1
Week 92	n	4	4	4	4	8	8
	Mean	141 0	0 0	138 8	-1.3	139 9	-0 6
	Median	142 0	1 0	138.5	-1 0	140.0	-0 5
	SD	3 46	2 83	1 71	2.06	2 80	2 39
	(SE)	(1 73)	(1 41)	(0 85)	(1 03)	(0 99)	(0 84)
	Min,	136,	-4,	137,	-4,	136,	-4,
	Max	144	2	141	1	144	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing: L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 96	n	4	4	4	4	8	8
	Mean	141 0	0.0	138 3	-1 8	139.6	-0.9
	Median	140 0	0 0	138.0	-2.5	139.0	-1 5
	SD	2 83	2 58	2 22	1 89	2 77	2 30
	(SE)	(1 41)	(1 29)	(1.11)	(0 95)	(0.98)	(0 81)
	Min,	139,	-3,	136,	-3,	136,	-3,
	Max	145	3	141	1	145	3
Week 100	n	4	4	4	4	8	8
	Mean	138 5	-2 5	139 8	-0 3	139 1	-1 4
	Median	138 0	-2 0	139 5	-1 0	138.5	-2 0
	SD	1 00	1 00	1 71	2 36	1.46	2 07
	(SE)	(0 50)	(0 50)	(0 85)	(1 18)	(0 52)	(0 73)
	Min,	138,	-4,	138,	-2,	138,	-4,
	Max	140	-2	142	3	142	3
Week 104	n	4	4	4	4	8	8
	Mean	140 0	-1 0	138.0	-2 0	139 0	-1 5
	Median	140 0	-1 5	137 5	-2 0	138.5	-2 0
	SD	1 83	2.16	2 16	1.63	2 14	1 85
	(SE)	(0.91)	(1 08)	(1 08)	(0 82)	(0 76)	(0 65)
	Min,	138,	-3,	136,	-4,	136,	-4,
	Max	142	2	141	0	142	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14:03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 108	n	4	4	4	4	8	8
	Mean	139 5	-1 5	140 0	0 0	139.8	-0 8
	Median	139 5	-1 5	140 0	0 0	139 5	-1.5
	SD	1 29	0 58	2 31	2 94	1 75	2 12
	(SE)	(0.65)	(0 29)	(1.15)	(1 47)	(0.62)	(0 75)
	Min.	138.	-2.	138.	-3.	138.	-3.
	Max	141	-1	142	3	142	3
Week 112	n	4	4	4	4	8	8
	Mean	139.3	-1 8	140 0	0 0	139 6	-0 9
	Median	139 5	-1 5	140 0	-0 5	139.5	-1 5
	SD	2.50	2 06	2 94	3 56	2 56	2 85
	(SE)	(1 25)	(1 03)	(1 47)	(1 78)	(0 91)	(1 01)
	Min.	136.	-4.	137.	-3.	136.	-4.
	Max	142	0	143	4	143	4
Week 116	n	4	4	4	4	8	8
	Mean	140 3	-0.8	138 0	-2 0	139 1	-1 4
	Median	139.0	-2 0	138.0	-2 0	138 5	-2 0
	SD	3 95	3.30	1 63	1.83	3 04	2 56
	(SE)	(1 97)	(1 65)	(0 82)	(0 91)	(1 08)	(0 91)
	Min.	137.	-3.	136.	-4.	136.	-4.
	Max	146	4	140	0	146	4

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L.16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 120	n	3	3	4	4	7	7
	Mean	139 0	-1 7	139 0	-1 0	139 0	-1 3
	Median	140 0	0 0	139 0	-0.5	140 0	0 0
	SD	1 73	2 89	1 83	2.45	1 63	2 43
	(SE)	(1 00)	(1.67)	(0 91)	(1 22)	(0 62)	(0 92)
	Min,	137,	-5,	137,	-4,	137,	-5,
	Max	140	0	141	1	141	1
Week 124	n	3	3	4	4	7	7
	Mean	139.3	-1.3	139 3	-0 8	139.3	-1 0
	Median	139 0	-1 0	139 0	-1 0	139 0	-1 0
	SD	0 58	0.58	1 50	2 22	1.11	1 63
	(SE)	(0 33)	(0 33)	(0 75)	(1 11)	(0 42)	(0 62)
	Min,	139,	-2,	138,	-3,	138,	-3,
	Max	140	-1	141	2	141	2
Week 128	n	4	4	4	4	8	8
	Mean	139 3	-1.8	140.3	0 3	139 8	-0.8
	Median	139 0	-1.5	140 0	0.0	139 5	-1 0
	SD	0 50	0.96	1 26	1 50	1.04	1 58
	(SE)	(0 25)	(0 48)	(0 63)	(0 75)	(0 37)	(0 56)
	Min,	139,	-3,	139,	-1,	139,	-3,
	Max	140	-1	142	2	142	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 132	n	4	4	4	4	8	8
	Mean	137 5	-3 5	139.3	-0 8	138 4	-2 1
	Median	137 0	-3 5	139 0	-0.5	137 5	-2 5
	SD	1 73	1 29	2 22	2 06	2 07	2 17
	(SE)	(0 87)	(0.65)	(1 11)	(1 03)	(0 73)	(0.77)
	Min,	136.	-5,	137,	-3,	136,	-5,
	Max	140	-2	142	1	142	1
Week 136	n	4	4	4	4	8	8
	Mean	137 5	-3 5	140 5	0.5	139.0	-1.5
	Median	137 5	-3 5	141 0	0 5	138 5	-1 5
	SD	0 58	1 29	1.00	1 29	1 77	2 45
	(SE)	(0.29)	(0.65)	(0 50)	(0 65)	(0 63)	(0 87)
	Min,	137,	-5,	139,	-1,	137,	-5,
	Max	138	-2	141	2	141	2
Week 140	n	4	4	4	4	8	8
	Mean	139.8	-1.3	139 5	-0.5	139 6	-0.9
	Median	139 5	-1.0	139 0	-0 5	139.0	-1 0
	SD	0 96	0.50	1 00	0.58	0 92	0 64
	(SE)	(0 48)	(0 25)	(0 50)	(0 29)	(0 32)	(0.23)
	Min,	139,	-2,	139,	-1,	139,	-2,
	Max	141	-1	141	0	141	0

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015 14:03
Reference listing: L 16.2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 144	n	4	4	4	4	8	8
	Mean	137.5	-3 5	141 5	1 5	139 5	-1.0
	Median	137 5	-3.5	141 0	1 0	139 5	-1.5
	SD	1 29	0 58	1.91	1 73	2.62	2 93
	(SE)	(0 65)	(0 29)	(0.96)	(0 87)	(0 93)	(1 04)
	Min,	136,	-4,	140,	0,	136,	-4,
	Max	139	-3	144	4	144	4
Week 148	n	4	4	4	4	8	8
	Mean	138.5	-2 5	138 5	-1 5	138 5	-2 0
	Median	139 0	-3 0	138 0	-1 5	138 5	-2 5
	SD	1 00	1.00	1 00	1.29	0.93	1 20
	(SE)	(0 50)	(0 50)	(0 50)	(0.65)	(0 33)	(0 42)
	Min,	137,	-3,	138,	-3,	137,	-3,
	Max	139	-1	140	0	140	0
Week 152	n	4	4	3	3	7	7
	Mean	137 5	-3 5	138 0	-2.3	137.7	-3 0
	Median	138 0	-3.0	137 0	-3 0	138 0	-3 0
	SD	1 00	1 91	1 73	1 15	1 25	1 63
	(SE)	(0 50)	(0 96)	(1.00)	(0.67)	(0 47)	(0 62)
	Min,	136,	-6,	137,	-3,	136,	-6,
	Max	138	-2	140	-1	140	-1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 156	n	4	4	4	4	8	8
	Mean	138.8	-2 3	138 0	-2 0	138 4	-2 1
	Median	139 0	-2 5	138 0	-1 5	139 0	-2 0
	SD	0 50	0 96	1 15	1 41	0 92	1 13
	(SE)	(0 25)	(0 48)	(0 58)	(0.71)	(0.32)	(0.40)
	Min,	138,	-3,	137,	-4,	137,	-4,
	Max	139	-1	139	-1	139	-1
Week 160	n	4	4	4	4	8	8
	Mean	138 0	-3 0	138 0	-2 0	138 0	-2 5
	Median	137 0	-3 0	138 0	-2 5	137 0	-3 0
	SD	2 00	1 63	1 83	2 16	1.77	1 85
	(SE)	(1 00)	(0 82)	(0 91)	(1 08)	(0.63)	(0 65)
	Min,	137,	-5,	136,	-4,	136,	-5,
	Max	141	-1	140	1	141	1
Week 164	n	4	4	4	4	8	8
	Mean	139 0	-2 0	139 3	-0 8	139 1	-1 4
	Median	139 5	-2 5	139 5	-0 5	139 5	-1 5
	SD	1 41	1 41	0 96	1 71	1 13	1 60
	(SE)	(0 71)	(0 71)	(0 48)	(0 85)	(0 40)	(0 56)
	Min,	137,	-3,	138,	-3,	137,	-3,
	Max	140	0	140	1	140	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14:03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 168	n	4	4	4	4	8	8
	Mean	139 0	-2 0	139 5	-0.5	139 3	-1 3
	Median	138 0	-2 0	139 5	0 0	138.5	-1.0
	SD	2 71	2 45	2 08	1.73	2 25	2 12
	(SE)	(1 35)	(1.22)	(1 04)	(0 87)	(0 80)	(0 75)
	Min,	137,	-5,	137,	-3,	137,	-5,
	Max	143	1	142	1	143	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L.16 2 8 1 1.1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Potassium (mmol/L)							
Baseline [1]	n	4		4		8	
	Mean	4 25		3.85		4 05	
	Median	4 20		3 90		4 00	
	SD	0 300		0 173		0 312	
	(SE)	(0 150)		(0.087)		(0 110)	
	Min,	4.0,		3 6,		3.6,	
	Max	4 6		4 0		4 6	
Week 2	n	4	4	4	4	8	8
	Mean	4 00	-0 25	3 60	-0.25	3 80	-0 25
	Median	3 95	-0 25	3 65	-0 15	3 85	-0.15
	SD	0 216	0 465	0 392	0.387	0 363	0 396
	(SE)	(0 108)	(0 233)	(0.196)	(0 194)	(0 128)	(0 140)
	Min,	3.8,	-0 8,	3 1,	-0 8,	3 1,	-0 8,
	Max	4 3	0 3	4 0	0 1	4 3	0 3
Week 4	n	4	4	4	4	8	8
	Mean	3 93	-0.33	4.05	0 20	3.99	-0 06
	Median	3 90	-0 35	4 20	0 25	4 05	-0 05
	SD	0.222	0.411	0 451	0 535	0 336	0 524
	(SE)	(0.111)	(0.206)	(0 225)	(0 268)	(0 119)	(0 185)
	Min,	3 7,	-0.8,	3 4,	-0 5,	3 4,	-0 8,
	Max	4 2	0 2	4 4	0 8	4.4	0 8

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing, L 16 2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	3 63	-0.63	3 88	0 03	3 75	-0 30
	Median	3 60	-0 65	3 90	0 05	3.80	-0 05
	SD	0 330	0 492	0 222	0 096	0.293	0 478
	(SE)	(0 165)	(0 246)	(0 111)	(0.048)	(0.104)	(0.169)
	Min.	3 3.	-1 2.	3 6.	-0 1.	3 3.	-1 2.
	Max	4.0	0 0	4 1	0 1	4 1	0.1
Week 8	n	4	4	4	4	8	8
	Mean	4 08	-0 18	3 95	0.10	4 01	-0 04
	Median	4 05	-0 20	4.20	0 40	4.05	0 05
	SD	0 171	0 377	0 926	0.902	0 620	0 657
	(SE)	(0 085)	(0 189)	(0 463)	(0 451)	(0.219)	(0.232)
	Min.	3 9.	-0 6.	2 7.	-1 2.	2 7.	-1 2.
	Max	4 3	0 3	4 7	0 8	4.7	0 8
Week 10	n	4	4	4	4	8	8
	Mean	3 88	-0 38	3.68	-0 18	3 78	-0 28
	Median	3 95	-0 25	3 80	0 00	3.90	-0 10
	SD	0.189	0 450	0 403	0 427	0 311	0 420
	(SE)	(0 095)	(0 225)	(0.202)	(0 214)	(0 110)	(0 149)
	Min.	3 6.	-1 0.	3 1.	-0 8.	3 1.	-1.0.
	Max	4 0	0.0	4 0	0 1	4.0	0 1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14 03
Reference listing L 16.2 8 1.1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	4.08	-0 18	3 90	0 05	3 99	-0.06
	Median	4.10	-0 15	3 75	0 00	4 00	0 00
	SD	0 126	0 320	0.337	0 265	0 253	0 297
	(SE)	(0 063)	(0 160)	(0 168)	(0 132)	(0 090)	(0 105)
	Min,	3 9,	-0 5,	3 7,	-0.2,	3 7,	-0.5,
	Max	4 2	0.1	4 4	0 4	4 4	0 4
Week 15	n	4	4	4	4	8	8
	Mean	3 73	-0 53	3 50	-0 35	3 61	-0 44
	Median	3.75	-0 45	3 40	-0 40	3.65	-0 40
	SD	0 096	0.359	0 271	0 191	0.223	0 283
	(SE)	(0 048)	(0 180)	(0.135)	(0 096)	(0 079)	(0 100)
	Min,	3.6,	-1 0,	3 3,	-0.5,	3 3,	-1 0,
	Max	3 8	-0 2	3 9	-0 1	3 9	-0 1
Week 18	n	4	4	4	4	8	8
	Mean	4 05	-0 20	3 98	0 13	4 01	-0 04
	Median	4 00	-0 20	4 05	0 10	4 00	0 00
	SD	0 173	0 440	0 263	0 377	0.210	0.417
	(SE)	(0.087)	(0 220)	(0 131)	(0.189)	(0 074)	(0 148)
	Min,	3.9,	-0 7,	3 6,	-0 3,	3 6,	-0 7,
	Max	4 3	0 3	4 2	0 6	4 3	0 6

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 21	n	4	4	4	4	8	8
	Mean	3 68	-0.58	3.75	-0 10	3 71	-0.34
	Median	3.70	-0 50	3 75	-0 15	3.70	-0 25
	SD	0 150	0 386	0 311	0 216	0 230	0 385
	(SE)	(0 075)	(0.193)	(0 155)	(0 108)	(0 081)	(0 136)
	Min,	3.5,	-1 1,	3 4,	-0 3,	3.4,	-1 1,
	Max	3 8	-0 2	4 1	0 2	4 1	0 2
Week 24	n	4	4	4	4	8	8
	Mean	3 95	-0.30	3 58	-0 28	3 76	-0 29
	Median	4 00	-0.40	3 60	-0 30	3 75	-0 35
	SD	0 252	0 346	0 222	0.126	0 297	0 242
	(SE)	(0.126)	(0.173)	(0 111)	(0 063)	(0 105)	(0 085)
	Min,	3 6,	-0 6,	3 3,	-0 4,	3.3,	-0.6,
	Max	4.2	0 2	3 8	-0 1	4 2	0 2
Week 25	n	4	4	4	4	8	8
	Mean	3 73	-0 53	3 60	-0 25	3 66	-0.39
	Median	3.80	-0 70	3 60	-0.30	3.70	-0 45
	SD	0 310	0 350	0.183	0 265	0 245	0 323
	(SE)	(0 155)	(0 175)	(0 091)	(0.132)	(0.086)	(0 114)
	Min,	3 3,	-0 7,	3.4,	-0 5,	3 3,	-0 7,
	Max	4 0	0 0	3 8	0 1	4 0	0 1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015·14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 28	n	4	4	4	4	8	8
	Mean	3 80	-0 45	3.50	-0.35	3 65	-0 40
	Median	3 80	-0 50	3.45	-0.35	3 70	-0 40
	SD	0 163	0 342	0 216	0 129	0 239	0 245
	(SE)	(0 082)	(0 171)	(0 108)	(0 065)	(0 085)	(0 087)
	Min,	3.6,	-0 8,	3.3,	-0.5,	3 3,	-0 8,
	Max	4 0	0 0	3 8	-0.2	4 0	0 0
Week 32	n	4	4	3	3	7	7
	Mean	4 10	-0 15	3 97	0 17	4 04	-0 01
	Median	4 10	-0 10	4 00	0 20	4 10	0 00
	SD	0 082	0 265	0.153	0 252	0 127	0 291
	(SE)	(0 041)	(0 132)	(0 088)	(0 145)	(0 048)	(0.110)
	Min,	4 0,	-0.5,	3 8,	-0.1,	3 8,	-0 5,
	Max	4 2	0 1	4 1	0 4	4 2	0 4
Week 36	n	4	4	4	4	8	8
	Mean	4 08	-0 18	4 00	0.15	4 04	-0 01
	Median	4 15	-0 05	4 00	0 15	4.00	0.15
	SD	0 506	0 806	0 183	0 311	0 354	0 591
	(SE)	(0 253)	(0 403)	(0 091)	(0 155)	(0 125)	(0.209)
	Min,	3 5,	-1 1,	3.8,	-0 2,	3 5,	-1 1,
	Max	4 5	0 5	4 2	0 5	4 5	0 5

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 40	n	4	4	4	4	8	8
	Mean	4 20	-0.05	3.88	0 03	4 04	-0 01
	Median	4 15	-0 15	3.90	0 05	4 10	0 05
	SD	0 455	0.656	0 263	0 171	0 385	0 445
	(SE)	(0 227)	(0 328)	(0 131)	(0 085)	(0 136)	(0 157)
	Min,	3 7,	-0 7,	3 6,	-0 2,	3.6,	-0 7,
	Max	4 8	0 8	4 1	0.2	4 8	0 8
Week 44	n	4	4	4	4	8	8
	Mean	3 93	-0 33	4 05	0 20	3 99	-0.06
	Median	3.85	-0.25	4.10	0 30	4 00	0 15
	SD	0 538	0 574	0.443	0 424	0 461	0 545
	(SE)	(0 269)	(0 287)	(0 222)	(0 212)	(0 163)	(0 193)
	Min,	3.4,	-1 0,	3 5,	-0 4,	3 4,	-1 0,
	Max	4 6	0 2	4 5	0 6	4 6	0 6
Week 48	n	4	4	4	4	8	8
	Mean	4 05	-0 20	4 05	0 20	4 05	0 00
	Median	4 00	-0 10	4 00	0.10	4.00	0 10
	SD	0 661	0 712	0 100	0 271	0 438	0 542
	(SE)	(0 330)	(0.356)	(0 050)	(0 135)	(0 155)	(0 192)
	Min,	3 4,	-1 0,	4 0,	0 0,	3 4,	-1 0,
	Max	4.8	0.4	4.2	0 6	4 8	0.6

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015:14 03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 52	n	4	4	4	4	8	8
	Mean	4 23	-0.03	4 18	0 33	4 20	0.15
	Median	4 20	0 00	4.20	0 30	4 20	0 10
	SD	0 492	0 634	0 330	0 443	0 389	0 540
	(SE)	(0.246)	(0 317)	(0 165)	(0.221)	(0 138)	(0 191)
	Min,	3 8,	-0 8,	3.8,	-0 1,	3.8,	-0 8,
	Max	4 7	0.7	4.5	0 8	4 7	0 8
Week 56	n	4	4	3	3	7	7
	Mean	4 08	-0 18	4 03	0 10	4 06	-0 06
	Median	4 10	-0.10	4.10	0.20	4 10	0 20
	SD	0 222	0 519	0.306	0.265	0 237	0 424
	(SE)	(0 111)	(0 259)	(0 176)	(0 153)	(0.090)	(0 160)
	Min,	3 8,	-0 8,	3 7,	-0 2,	3 7,	-0 8,
	Max	4 3	0 3	4 3	0 3	4 3	0 3
Week 60	n	4	4	4	4	8	8
	Mean	4 23	-0 03	4 05	0 20	4.14	0 09
	Median	4 30	-0 10	4.10	0 15	4 30	-0 05
	SD	0 222	0 299	0 370	0 469	0.297	0 383
	(SE)	(0 111)	(0 149)	(0 185)	(0 235)	(0 105)	(0 136)
	Min,	3 9,	-0 3,	3 6,	-0 3,	3 6,	-0 3,
	Max	4 4	0.4	4 4	0.8	4 4	0 8

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing L.16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 64	n	4	4	4	4	8	8
	Mean	4 10	-0.15	4 13	0 28	4 11	0 06
	Median	4 00	-0 20	4 20	0.25	4 05	0 10
	SD	0 200	0 443	0.236	0 330	0.203	0 427
	(SE)	(0 100)	(0 222)	(0 118)	(0 165)	(0 072)	(0 151)
	Min,	4 0,	-0 6,	3 8,	-0 1,	3 8,	-0.6,
	Max	4 4	0 4	4 3	0 7	4 4	0 7
Week 68	n	4	4	4	4	8	8
	Mean	3 88	-0 38	4 25	0 40	4 06	0 01
	Median	3 95	-0.35	4 20	0 35	4.10	0 15
	SD	0 189	0 386	0 100	0 141	0.245	0.494
	(SE)	(0 095)	(0 193)	(0 050)	(0 071)	(0 086)	(0 175)
	Min,	3 6,	-0 8,	4.2,	0 3,	3 6,	-0 8,
	Max	4 0	0 0	4.4	0 6	4.4	0 6
Week 72	n	3	3	4	4	7	7
	Mean	4.53	0 33	4.03	0 18	4 24	0 24
	Median	3 80	-0 20	4 05	0.15	4 00	0 10
	SD	1 358	1 570	0 171	0 330	0.838	0 940
	(SE)	(0.784)	(0.906)	(0 085)	(0 165)	(0 317)	(0 355)
	Min,	3 7,	-0 9,	3 8,	-0 2,	3 7,	-0.9,
	Max	6 1	2 1	4 2	0 6	6 1	2 1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 73	n	2	2	0	0	2	2
	Mean	4 55	0.35			4 55	0 35
	Median	4 55	0 35			4 55	0 35
	SD	0 212	0 495			0 212	0 495
	(SE)	(0.150)	(0 350)			(0.150)	(0 350)
	Min,	4 4,	0 0,			4.4,	0 0,
	Max	4 7	0 7			4 7	0 7
Week 76	n	4	4	4	4	8	8
	Mean	4 08	-0 18	3 88	0 03	3 98	-0 08
	Median	4 05	-0 15	3.90	0 05	4 00	0.00
	SD	0 250	0 506	0 222	0.096	0 243	0 354
	(SE)	(0 125)	(0 253)	(0 111)	(0.048)	(0.086)	(0 125)
	Min,	3.8,	-0.8,	3 6,	-0 1,	3 6,	-0 8,
	Max	4 4	0 4	4.1	0 1	4 4	0 4
Week 80	n	4	4	4	4	8	8
	Mean	4 15	-0.10	3 83	-0 03	3 99	-0 06
	Median	4 05	-0 15	3 80	-0 05	3 85	-0 05
	SD	0 387	0 627	0.050	0 171	0 309	0 427
	(SE)	(0 194)	(0 314)	(0 025)	(0 085)	(0 109)	(0 151)
	Min,	3.8,	-0 8,	3 8,	-0 2,	3 8,	-0 8,
	Max	4 7	0 7	3.9	0.2	4 7	0 7

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 84	n	4	4	4	4	8	8
	Mean	4 20	-0 05	3.88	0 02	4 04	-0 01
	Median	4 15	-0.05	3 95	0 00	4 00	-0 05
	SD	0 245	0 451	0 340	0 222	0 325	0 331
	(SE)	(0 122)	(0 225)	(0 170)	(0.111)	(0 115)	(0 117)
	Min,	4 0,	-0 6,	3.4,	-0 2,	3 4,	-0 6,
	Max	4 5	0.5	4.2	0 3	4 5	0 5
Week 88	n	4	4	4	4	8	8
	Mean	4.18	-0 08	4 45	0 60	4 31	0 26
	Median	4 10	-0.10	4 20	0 45	4 15	0 30
	SD	0 310	0 585	0 574	0 483	0 452	0 614
	(SE)	(0 155)	(0 293)	(0.287)	(0.242)	(0 160)	(0 217)
	Min,	3 9,	-0 7,	4 1,	0 2,	3 9,	-0 7,
	Max	4 6	0 6	5 3	1 3	5 3	1 3
Week 92	n	4	4	4	4	8	8
	Mean	4 28	0 03	4 00	0 15	4.14	0 09
	Median	4 30	0 15	4 00	0.05	4 00	0 05
	SD	0 443	0 602	0 245	0 387	0.362	0 473
	(SE)	(0 221)	(0.301)	(0 122)	(0 194)	(0 128)	(0 167)
	Min,	3 8,	-0.8,	3 7,	-0 2,	3 7,	-0 8,
	Max	4 7	0 6	4 3	0 7	4 7	0 7

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015·14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 96	n	4	4	4	4	8	8
	Mean	4 20	-0 05	4 00	0 15	4 10	0 05
	Median	4 00	-0.25	4 10	0 25	4 05	0 10
	SD	0 469	0.686	0 271	0 387	0.370	0 526
	(SE)	(0.235)	(0 343)	(0 135)	(0 194)	(0 131)	(0.186)
	Min,	3 9,	-0 6,	3 6,	-0 4,	3 6,	-0 6,
	Max	4 9	0 9	4 2	0 5	4 9	0 9
Week 100	n	4	4	4	4	8	8
	Mean	4 18	-0 08	4 15	0 30	4 16	0 11
	Median	4 10	0.00	4 15	0.20	4.15	0 20
	SD	0.486	0 519	0.208	0 346	0 346	0 455
	(SE)	(0.243)	(0 259)	(0 104)	(0.173)	(0.122)	(0 161)
	Min,	3 7,	-0 7,	3 9,	0 0,	3 7,	-0 7,
	Max	4.8	0 4	4.4	0 8	4 8	0 8
Week 104	n	4	4	4	4	8	8
	Mean	4 00	-0 25	4 20	0.35	4 10	0 05
	Median	4 00	-0 20	4 05	0 10	4.00	-0 05
	SD	0.183	0 332	0 497	0.645	0.363	0.573
	(SE)	(0 091)	(0.166)	(0 248)	(0 323)	(0 128)	(0 203)
	Min,	3 8,	-0.7,	3.8,	-0 1,	3 8,	-0.7,
	Max	4 2	0.1	4.9	1 3	4 9	1 3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015·14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 108	n	4	4	4	4	8	8
	Mean	3 88	-0.38	3 93	0.08	3 90	-0 15
	Median	3.80	-0 40	3 95	0 00	3.95	-0 15
	SD	0 340	0 532	0 250	0.377	0 278	0 490
	(SE)	(0.170)	(0 266)	(0 125)	(0 189)	(0.098)	(0 173)
	Min,	3 6,	-1 0,	3 6,	-0 3,	3 6,	-1 0,
	Max	4 3	0 3	4.2	0.6	4 3	0 6
Week 112	n	4	4	4	4	8	8
	Mean	3 95	-0 30	3 95	0 10	3.95	-0 10
	Median	3 95	-0 30	4 05	0 20	4 05	-0 10
	SD	0 238	0 408	0 311	0.432	0 256	0 444
	(SE)	(0 119)	(0 204)	(0 155)	(0 216)	(0 091)	(0 157)
	Min,	3 7,	-0 8,	3 5,	-0.5,	3 5,	-0 8,
	Max	4 2	0 2	4 2	0 5	4 2	0 5
Week 116	n	4	4	4	4	8	8
	Mean	4 28	0 03	4.13	0.28	4 20	0 15
	Median	4 25	0 00	4 10	0 30	4 10	0 20
	SD	0 386	0 538	0 150	0 126	0 283	0.385
	(SE)	(0 193)	(0 269)	(0 075)	(0.063)	(0 100)	(0 136)
	Min,	3 9,	-0 6,	4 0,	0 1,	3.9,	-0 6,
	Max	4 7	0 7	4 3	0 4	4.7	0 7

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 120	n	3	3	4	4	7	7
	Mean	4 00	-0 20	4 20	0 35	4 11	0.11
	Median	3 90	-0.10	4 20	0.30	4 10	0 10
	SD	0 265	0 557	0 183	0.300	0 227	0 485
	(SE)	(0.153)	(0 321)	(0 091)	(0 150)	(0 086)	(0 183)
	Min,	3.8,	-0 8,	4.0,	0 1,	3 8.	-0 8,
	Max	4.3	0.3	4 4	0 7	4.4	0 7
Week 124	n	3	3	4	4	7	7
	Mean	3 93	-0.27	4 10	0 25	4 03	0 03
	Median	3.70	-0 40	4 15	0 35	4 10	0 20
	SD	0.493	0 709	0 294	0 404	0 364	0 571
	(SE)	(0 285)	(0 410)	(0 147)	(0 202)	(0 138)	(0 216)
	Min,	3 6,	-0 9,	3 7,	-0 3,	3 6,	-0 9,
	Max	4 5	0.5	4 4	0 6	4.5	0 6
Week 128	n	4	4	4	4	8	8
	Mean	4 20	-0.05	3.70	-0 15	3 95	-0 10
	Median	4 10	-0 20	3 85	-0 10	3 90	-0 10
	SD	0 346	0 526	0 337	0.412	0 414	0 441
	(SE)	(0 173)	(0 263)	(0.168)	(0 206)	(0 146)	(0 156)
	Min,	3 9,	-0 5,	3 2,	-0 7,	3 2,	-0.7,
	Max	4.7	0 7	3 9	0 3	4 7	0 7

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14.03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 132	n	4	4	4	4	8	8
	Mean	4.25	0 00	4 05	0.20	4 15	0 10
	Median	4 05	-0 20	4 05	0 20	4 05	0 10
	SD	0 507	0 735	0 129	0 183	0.359	0.507
	(SE)	(0 253)	(0.367)	(0.065)	(0 091)	(0 127)	(0 179)
	Min,	3 9,	-0..6,	3.9,	0 0,	3 9,	-0 6,
	Max	5 0	1 0	4 2	0 4	5 0	1 0
Week 136	n	4	4	4	4	8	8
	Mean	3 98	-0 28	3 95	0.10	3 96	-0 09
	Median	4 00	-0.45	3.90	0.15	4.00	0 00
	SD	0 369	0 457	0 191	0.141	0 272	0 372
	(SE)	(0 184)	(0 229)	(0.096)	(0 071)	(0 096)	(0 132)
	Min,	3 5,	-0 6,	3 8,	-0 1,	3 5,	-0 6,
	Max	4 4	0.4	4 2	0 2	4 4	0 4
Week 140	n	4	4	4	4	8	8
	Mean	4 20	-0 05	3 75	-0.10	3 98	-0 08
	Median	4 05	-0 20	3 75	-0 15	3 90	-0 15
	SD	0 337	0 545	0 058	0 141	0.328	0 369
	(SE)	(0 168)	(0 272)	(0 029)	(0 071)	(0 116)	(0 131)
	Min,	4 0,	-0 5,	3 7,	-0 2,	3.7,	-0.5,
	Max	4 7	0 7	3.8	0.1	4 7	0 7

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source, P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing: L.16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 144	n	4	4	4	4	8	8
	Mean	3 95	-0.30	3 83	-0 03	3 89	-0 16
	Median	4.10	-0.20	3.90	0 05	4 00	0.00
	SD	0 451	0 668	0 386	0.465	0 394	0 553
	(SE)	(0.225)	(0 334)	(0 193)	(0 232)	(0 139)	(0 195)
	Min,	3 3,	-1 1,	3 3,	-0.6,	3 3,	-1 1,
	Max	4.3	0 3	4.2	0 4	4.3	0 4
Week 148	n	4	4	4	4	8	8
	Mean	3.93	-0 33	3 90	0 05	3 91	-0 14
	Median	3 90	-0 20	3 85	0.00	3 90	-0 10
	SD	0 330	0.479	0 424	0 342	0.352	0 434
	(SE)	(0 165)	(0 239)	(0.212)	(0 171)	(0 125)	(0 153)
	Min,	3 6,	-1 0,	3 5,	-0 3,	3 5,	-1 0,
	Max	4 3	0 1	4.4	0 5	4 4	0 5
Week 152	n	4	4	3	3	7	7
	Mean	4 18	-0 08	3.90	0 10	4.06	0 00
	Median	4 20	-0 05	3 90	0.10	4 10	0 10
	SD	0 369	0 320	0 200	0 100	0.321	0 252
	(SE)	(0 184)	(0 160)	(0 115)	(0 058)	(0 121)	(0 095)
	Min,	3 7,	-0 4,	3 7,	0 0,	3 7,	-0 4,
	Max	4 6	0 2	4 1	0 2	4 6	0 2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14 03
Reference listing L 16 2 8.1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 156	n	4	4	4	4	8	8
	Mean	4 28	0 03	4 00	0 15	4 14	0 09
	Median	4 25	-0 10	4 00	0 10	4.05	0 05
	SD	0 427	0 556	0 082	0 173	0.320	0 387
	(SE)	(0 214)	(0.278)	(0 041)	(0.087)	(0 113)	(0 137)
	Min,	3 8,	-0 5,	3 9,	0 0,	3 8,	-0 5,
	Max	4 8	0 8	4.1	0 4	4.8	0 8
Week 160	n	4	4	4	4	8	8
	Mean	4 18	-0 08	4 13	0 28	4 15	0 10
	Median	4 20	-0.10	4 15	0 30	4 15	0 20
	SD	0.222	0 369	0 171	0 126	0 185	0 316
	(SE)	(0 111)	(0 184)	(0 085)	(0.063)	(0 065)	(0 112)
	Min,	3 9,	-0 5,	3 9,	0 1,	3.9,	-0.5,
	Max	4 4	0 4	4 3	0 4	4.4	0 4
Week 164	n	4	4	4	4	8	8
	Mean	3 98	-0 28	4 03	0 18	4 00	-0 05
	Median	4 00	-0 25	4 05	0 25	4 05	0 10
	SD	0 556	0.640	0 250	0 287	0 400	0 518
	(SE)	(0 278)	(0 320)	(0 125)	(0 144)	(0 141)	(0 183)
	Min,	3 4,	-1 0,	3 7,	-0 2,	3 4,	-1 0,
	Max	4 5	0 4	4.3	0.4	4 5	0 4

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 168	n	4	4	4	4	8	8
	Mean	3 98	-0.28	3 93	0.08	3.95	-0 10
	Median	3 95	-0 20	3.90	0 05	3 95	-0 10
	SD	0 171	0 299	0 330	0 250	0.245	0 316
	(SE)	(0 085)	(0.149)	(0 165)	(0 125)	(0 087)	(0 112)
	Min,	3.8,	-0 7,	3 6,	-0 2,	3.6,	-0 7,
	Max	4.2	0 0	4 3	0 4	4 3	0 4

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing: L 16 2 8.1 1.1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Chloride (mmol/L)							
Baseline [1]	n	4		4		8	
	Mean	102.5		102 8		102 6	
	Median	102.5		103.0		103.0	
	SD	1 29		2 06		1 60	
	(SE)	(0.65)		(1 03)		(0 56)	
	Min,	101,		100,		100,	
	Max	104		105		105	
Week 2	n	4	4	4	4	8	8
	Mean	103 3	0 8	104 3	1 5	103 8	1 1
	Median	104 0	0 5	105 0	2 0	105 0	1.5
	SD	2 36	2 50	1 50	3 32	1 91	2 75
	(SE)	(1 18)	(1 25)	(0 75)	(1 66)	(0 67)	(0 97)
	Min,	100,	-2,	102,	-3,	100,	-3,
	Max	105	4	105	5	105	5
Week 4	n	4	4	4	4	8	8
	Mean	102.5	0 0	102 3	-0.5	102 4	-0 3
	Median	102 5	0 0	102 0	-0 5	102 0	0 0
	SD	1 29	2 45	0 50	2.08	0 92	2 12
	(SE)	(0 65)	(1 22)	(0.25)	(1 04)	(0 32)	(0 75)
	Min,	101,	-3,	102,	-3,	101,	-3,
	Max	104	3	103	2	104	3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	105 3	2 8	101 8	-1 0	103 5	0 9
	Median	105 0	2 5	102 5	-1 5	104 5	2 0
	SD	0 50	0 96	2.63	3 74	2 56	3 23
	(SE)	(0 25)	(0 48)	(1.31)	(1.87)	(0.91)	(1.14)
	Min,	105,	2,	98,	-5,	98,	-5,
	Max	106	4	104	4	106	4
Week 8	n	4	4	4	4	8	8
	Mean	103 0	0 5	103 8	1 0	103 4	0 8
	Median	102 0	0 5	104 0	2 5	102 5	1.5
	SD	2 71	2 08	2 22	3 37	2 33	2 60
	(SE)	(1 35)	(1 04)	(1.11)	(1.68)	(0 82)	(0 92)
	Min,	101,	-2,	101,	-4,	101,	-4,
	Max	107	3	106	3	107	3
Week 10	n	4	4	4	4	8	8
	Mean	102.3	-0 3	103 3	0 5	102 8	0 1
	Median	101 5	0.0	103 5	0 5	102 5	0 5
	SD	2 63	2 22	0 96	2 89	1.91	2 42
	(SE)	(1 31)	(1 11)	(0 48)	(1 44)	(0.67)	(0 85)
	Min,	100,	-3,	102,	-3,	100,	-3,
	Max	106	2	104	4	106	4

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14:03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	102.5	0 0	101 3	-1 5	101 9	-0.8
	Median	102 0	0.0	101 5	-1.5	102.0	0 0
	SD	1 00	0 82	1 71	2 38	1 46	1 83
	(SE)	(0 50)	(0.41)	(0.85)	(1 19)	(0.52)	(0 65)
	Min,	102,	-1,	99,	-4,	99,	-4,
	Max	104	1	103	1	104	1
Week 15	n	4	4	4	4	8	8
	Mean	103 5	1 0	103 5	0 8	103 5	0 9
	Median	103 5	1 0	103 0	0 0	103 0	0 0
	SD	2 38	2 58	1 00	1 50	1 69	1.96
	(SE)	(1 19)	(1.29)	(0 50)	(0 75)	(0.60)	(0 69)
	Min,	101,	-2,	103,	0,	101,	-2.
	Max	106	4	105	3	106	4
Week 18	n	4	4	4	4	8	8
	Mean	103 5	1 0	102 0	-0.8	102 8	0 1
	Median	103 0	1 0	102 0	-0 5	103.0	1 0
	SD	1 73	0 82	2.58	2.75	2 19	2 10
	(SE)	(0 87)	(0 41)	(1 29)	(1 38)	(0 77)	(0 74)
	Min,	102,	0,	99,	-4,	99,	-4,
	Max	106	2	105	2	106	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 21	n	4	4	4	4	8	8
	Mean	106 0	3.5	106 8	4.0	106 4	3 8
	Median	106 0	3.5	107 0	4 0	106 5	3 5
	SD	1.83	0 58	1 50	1 83	1 60	1 28
	(SE)	(0 91)	(0.29)	(0 75)	(0 91)	(0 56)	(0 45)
	Min,	104,	3,	105,	2,	104,	2,
	Max	108	4	108	6	108	6
Week 24	n	4	4	4	4	8	8
	Mean	104 5	2.0	103 8	1 0	104 1	1.5
	Median	105 0	2 0	104 0	1 0	104 0	1.5
	SD	1.73	0.82	0 50	1 63	1 25	1 31
	(SE)	(0 87)	(0 41)	(0.25)	(0 82)	(0.44)	(0 46)
	Min,	102,	1,	103,	-1,	102,	-1,
	Max	106	3	104	3	106	3
Week 25	n	4	4	4	4	8	8
	Mean	104 0	1 5	104.3	1 5	104 1	1 5
	Median	103 5	2.0	104.5	1 0	104 0	1 5
	SD	2 16	1 91	0 96	2 65	1 55	2 14
	(SE)	(1 08)	(0 96)	(0 48)	(1 32)	(0 55)	(0 76)
	Min,	102,	-1,	103,	-1,	102,	-1,
	Max	107	3	105	5	107	5

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14 03
Reference listing. L 16 2 8.1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 28	n	4	4	4	4	8	8
	Mean	105 5	3 0	104 5	1 8	105 0	2 4
	Median	105 5	3 0	104.5	1 0	105.0	2 5
	SD	0 58	0.82	1 29	3 10	1 07	2 20
	(SE)	(0 29)	(0.41)	(0 65)	(1 55)	(0 38)	(0 78)
	Min,	105,	2,	103,	-1,	103,	-1,
	Max	106	4	106	6	106	6
Week 32	n	4	4	3	3	7	7
	Mean	102 3	-0 3	102 3	-1.3	102.3	-0 7
	Median	102 5	-0.5	102.0	-2 0	102 0	-1 0
	SD	0 96	0.96	1 53	2 08	1 11	1 50
	(SE)	(0 48)	(0 48)	(0 88)	(1 20)	(0 42)	(0 57)
	Min,	101,	-1,	101,	-3,	101,	-3,
	Max	103	1	104	1	104	1
Week 36	n	4	4	4	4	8	8
	Mean	101 8	-0 8	101 3	-1.5	101.5	-1 1
	Median	101 5	-0.5	101 5	-1 5	101 5	-1 0
	SD	1 71	1 71	0 96	2 89	1 31	2 23
	(SE)	(0 85)	(0 85)	(0.48)	(1 44)	(0 46)	(0.79)
	Min,	100,	-3,	100,	-5,	100,	-5,
	Max	104	1	102	2	104	2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run: 06MAR2015 14:03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 40	n	4	4	4	4	8	8
	Mean	102 5	0 0	102 5	-0.3	102.5	-0.1
	Median	102 0	0 0	102.5	-0 5	102 5	-0 5
	SD	1 91	1.83	2.08	1.71	1.85	1 64
	(SE)	(0 96)	(0 91)	(1 04)	(0 85)	(0 65)	(0 58)
	Min,	101,	-2,	100,	-2,	100,	-2,
	Max	105	2	105	2	105	2
Week 44	n	4	4	4	4	8	8
	Mean	101 8	-0 8	102 8	0 0	102 3	-0 4
	Median	102 0	-0 5	103.0	-0 5	103 0	-0 5
	SD	1 50	2 50	0 50	2 16	1.16	2 20
	(SE)	(0 75)	(1 25)	(0 25)	(1.08)	(0.41)	(0 78)
	Min,	100,	-4,	102,	-2,	100,	-4,
	Max	103	2	103	3	103	3
Week 48	n	4	4	4	4	8	8
	Mean	102.3	-0 3	101.5	-1 3	101 9	-0 8
	Median	102 0	-0 5	101 5	-2.0	102 0	-1 0
	SD	0 50	1 71	1.29	3.10	0 99	2 38
	(SE)	(0 25)	(0 85)	(0 65)	(1.55)	(0 35)	(0 84)
	Min,	102,	-2,	100,	-4,	100,	-4,
	Max	103	2	103	3	103	3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 52	n	4	4	4	4	8	8
	Mean	101 5	-1 0	101 5	-1 3	101 5	-1.1
	Median	101 5	-1 5	101 5	-0 5	101 5	-1 0
	SD	1 29	2 16	1 29	1 89	1 20	1.89
	(SE)	(0 65)	(1 08)	(0 65)	(0 95)	(0 42)	(0.67)
	Min,	100,	-3,	100,	-4,	100,	-4,
	Max	103	2	103	0	103	2
Week 56	n	4	4	3	3	7	7
	Mean	101 3	-1 3	103 0	0 3	102 0	-0 6
	Median	101.0	-1 5	103 0	1 0	102.0	-1 0
	SD	0 50	1 71	1 00	3.06	1 15	2 30
	(SE)	(0 25)	(0 85)	(0 58)	(1 76)	(0 44)	(0.87)
	Min,	101,	-3,	102,	-3,	101,	-3,
	Max	102	1	104	3	104	3
Week 60	n	4	4	4	4	8	8
	Mean	101.0	-1 5	103.8	1 0	102 4	-0 3
	Median	101 0	-1 5	104 0	1.0	102 0	-0 5
	SD	0 82	1 29	1 50	3 37	1 85	2 71
	(SE)	(0 41)	(0 65)	(0 75)	(1 68)	(0 65)	(0 96)
	Min,	100,	-3,	102,	-3,	100,	-3,
	Max	102	0	105	5	105	5

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 64	n	4	4	4	4	8	8
	Mean	100 5	-2 0	101 8	-1 0	101 1	-1 5
	Median	100 0	-2 0	101 5	-0 5	101 0	-1.0
	SD	1 00	1 83	0 96	2 16	1.13	1 93
	(SE)	(0 50)	(0.91)	(0 48)	(1.08)	(0.40)	(0 68)
	Min,	100,	-4,	101,	-4,	100,	-4,
	Max	102	0	103	1	103	1
Week 68	n	4	4	4	4	8	8
	Mean	100 5	-2.0	102 0	-0.8	101 3	-1 4
	Median	100 5	-2 5	102 0	-0 5	101 5	-1 5
	SD	1 29	2.16	0 82	1.71	1 28	1.92
	(SE)	(0 65)	(1 08)	(0.41)	(0 85)	(0 45)	(0 68)
	Min,	99,	-4,	101,	-3,	99,	-4,
	Max	102	1	103	1	103	1
Week 72	n	3	3	4	4	7	7
	Mean	100 7	-1 3	102 0	-0.8	101 4	-1 0
	Median	101 0	-1 0	102 0	-1.0	101.0	-1 0
	SD	0 58	1 53	0 82	1 26	0 98	1 29
	(SE)	(0 33)	(0 88)	(0 41)	(0 63)	(0 37)	(0 49)
	Min,	100,	-3,	101,	-2,	100,	-3,
	Max	101	0	103	1	103	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015:14.03
Reference listing: L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 73	n	2	2	0	0	2	2
	Mean	102.5	0 0			102.5	0.0
	Median	102 5	0 0			102 5	0 0
	SD	0 71	2.83			0 71	2 83
	(SE)	(0 50)	(2 00)			(0 50)	(2 00)
	Min,	102,	-2,			102,	-2,
	Max	103	2			103	2
Week 76	n	4	4	4	4	8	8
	Mean	101.0	-1 5	101 8	-1 0	101 4	-1 3
	Median	101 0	-1 5	102 0	-2 0	101 5	-1 5
	SD	1 63	1 29	1 26	2 83	1.41	2 05
	(SE)	(0 82)	(0 65)	(0 63)	(1 41)	(0 50)	(0 73)
	Min,	99,	-3,	100,	-3,	99,	-3,
	Max	103	0	103	3	103	3
Week 80	n	4	4	4	4	8	8
	Mean	101 0	-1 5	102 5	-0.3	101 8	-0 9
	Median	101 0	-1 0	103 0	-1.0	101 5	-1 0
	SD	0 82	1.00	1 91	3 40	1 58	2 42
	(SE)	(0 41)	(0 50)	(0 96)	(1 70)	(0 56)	(0 85)
	Min,	100,	-3,	100,	-3,	100,	-3,
	Max	102	-1	104	4	104	4

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 84	n	4	4	4	4	8	8
	Mean	101 3	-1 3	100 5	-2 3	100 9	-1 8
	Median	101 0	-1 0	100 5	-3 5	100 5	-2 5
	SD	1 50	2 22	1 29	2 87	1 36	2 43
	(SE)	(0.75)	(1.11)	(0 65)	(1.44)	(0.48)	(0 86)
	Min,	100,	-4,	99,	-4,	99,	-4,
	Max	103	1	102	2	103	2
Week 88	n	4	4	4	4	8	8
	Mean	102.0	-0 5	102.5	-0.3	102 3	-0 4
	Median	101 5	-0 5	102 5	1.0	102 0	0 0
	SD	2.16	1 73	2 89	4 27	2 38	3 02
	(SE)	(1.08)	(0 87)	(1 44)	(2 14)	(0 84)	(1.07)
	Min,	100,	-2,	99,	-6,	99,	-6,
	Max	105	1	106	3	106	3
Week 92	n	4	4	4	4	8	8
	Mean	104 0	1.5	103.0	0.3	103.5	0 9
	Median	104 5	1 5	102.5	0.5	103.5	1 5
	SD	2 16	1 29	1 41	2 75	1 77	2 10
	(SE)	(1.08)	(0.65)	(0 71)	(1.38)	(0 63)	(0 74)
	Min,	101,	0,	102,	-3,	101,	-3,
	Max	106	3	105	3	106	3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run: 06MAR2015 14:03
Reference listing L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 96	n	4	4	4	4	8	8
	Mean	101 8	-0.8	102 8	0 0	102 3	-0 4
	Median	102 0	-1.0	102 5	1.0	102 0	0 0
	SD	0 50	1 26	2 06	2 71	1 49	2 00
	(SE)	(0.25)	(0 63)	(1 03)	(1 35)	(0 53)	(0 71)
	Min,	101,	-2,	101,	-4,	101,	-4,
	Max	102	1	105	2	105	2
Week 100	n	4	4	4	4	8	8
	Mean	100 5	-2 0	101·8	-1 0	101 1	-1 5
	Median	100 5	-2 5	101 5	-0 5	101.5	-1 5
	SD	1 73	2 16	0 96	2 16	1 46	2 07
	(SE)	(0 87)	(1 08)	(0 48)	(1.08)	(0.52)	(0 73)
	Min,	99,	-4,	101,	-4,	99,	-4,
	Max	102	1	103	1	103	1
Week 104	n	4	4	4	4	8	8
	Mean	102.3	-0 3	102.5	-0 3	102 4	-0 3
	Median	102 5	-0 5	103.0	-1 0	103.0	-0 5
	SD	0 96	1 71	1 00	2 36	0 92	1 91
	(SE)	(0 48)	(0 85)	(0 50)	(1.18)	(0 32)	(0 67)
	Min,	101,	-2,	101,	-2,	101,	-2,
	Max	103	2	103	3	103	3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015·14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 108	n	4	4	4	4	8	8
	Mean	102 0	-0 5	102.5	-0.3	102 3	-0 4
	Median	102 0	-1 0	102.5	0.0	102 5	-1 0
	SD	1 15	1 73	2 08	2 63	1 58	2.07
	(SE)	(0.58)	(0 87)	(1.04)	(1 31)	(0 56)	(0 73)
	Min,	101,	-2,	100,	-3,	100,	-3,
	Max	103	2	105	2	105	2
Week 112	n	4	4	4	4	8	8
	Mean	102 8	0.3	103.5	0 8	103 1	0 5
	Median	102 5	0 0	104.0	1 0	102 5	0 0
	SD	1 71	0.50	3.00	4 79	2 30	3 16
	(SE)	(0 85)	(0.25)	(1 50)	(2 39)	(0 81)	(1 12)
	Min,	101,	0,	100,	-5,	100,	-5,
	Max	105	1	106	6	106	6
Week 116	n	4	4	4	4	8	8
	Mean	103 5	1.0	102.0	-0 8	102 8	0 1
	Median	102 0	0 0	101 5	-0 5	102 0	0 0
	SD	3 70	2.71	2 16	2.50	2 92	2 59
	(SE)	(1 85)	(1.35)	(1 08)	(1.25)	(1 03)	(0 91)
	Min,	101,	-1,	100,	-4,	100,	-4,
	Max	109	5	105	2	109	5

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015:14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 120	n	3	3	4	4	7	7
	Mean	103 0	1 0	103 0	0.3	103 0	0 6
	Median	103 0	1.0	103 0	0 0	103 0	1.0
	SD	1 00	2 00	0.82	2.22	0 82	1 99
	(SE)	(0 58)	(1.15)	(0.41)	(1 11)	(0 31)	(0 75)
	Min,	102,	-1,	102,	-2,	102,	-2,
	Max	104	3	104	3	104	3
Week 124	n	3	3	4	4	7	7
	Mean	101 7	-0 3	103 0	0 3	102 4	0 0
	Median	101 0	0 0	102 5	1 0	102.0	0 0
	SD	1 15	0 58	2 16	3 40	1.81	2 45
	(SE)	(0 67)	(0.33)	(1 08)	(1 70)	(0 69)	(0 93)
	Min,	101,	-1,	101,	-4,	101,	-4,
	Max	103	0	106	3	106	3
Week 128	n	4	4	4	4	8	8
	Mean	102 5	0 0	103 5	0 8	103 0	0 4
	Median	102 5	0.0	103.0	1 0	103 0	0 0
	SD	0 58	0.82	1 73	2.63	1 31	1 85
	(SE)	(0 29)	(0 41)	(0 87)	(1 31)	(0.46)	(0 65)
	Min,	102,	-1,	102,	-2,	102,	-2,
	Max	103	1	106	3	106	3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 132	n	4	4	4	4	8	8
	Mean	101 3	-1 3	102 0	-0 8	101 6	-1 0
	Median	100 5	-1.0	101.5	0.0	101 0	-1 0
	SD	1 89	1 50	2 16	3 40	1 92	2 45
	(SE)	(0 95)	(0 75)	(1 08)	(1 70)	(0 68)	(0 87)
	Min,	100,	-3,	100,	-5,	100,	-5,
	Max	104	0	105	2	105	2
Week 136	n	4	4	4	4	8	8
	Mean	100 8	-1 8	103 3	0 5	102.0	-0 6
	Median	100 0	-2 0	103 5	0 5	102 0	-1 0
	SD	2 22	1 50	1 71	2 38	2 27	2 20
	(SE)	(1 11)	(0.75)	(0.85)	(1 19)	(0 80)	(0 78)
	Min,	99,	-3,	101,	-2,	99,	-3,
	Max	104	0	105	3	105	3
Week 140	n	4	4	4	4	8	8
	Mean	102 5	0.0	101 8	-1.0	102 1	-0 5
	Median	103 0	0 0	101 5	-0 5	102.0	0 0
	SD	1.91	0 82	1.71	2 16	1 73	1 60
	(SE)	(0 96)	(0 41)	(0 85)	(1 08)	(0 61)	(0 57)
	Min,	100,	-1,	100,	-4,	100,	-4,
	Max	104	1	104	1	104	1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015:14.03
Reference listing L 16 2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 144	n	4	4	4	4	8	8
	Mean	102 5	0.0	104 0	1.3	103 3	0 6
	Median	101 0	-0 5	104 0	0 5	103 5	0 0
	SD	3 70	2 94	0 82	1.89	2 60	2 39
	(SE)	(1 85)	(1 47)	(0 41)	(0 95)	(0 92)	(0.84)
	Min,	100,	-3,	103,	0,	100,	-3,
	Max	108	4	105	4	108	4
Week 148	n	4	4	4	4	8	8
	Mean	102 0	-0 5	102 3	-0 5	102 1	-0 5
	Median	102 0	-0 5	102.5	-0.5	102 0	-0 5
	SD	1 63	0 58	0.96	1 29	1 25	0.93
	(SE)	(0 82)	(0 29)	(0.48)	(0 65)	(0 44)	(0 33)
	Min,	100,	-1,	101,	-2,	100,	-2,
	Max	104	0	103	1	104	1
Week 152	n	4	4	3	3	7	7
	Mean	101 5	-1 0	102.3	-1 3	101 9	-1 1
	Median	101 0	-1 0	103 0	0 0	101 0	-1 0
	SD	1 00	0 82	1 15	2 31	1 07	1 46
	(SE)	(0 50)	(0 41)	(0 67)	(1 33)	(0 40)	(0 55)
	Min,	101,	-2,	101,	-4,	101,	-4,
	Max	103	0	103	0	103	0

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14.03
Reference listing L 16 2 8.1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 156	n	4	4	4	4	8	8
	Mean	102 3	-0.3	102 3	-0 5	102 3	-0.4
	Median	101 5	0 0	102 5	-0 5	102 0	-0 5
	SD	1 89	1 50	1 71	1 29	1 67	1 30
	(SE)	(0.95)	(0.75)	(0 85)	(0 65)	(0 59)	(0 46)
	Min,	101,	-2,	100,	-2,	100,	-2,
	Max	105	1	104	1	105	1
Week 160	n	4	4	4	4	8	8
	Mean	101 8	-0.8	101 5	-1 3	101 6	-1 0
	Median	101 5	-1.0	101 5	-1 5	101 5	-1 0
	SD	0 96	0 50	1 29	3 30	1 06	2 20
	(SE)	(0 48)	(0 25)	(0 65)	(1 65)	(0 37)	(0 78)
	Min,	101,	-1,	100,	-5,	100,	-5,
	Max	103	0	103	3	103	3
Week 164	n	4	4	4	4	8	8
	Mean	103.0	0.5	103 8	1.0	103 4	0.8
	Median	103 0	0.5	103 5	1 0	103 0	0 5
	SD	1 63	1 29	1.71	2 94	1 60	2 12
	(SE)	(0 82)	(0 65)	(0 85)	(1 47)	(0 56)	(0.75)
	Min,	101,	-1,	102,	-2,	101,	-2,
	Max	105	2	106	4	106	4

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14.03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 168	n	4	4	4	4	8	8
	Mean	103 5	1.0	102 5	-0.3	103 0	0.4
	Median	103 0	0.5	102 5	0.0	103 0	0 5
	SD	2 52	1 41	1 29	2.99	1.93	2 26
	(SE)	(1 26)	(0 71)	(0 65)	(1.49)	(0 68)	(0 80)
	Min,	101,	0.	101,	-4.	101,	-4.
	Max	107	3	104	3	107	3

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Calcium (mmol/L)							
Baseline [1]	n	4		4		8	
	Mean	2.2875		2.3438		2 3156	
	Median	2 3125		2 3500		2.3375	
	SD	0 09682		0.05154		0.07785	
	(SE)	(0 04841)		(0 02577)		(0 02752)	
	Min,	2 150,		2 275,		2 150,	
	Max	2 375		2 400		2 400	
Week 2	n	4	4	4	4	8	8
	Mean	2.3250	0 0375	2.2938	-0 0500	2 3094	-0 0063
	Median	2.3125	0 0625	2.3000	-0.0375	2 3000	-0 0250
	SD	0 07906	0.10104	0.03146	0 03536	0.05815	0 08425
	(SE)	(0.03953)	(0 05052)	(0.01573)	(0 01768)	(0 02056)	(0 02979)
	Min,	2 250,	-0 100,	2 250,	-0 100,	2.250,	-0.100,
	Max	2 425	0 125	2.325	-0 025	2 425	0 125
Week 4	n	4	4	4	4	8	8
	Mean	2 3063	0.0188	2 4125	0 0688	2 3594	0 0438
	Median	2 3125	0.0000	2.4375	0 0625	2 3250	0 0000
	SD	0 02394	0 11434	0 11990	0.09656	0 09814	0 10155
	(SE)	(0 01197)	(0.05717)	(0 05995)	(0 04828)	(0 03470)	(0 03590)
	Min,	2 275,	-0 100,	2 250,	-0 025,	2.250,	-0 100,
	Max	2 325	0 175	2 525	0.175	2 525	0 175

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source. P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015:14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	2 2813	-0 0063	2 2188	-0 1250	2 2500	-0 0656
	Median	2.2750	-0 0125	2.3250	0 0000	2 2750	0 0000
	SD	0.03146	0 10680	0 26800	0.26693	0 17978	0 19863
	(SE)	(0 01573)	(0 05340)	(0 13400)	(0 13346)	(0.06356)	(0 07023)
	Min.	2 250,	-0 125,	1.825,	-0 525,	1 825,	-0 525,
	Max	2 325	0 125	2.400	0.025	2 400	0 125
Week 8	n	4	4	4	4	8	8
	Mean	2 3250	0 0375	2 3125	-0 0313	2.3187	0 0031
	Median	2.3250	0 0500	2.3500	-0 0125	2 3250	0.0000
	SD	0 06124	0 12990	0.11273	0 06575	0 08425	0 10215
	(SE)	(0 03062)	(0 06495)	(0 05637)	(0 03287)	(0.02979)	(0 03612)
	Min.	2 250,	-0 125,	2.150,	-0.125,	2 150,	-0 125,
	Max	2 400	0.175	2 400	0 025	2 400	0 175
Week 10	n	4	4	4	4	8	8
	Mean	2 2938	0 0063	2 2875	-0.0563	2 2906	-0 0250
	Median	2 2875	-0 0625	2 3125	-0 0500	2 2875	-0 0625
	SD	0 08750	0 16378	0 10897	0 09437	0 09155	0 12817
	(SE)	(0 04375)	(0.08189)	(0 05449)	(0 04719)	(0 03237)	(0 04532)
	Min.	2 200,	-0 100,	2 150,	-0 150,	2 150,	-0 150,
	Max	2 400	0 250	2 375	0 025	2 400	0 250

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing: L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	2 3125	0 0250	2 3500	0 0063	2 3313	0 0156
	Median	2 2875	0 0250	2 3250	0 0000	2 2875	0 0000
	SD	0 05951	0 10206	0 11365	0.07465	0 08634	0 08339
	(SE)	(0 02976)	(0 05103)	(0 05683)	(0.03733)	(0 03053)	(0 02948)
	Min,	2.275,	-0 075,	2.250,	-0 075,	2 250,	-0 075,
	Max	2 400	0.125	2 500	0 100	2 500	0.125
Week 15	n	4	4	4	4	8	8
	Mean	2 3000	0 0125	2 3188	-0 0250	2.3094	-0 0062
	Median	2.3000	0 0000	2 3000	-0.0375	2 3000	-0 0125
	SD	0 06124	0 10308	0 09656	0 05401	0 07552	0 07877
	(SE)	(0 03062)	(0.05154)	(0 04828)	(0 02700)	(0 02670)	(0 02785)
	Min,	2.225,	-0 100,	2 225,	-0 075,	2 225,	-0 100,
	Max	2.375	0 150	2 450	0 050	2 450	0.150
Week 18	n	4	4	4	4	8	8
	Mean	2 2938	0 0063	2.3375	-0.0063	2 3156	0 0000
	Median	2 3000	-0.0250	2 3500	-0 0125	2 3250	-0.0125
	SD	0 03750	0 12479	0 06614	0 02394	0.05500	0 08345
	(SE)	(0 01875)	(0 06240)	(0 03307)	(0 01197)	(0 01944)	(0 02950)
	Min,	2 250,	-0 100,	2 250,	-0 025,	2.250,	-0 100,
	Max	2 325	0.175	2 400	0 025	2 400	0 175

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14:03
Reference listing. L 16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 21	n	4	4	4	4	8	8
	Mean	2 2875	0 0000	2 2875	-0 0563	2 2875	-0 0281
	Median	2 2750	0.0000	2.2500	-0 1000	2 2625	-0 0750
	SD	0 06614	0 08660	0 11273	0 10680	0.08557	0 09490
	(SE)	(0 03307)	(0.04330)	(0.05637)	(0 05340)	(0 03025)	(0.03355)
	Min,	2 225,	-0 075,	2.200,	-0 125,	2.200,	-0.125,
	Max	2.375	0 075	2.450	0 100	2 450	0 100
Week 24	n	4	4	4	4	8	8
	Mean	2.3063	0 0188	2 2813	-0.0625	2 2938	-0 0219
	Median	2 3000	0.0500	2 2750	-0 0500	2 2750	-0 0250
	SD	0 07465	0 12479	0 03146	0 04787	0 05469	0 09769
	(SE)	(0.03733)	(0 06240)	(0 01573)	(0 02394)	(0 01934)	(0 03454)
	Min,	2 225,	-0 150,	2 250,	-0 125,	2 225,	-0 150,
	Max	2 400	0 125	2.325	-0 025	2.400	0 125
Week 25	n	4	4	4	4	8	8
	Mean	2.3000	0 0125	2.2875	-0.0563	2 2938	-0 0219
	Median	2 2875	-0 0500	2.2875	-0 0500	2.2875	-0 0500
	SD	0 06124	0 14216	0 08780	0 05543	0.07039	0.10643
	(SE)	(0 03062)	(0 07108)	(0.04390)	(0.02772)	(0 02489)	(0 03763)
	Min,	2 250,	-0 075,	2 200,	-0.125,	2 200,	-0 125,
	Max	2 375	0 225	2.375	0 000	2 375	0 225

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 28	n	4	4	4	4	8	8
	Mean	2 2375	-0.0500	2 2375	-0 1063	2 2375	-0.0781
	Median	2 2375	-0 1000	2.2375	-0.1000	2 2375	-0.1000
	SD	0 03227	0 10000	0 04330	0 03750	0.03536	0 07611
	(SE)	(0 01614)	(0.05000)	(0.02165)	(0 01875)	(0 01250)	(0 02691)
	Min,	2 200,	-0 100,	2.200,	-0 150,	2.200,	-0 150,
	Max	2 275	0 100	2 275	-0 075	2 275	0 100
Week 32	n	4	4	3	3	7	7
	Mean	2 3563	0.0688	2 3167	-0.0083	2 3393	0 0357
	Median	2 3500	0 0625	2.2750	0 0000	2.3250	0 0000
	SD	0 05543	0.09656	0.07217	0 06292	0 06099	0 08763
	(SE)	(0.02772)	(0 04828)	(0.04167)	(0 03632)	(0 02305)	(0 03312)
	Min,	2 300,	-0 025,	2 275,	-0 075,	2.275,	-0.075,
	Max	2 425	0 175	2.400	0 050	2 425	0 175
Week 36	n	4	4	4	4	8	8
	Mean	2 3563	0 0688	2 3375	-0.0063	2.3469	0 0313
	Median	2 3625	0 0500	2 3375	0 0250	2 3625	0 0250
	SD	0 04270	0.05543	0.04330	0 06250	0 04105	0.06781
	(SE)	(0 02135)	(0.02772)	(0 02165)	(0.03125)	(0 01451)	(0 02397)
	Min,	2 300,	0 025,	2 300,	-0 100,	2 300,	-0.100,
	Max	2 400	0 150	2 375	0.025	2 400	0 150

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 40	n	4	4	4	4	8	8
	Mean	2 3125	0 0250	2 3438	0 0000	2 3281	0 0125
	Median	2 3125	0 0000	2 3375	0 0000	2 3125	0 0000
	SD	0 07217	0 10801	0 08750	0 04564	0 07611	0.07792
	(SE)	(0 03608)	(0 05401)	(0 04375)	(0 02282)	(0 02691)	(0 02755)
	Min,	2 225.	-0 075,	2 250,	-0 050,	2 225,	-0.075.
	Max	2 400	0 175	2 450	0 050	2 450	0 175
Week 44	n	4	4	4	4	8	8
	Mean	2 3250	0 0375	2 3438	0 0000	2.3344	0 0188
	Median	2 3125	0 0250	2.3375	0.0000	2 3125	0 0000
	SD	0 03536	0.09242	0 08004	0.06124	0.05815	0 07530
	(SE)	(0 01768)	(0 04621)	(0 04002)	(0 03062)	(0 02056)	(0 02662)
	Min,	2 300,	-0 050,	2 275,	-0 075,	2.275,	-0 075,
	Max	2 375	0 150	2.425	0 075	2.425	0 150
Week 48	n	4	4	4	4	8	8
	Mean	2 3375	0 0500	2 3375	-0.0063	2 3375	0 0219
	Median	2 3375	0 0625	2 3375	0 0000	2.3375	0 0375
	SD	0 05204	0.07906	0 07217	0 08260	0.05825	0 08066
	(SE)	(0 02602)	(0.03953)	(0.03608)	(0 04130)	(0 02059)	(0 02852)
	Min,	2 275,	-0 050,	2 250,	-0 100,	2.250,	-0.100.
	Max	2.400	0 125	2.425	0.075	2 425	0 125

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source. P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 52	n	4	4	4	4	8	8
	Mean	2 3688	0 0813	2 3813	0 0375	2 3750	0 0594
	Median	2 3750	0 0750	2 3875	0.0125	2 3875	0 0250
	SD	0 05543	0 13901	0 08260	0 05951	0.06547	0 10172
	(SE)	(0 02772)	(0 06950)	(0.04130)	(0 02976)	(0 02315)	(0 03596)
	Min,	2.300,	-0 075,	2.275,	0 000,	2 275,	-0.075,
	Max	2 425	0 250	2 475	0 125	2 475	0 250
Week 56	n	4	4	3	3	7	7
	Mean	2 4063	0 1188	2.2500	-0 0917	2 3393	0 0286
	Median	2.3875	0 1250	2 2750	-0 1000	2 3250	0 0000
	SD	0 08985	0.11063	0 06614	0 03819	0 11167	0 13877
	(SE)	(0 04492)	(0.05532)	(0 03819)	(0.02205)	(0 04221)	(0 05245)
	Min,	2 325,	0.000,	2 175,	-0.125,	2 175,	-0 125,
	Max	2 525	0 225	2 300	-0 050	2 525	0.225
Week 60	n	4	4	4	4	8	8
	Mean	2 3813	0.0938	2 2938	-0 0500	2.3375	0.0219
	Median	2 3750	0.0750	2 2625	-0.0500	2 3375	-0 0125
	SD	0 05543	0 12970	0 11250	0 12247	0 09449	0 13979
	(SE)	(0 02772)	(0 06485)	(0.05625)	(0 06124)	(0 03341)	(0 04942)
	Min,	2 325,	-0 025,	2.200,	-0.200,	2 200,	-0 200,
	Max	2.450	0.250	2 450	0 100	2 450	0.250

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015 14 03
Reference listing, L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 64	n	4	4	4	4	8	8
	Mean	2 3812	0 0938	2 3750	0 0313	2 3781	0 0625
	Median	2 3750	0 1375	2 3625	0 0125	2.3750	0 0625
	SD	0 10078	0.13598	0 10607	0.08509	0 09584	0 11019
	(SE)	(0 05039)	(0 06799)	(0.05303)	(0 04254)	(0 03388)	(0.03896)
	Min,	2 275,	-0 100,	2 275,	-0 050,	2 275,	-0 100,
	Max	2 500	0.200	2.500	0.150	2 500	0 200
Week 68	n	4	4	4	4	8	8
	Mean	2 3750	0 0875	2 3813	0 0375	2.3781	0 0625
	Median	2 3750	0 0625	2 4000	0 0250	2 3875	0.0500
	SD	0 02887	0 11990	0 09656	0 07500	0 06606	0 09636
	(SE)	(0 01443)	(0 05995)	(0 04828)	(0 03750)	(0 02336)	(0 03407)
	Min,	2 350,	-0.025,	2 250,	-0 025,	2 250,	-0 025,
	Max	2.400	0 250	2 475	0.125	2 475	0 250
Week 72	n	3	3	4	4	7	7
	Mean	2 3417	0 0583	2 3438	0 0000	2 3429	0 0250
	Median	2 3750	0 0000	2 3000	-0.0125	2.3250	0 0000
	SD	0 08036	0 17017	0 10680	0 12416	0 08864	0 13540
	(SE)	(0 04640)	(0 09825)	(0 05340)	(0 06208)	(0 03350)	(0.05118)
	Min,	2 250,	-0 075,	2 275,	-0.125,	2 250,	-0 125,
	Max	2 400	0 250	2 500	0.150	2 500	0 250

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14:03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 73	n	2	2	0	0	2	2
	Mean	2 3375	0 0250			2.3375	0 0250
	Median	2 3375	0 0250			2 3375	0 0250
	SD	0 08839	0 10607			0 08839	0 10607
	(SE)	(0 06250)	(0 07500)			(0 06250)	(0.07500)
	Min,	2.275,	-0 050,			2 275,	-0 050,
	Max	2 400	0.100			2 400	0 100
Week 76	n	4	4	4	4	8	8
	Mean	2 3750	0 0875	2 3438	0 0000	2 3594	0 0438
	Median	2 3625	0 0750	2 3250	0 0125	2 3375	0 0250
	SD	0 05401	0 11990	0 05543	0 07906	0.05335	0 10501
	(SE)	(0 02700)	(0.05995)	(0.02772)	(0.03953)	(0 01886)	(0 03713)
	Min,	2 325,	-0.025,	2 300,	-0 100,	2 300,	-0 100,
	Max	2 450	0 225	2.425	0 075	2.450	0 225
Week 80	n	4	4	4	4	8	8
	Mean	2 3500	0.0625	2 3188	-0 0250	2 3344	0 0188
	Median	2 3375	0 0000	2 3250	0 0000	2 3375	0 0000
	SD	0 05401	0 14216	0.09437	0 09574	0 07312	0.12156
	(SE)	(0 02700)	(0 07108)	(0 04719)	(0 04787)	(0 02585)	(0 04298)
	Min,	2 300,	-0 025,	2 225,	-0 150,	2 225,	-0 150,
	Max	2 425	0 275	2 400	0 050	2 425	0 275

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 84	n	4	4	4	4	8	8
	Mean	2 3813	0 0938	2 3438	0 0000	2 3625	0 0469
	Median	2 3750	0.0875	2 3500	0 0375	2 3500	0 0500
	SD	0 05543	0 09656	0 07181	0 10607	0 06268	0 10643
	(SE)	(0.02772)	(0.04828)	(0 03590)	(0 05303)	(0.02216)	(0.03763)
	Min,	2 325,	0 000,	2 250,	-0.150,	2 250,	-0 150,
	Max	2 450	0 200	2 425	0 075	2 450	0 200
Week 88	n	4	4	4	4	8	8
	Mean	2 3438	0 0563	2 3000	-0 0438	2 3219	0 0063
	Median	2 3250	0 0250	2 3125	-0 0375	2.3250	0.0125
	SD	0 05907	0.10078	0 10607	0 09656	0.08285	0 10586
	(SE)	(0 02954)	(0 05039)	(0 05303)	(0.04828)	(0 02929)	(0 03743)
	Min,	2 300,	-0 025,	2 175,	-0 150,	2 175,	-0.150,
	Max	2 425	0 200	2 400	0.050	2 425	0 200
Week 92	n	4	4	4	4	8	8
	Mean	2 3187	0.0313	2 3313	-0.0125	2 3250	0 0094
	Median	2 3250	0 0500	2 3250	-0 0500	2 3250	-0 0250
	SD	0 09437	0 12970	0 15326	0 13150	0 11802	0 12316
	(SE)	(0 04719)	(0 06485)	(0.07663)	(0.06575)	(0 04173)	(0 04354)
	Min,	2 200,	-0 125,	2 150,	-0 125,	2 150,	-0 125,
	Max	2 425	0 150	2 525	0 175	2 525	0 175

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 96	n	4	4	4	4	8	8
	Mean	2 4063	0 1188	2 3250	-0.0188	2 3656	0 0500
	Median	2 4125	0 0750	2.3125	0 0000	2.3625	0 0375
	SD	0 06250	0.14773	0 05401	0 08509	0.06936	0 13363
	(SE)	(0 03125)	(0.07386)	(0 02700)	(0 04254)	(0.02452)	(0 04725)
	Min,	2 325,	0 000,	2 275,	-0.125,	2 275,	-0 125,
	Max	2.475	0 325	2.400	0 050	2 475	0.325
Week 100	n	4	4	4	4	8	8
	Mean	2 4063	0 1188	2 3375	-0 0063	2.3719	0 0563
	Median	2 4125	0.0875	2 3250	-0.0375	2 3625	0 0000
	SD	0 06884	0 14631	0 08539	0 07181	0.08066	0 12589
	(SE)	(0.03442)	(0.07315)	(0.04270)	(0.03590)	(0 02852)	(0 04451)
	Min,	2 325,	0 000,	2 250,	-0.050,	2 250,	-0.050,
	Max	2 475	0 300	2 450	0 100	2.475	0.300
Week 104	n	4	4	4	4	8	8
	Mean	2 4000	0 1125	2 3000	-0 0438	2 3500	0 0344
	Median	2 4125	0 1000	2 2750	-0 0500	2 3625	0 0250
	SD	0 05401	0 10508	0.06770	0 07465	0 07792	0 11873
	(SE)	(0 02700)	(0 05254)	(0 03385)	(0 03733)	(0 02755)	(0 04198)
	Min,	2 325,	0 000,	2 250,	-0 125,	2 250,	-0 125,
	Max	2 450	0 250	2 400	0 050	2 450	0.250

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 108	n	4	4	4	4	8	8
	Mean	2 4250	0 1375	2.3438	0 0000	2 3844	0 0688
	Median	2 4000	0.1375	2 3250	-0 0375	2.3875	0 0375
	SD	0 06770	0.11637	0 12141	0 10206	0.10083	0 12518
	(SE)	(0.03385)	(0 05818)	(0.06070)	(0 05103)	(0 03565)	(0 04426)
	Min,	2.375,	0 025,	2 225,	-0 075,	2.225,	-0 075,
	Max	2 525	0.250	2.500	0 150	2 525	0 250
Week 112	n	4	4	4	4	8	8
	Mean	2 3875	0 1000	2 3313	-0 0125	2 3594	0 0438
	Median	2 4000	0 0625	2.3500	0 0000	2 4000	0 0500
	SD	0 04330	0.12416	0 11250	0 10308	0 08445	0 12156
	(SE)	(0 02165)	(0 06208)	(0 05625)	(0 05154)	(0 02986)	(0 04298)
	Min,	2 325,	0.000,	2.200,	-0.125,	2 200,	-0 125,
	Max	2 425	0 275	2.425	0 075	2 425	0.275
Week 116	n	4	4	4	4	8	8
	Mean	2 4250	0 1375	2 3563	0 0125	2 3906	0.0750
	Median	2 4250	0 1125	2 3375	0 0000	2 4000	0 0625
	SD	0.04082	0.10104	0 08985	0 08539	0 07433	0 10938
	(SE)	(0 02041)	(0 05052)	(0 04492)	(0 04270)	(0 02628)	(0 03867)
	Min,	2 375,	0 050,	2 275,	-0 075,	2 275,	-0 075,
	Max	2.475	0 275	2 475	0.125	2 475	0 275

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 120	n	3	3	4	4	7	7
	Mean	2 3417	0 0583	2 3750	0 0313	2 3607	0 0429
	Median	2 3500	0.0000	2.3500	0 0250	2 3500	0 0000
	SD	0 03819	0 12332	0 06770	0 08260	0 05563	0 09322
	(SE)	(0.02205)	(0 07120)	(0 03385)	(0.04130)	(0 02103)	(0.03523)
	Min.	2 300,	-0 025,	2 325,	-0 050,	2.300,	-0 050,
	Max	2.375	0 200	2 475	0.125	2.475	0 200
Week 124	n	3	3	4	4	7	7
	Mean	2 4250	0 1417	2 3875	0 0438	2 4036	0 0857
	Median	2 4250	0 1000	2 3375	0 0250	2 4250	0 0500
	SD	0 00000	0 11815	0 10897	0 11614	0 07962	0 11890
	(SE)	(0 00000)	(0 06821)	(0 05449)	(0 05807)	(0 03009)	(0 04494)
	Min,	2 425,	0 050,	2.325,	-0 075,	2 325,	-0 075,
	Max	2 425	0 275	2 550	0.200	2 550	0 275
Week 128	n	4	4	4	4	8	8
	Mean	2.4250	0 1375	2 3375	-0.0063	2 3812	0 0656
	Median	2.4250	0 1750	2.3625	0.0250	2 4000	0 0875
	SD	0.07360	0 11273	0 16394	0 15729	0 12660	0 14817
	(SE)	(0 03680)	(0 05637)	(0 08197)	(0 07864)	(0 04476)	(0 05238)
	Min,	2 350,	-0 025,	2.125,	-0 225,	2 125,	-0 225,
	Max	2.500	0 225	2 500	0 150	2 500	0 225

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14 03
Reference listing: L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 132	n	4	4	4	4	8	8
	Mean	2 3375	0 0500	2 3250	-0 0188	2.3313	0 0156
	Median	2.3375	0 0250	2.3250	-0 0125	2 3250	0 0125
	SD	0 03227	0 12583	0 02041	0 06250	0 02588	0 09905
	(SE)	(0 01614)	(0 06292)	(0.01021)	(0 03125)	(0 00915)	(0 03502)
	Min,	2 300,	-0 075,	2 300,	-0 100,	2 300,	-0.100,
	Max	2 375	0 225	2 350	0 050	2 375	0 225
Week 136	n	4	4	4	4	8	8
	Mean	2 3500	0 0625	2 3063	-0.0375	2 3281	0 0125
	Median	2 3375	0 0375	2 3125	-0 0625	2.3375	-0.0125
	SD	0 07360	0.10104	0 12645	0 09682	0 09860	0 10607
	(SE)	(0 03680)	(0.05052)	(0 06322)	(0 04841)	(0 03486)	(0.03750)
	Min,	2 275,	-0 025,	2.150,	-0 125,	2 150,	-0 125,
	Max	2 450	0 200	2.450	0 100	2 450	0 200
Week 140	n	4	4	4	4	8	8
	Mean	2 3000	0.0125	2 2813	-0 0625	2.2906	-0.0250
	Median	2 3000	0 0625	2 3000	-0 0750	2 3000	-0 0125
	SD	0.08660	0.10897	0.11614	0 08292	0 09537	0 09820
	(SE)	(0 04330)	(0 05449)	(0 05807)	(0 04146)	(0 03372)	(0 03472)
	Min,	2 225,	-0 150,	2 125,	-0.150,	2 125,	-0 150,
	Max	2 375	0.075	2 400	0 050	2 400	0.075

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing: L 16 2 8.1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 144	n	4	4	4	4	8	8
	Mean	2 2813	-0 0063	2.2938	-0 0500	2 2875	-0 0281
	Median	2 3250	0.0000	2.2750	-0 0750	2 3250	-0.0375
	SD	0 14343	0.17485	0 12970	0 10801	0 12677	0 13656
	(SE)	(0 07172)	(0.08743)	(0.06485)	(0 05401)	(0 04482)	(0 04828)
	Min,	2 075,	-0 225,	2 175,	-0 150,	2.075,	-0 225,
	Max	2.400	0 200	2 450	0 100	2 450	0 200
Week 148	n	4	4	4	4	8	8
	Mean	2 3750	0.0875	2 3188	-0 0250	2 3469	0 0313
	Median	2.3750	0 0500	2.3000	-0.0250	2 3375	0 0125
	SD	0 04564	0 13617	0 07739	0 07360	0 06606	0 11783
	(SE)	(0 02282)	(0 06808)	(0 03870)	(0 03680)	(0 02336)	(0 04166)
	Min,	2 325,	-0 025,	2 250,	-0 100,	2 250,	-0 100,
	Max	2.425	0.275	2.425	0 050	2 425	0 275
Week 152	n	4	4	3	3	7	7
	Mean	2 3188	0.0313	2 2250	-0.1000	2 2786	-0 0250
	Median	2 3250	0 0250	2 1750	-0.1000	2 3250	-0 0250
	SD	0 09214	0.07465	0 08660	0 07500	0 09621	0 09789
	(SE)	(0 04607)	(0 03733)	(0 05000)	(0 04330)	(0 03636)	(0 03700)
	Min,	2 200,	-0 050,	2 175,	-0.175,	2 175,	-0 175,
	Max	2 425	0 125	2 325	-0 025	2 425	0 125

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 156	n	4	4	4	4	8	8
	Mean	2 3563	0 0688	2 3500	0 0063	2 3531	0 0375
	Median	2 3500	0 0500	2 3625	-0 0125	2 3500	0 0000
	SD	0 03750	0 12141	0 11726	0 08509	0 08066	0.10264
	(SE)	(0 01875)	(0.06070)	(0.05863)	(0.04254)	(0 02852)	(0 03629)
	Min.	2 325,	-0 050,	2.200,	-0 075,	2.200,	-0.075,
	Max	2.400	0.225	2 475	0 125	2 475	0 225
Week 160	n	4	4	4	4	8	8
	Mean	2 4000	0.1125	2 3438	0 0000	2 3719	0 0563
	Median	2 4000	0 0750	2 3500	0 0125	2 3875	0 0375
	SD	0 02041	0 11273	0 05543	0.05401	0.04899	0 10155
	(SE)	(0 01021)	(0.05637)	(0 02772)	(0 02700)	(0 01732)	(0 03590)
	Min.	2 375,	0.025,	2.275,	-0 075,	2 275,	-0 075,
	Max	2 425	0 275	2.400	0.050	2 425	0.275
Week 164	n	4	4	4	4	8	8
	Mean	2 3688	0.0813	2 3125	-0 0313	2 3406	0 0250
	Median	2.3625	0 0250	2 3250	-0 0500	2.3250	0.0125
	SD	0 05154	0.12970	0 10308	0 07739	0 08122	0 11573
	(SE)	(0.02577)	(0 06485)	(0 05154)	(0 03870)	(0 02871)	(0 04092)
	Min.	2 325,	0 000,	2 175,	-0 100,	2.175,	-0 100,
	Max	2 425	0 275	2 425	0 075	2 425	0 275

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 168	n	4	4	4	4	8	8
	Mean	2.3813	0 0938	2 2813	-0.0625	2.3313	0 0156
	Median	2 3875	0 0625	2 2625	-0 0750	2 3625	0 0250
	SD	0 02394	0 09437	0.06575	0 06614	0 07039	0 11255
	(SE)	(0 01197)	(0.04719)	(0 03287)	(0 03307)	(0 02489)	(0.03979)
	Min,	2 350,	0 025,	2 225,	-0.125,	2 225,	-0 125,
	Max	2 400	0 225	2 375	0 025	2.400	0.225

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing: L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Glucose (mmol/L)							
Baseline [1]	n	4		4		8	
	Mean	5 05050		4 70363		4 87706	
	Median	4.93950		4.46775		4 88400	
	SD	0 604585		1 171541		0 882750	
	(SE)	(0 302292)		(0 585770)		(0 312099)	
	Min,	4 4400,		3 6075,		3 6075,	
	Max	5 8830		6 2715		6 2715	
Week 2	n	4	4	4	4	8	8
	Mean	5.31413	0 26363	4 32900	-0 37463	4 82156	-0 05550
	Median	4 71750	0 05550	4.27350	-0 08325	4 35675	-0 02775
	SD	1 608144	1.808298	0 282996	1 097325	1.191612	1 426129
	(SE)	(0 804072)	(0.904149)	(0 141498)	(0 548662)	(0 421299)	(0 504213)
	Min,	4 1625,	-1.7205,	4 0515,	-1 9425,	4 0515,	-1 9425,
	Max	7.6590	2 6640	4.7175	0 6105	7.6590	2 6640
Week 4	n	3	3	4	4	7	7
	Mean	5 99400	0.88800	5 23088	0.52725	5 55793	0 68186
	Median	5 21700	0.77700	5 38350	0 69375	5 21700	0 72150
	SD	1.345803	0.727876	0 704034	1.216366	1 008935	0 976503
	(SE)	(0 777000)	(0.420239)	(0 352017)	(0.608183)	(0.381341)	(0 369083)
	Min,	5 2170,	0 2220,	4.2735,	-1 1100,	4 2735,	-1 1100,
	Max	7 5480	1.6650	5 8830	1 8315	7.5480	1 8315

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	5 88300	0 83250	4 23188	-0 47175	5 05744	0 18038
	Median	5 68875	0 86025	4 30125	-0 19425	4 91175	0 13875
	SD	1 014300	0 522605	0 708395	0 690969	1 197874	0 898713
	(SE)	(0 507150)	(0 261302)	(0 354198)	(0 345485)	(0.423512)	(0 317743)
	Min,	4 9395,	0 2775,	3 4410,	-1 4985,	3.4410,	-1 4985,
	Max	7 2150	1 3320	4 8840	0.0000	7.2150	1.3320
Week 8	n	4	4	4	4	8	8
	Mean	5 50838	0.45788	4 74525	0.04162	5 12681	0 24975
	Median	5 49450	0 41625	4 77300	0 38850	4 96725	0 41625
	SD	0 408783	0.886119	0 146839	1 153880	0.497237	0.978079
	(SE)	(0 204392)	(0.443060)	(0.073420)	(0 576940)	(0 175800)	(0 345803)
	Min,	5 0505,	-0 5550,	4 5510,	-1 5540,	4.5510,	-1 5540,
	Max	5 9940	1 5540	4 8840	0.9435	5 9940	1 5540
Week 10	n	4	4	4	4	8	8
	Mean	5 05050	0 00000	4 20413	-0 49950	4 62731	-0 24975
	Median	5 02275	0 13875	3 85725	0 02775	4 71750	0 11100
	SD	0 481711	0 679733	1 160976	1 766145	0 939030	1 267332
	(SE)	(0 240856)	(0 339867)	(0.580488)	(0 883072)	(0 331997)	(0 448070)
	Min,	4 4955,	-0 9435,	3 2190,	-3.0525,	3 2190,	-3 0525,
	Max	5 6610	0 6660	5 8830	0 9990	5 8830	0 9990

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14:03
Reference listing: L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	6 66000	1 60950	4 78688	0.08325	5 72344	0.84638
	Median	6 68775	1 60950	4.71750	0 24975	5 63325	0 61050
	SD	0 875189	1 317273	0.529194	0 666770	1 204468	1 264812
	(SE)	(0 437594)	(0.658636)	(0 264597)	(0 333385)	(0 425844)	(0 447179)
	Min,	5 7720,	0 1665,	4 2180,	-0 7770,	4 2180,	-0 7770,
	Max	7.4925	3.0525	5 4945	0 6105	7 4925	3 0525
Week 15	n	4	4	4	4	8	8
	Mean	4 20413	-0.84638	3.95438	-0 74925	4 07925	-0 79781
	Median	4 32900	-0 88800	3 94050	-1.05450	4 19025	-0 88800
	SD	0 546377	0 390806	0.649413	1 039792	0 571407	0 729046
	(SE)	(0 273188)	(0 195403)	(0 324707)	(0 519896)	(0.202023)	(0.257757)
	Min,	3 4965,	-1 2765,	3 2745,	-1 6095,	3 2745,	-1 6095,
	Max	4 6620	-0 3330	4 6620	0 7215	4 6620	0 7215
Week 18	n	4	4	4	4	8	8
	Mean	4 91175	-0 13875	5 20313	0 49950	5 05744	0 18038
	Median	4 93950	-0 05550	5 16150	0.49950	4 93950	0 41625
	SD	0 273775	0 675947	0 947708	1 495413	0 664305	1 127210
	(SE)	(0 136887)	(0 337973)	(0 473854)	(0 747707)	(0 234867)	(0 398529)
	Min,	4 5510,	-0 9435,	4 1070,	-1 3320,	4 1070,	-1 3320,
	Max	5 2170	0 4995	6.3825	2 3310	6.3825	2 3310

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14 03
Reference listing. L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 21	n	4	4	4	4	8	8
	Mean	3 92663	-1 12388	4 03763	-0.66600	3.98213	-0 89494
	Median	3 63525	-1.22100	4 13475	-0 33300	4 02375	-0 77700
	SD	1.146739	1 360505	0 315179	1.430853	0.780813	1 315525
	(SE)	(0 573369)	(0.680252)	(0 157590)	(0 715426)	(0.276059)	(0 465108)
	Min,	2.9415,	-2.6640,	3 6075,	-2 6640,	2 9415,	-2 6640,
	Max	5.4945	0.6105	4 2735	0 6660	5 4945	0 6660
Week 24	n	4	4	4	4	8	8
	Mean	5.38350	0 33300	4 45388	-0.24975	4.91869	0 04163
	Median	5 38350	0 22200	4 38450	-0.24975	4.88400	0 00000
	SD	0 801714	0.648820	1 040656	0 346597	0 993230	0 573521
	(SE)	(0 400857)	(0.324410)	(0 520328)	(0 173299)	(0 351160)	(0.202770)
	Min,	4.6620,	-0.3330,	3 4410,	-0 6660,	3.4410,	-0 6660,
	Max	6 1050	1.2210	5 6055	0.1665	6 1050	1.2210
Week 25	n	4	4	4	4	8	8
	Mean	3.84338	-1 20713	3.48263	-1 22100	3 66300	-1.21406
	Median	3 80175	-1 47075	3.66300	-0.94350	3 66300	-1 16550
	SD	0 742367	0 706945	0 577440	0 834348	0 645193	0 715952
	(SE)	(0 371183)	(0 353472)	(0 288720)	(0 417174)	(0 228110)	(0 253127)
	Min,	3 0525,	-1 7205,	2 6640,	-2 4420,	2 6640,	-2 4420,
	Max	4 7175	-0 1665	3 9405	-0 5550	4.7175	-0 1665

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 28	n	4	4	4	4	8	8
	Mean	4 96725	-0.08325	4.20413	-0 49950	4 58569	-0 29138
	Median	4 91175	0 02775	4 19025	-0.77700	4 55100	-0.36075
	SD	0 705673	0 537138	0 816152	0.752839	0 815646	0 645022
	(SE)	(0 352836)	(0 268569)	(0 408076)	(0 376419)	(0 288375)	(0 228050)
	Min,	4 1625,	-0 8325,	3 2190,	-1 0545,	3 2190,	-1 0545,
	Max	5 8830	0 4440	5 2170	0 6105	5 8830	0 6105
Week 32	n	4	4	3	3	7	7
	Mean	5 73038	0 67988	4 36600	0.18500	5 14564	0 46779
	Median	5 63325	0 69375	4 38450	0 05550	4 93950	0 55500
	SD	0 781447	1 364273	0 582970	0 539046	0 974924	1 047592
	(SE)	(0 390724)	(0 682137)	(0.336578)	(0 311218)	(0 368487)	(0 395953)
	Min,	4 8840,	-0 9990,	3 7740,	-0.2775,	3.7740,	-0 9990,
	Max	6 7710	2 3310	4 9395	0 7770	6 7710	2 3310
Week 36	n	4	4	4	4	8	8
	Mean	5 53613	0 48563	4 41225	-0 29138	4.97419	0 09713
	Median	5 49450	0 80475	4 10700	0.27750	5 07825	0 52725
	SD	0 536899	0 868565	0.686497	1 341505	0 828493	1 125648
	(SE)	(0 268449)	(0.434282)	(0 343249)	(0 670753)	(0 292917)	(0 397977)
	Min,	5 0505,	-0 7770,	3 9960,	-2 2755,	3 9960,	-2 2755,
	Max	6 1050	1 1100	5.4390	0 5550	6 1050	1.1100

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1.1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 40	n	4	4	4	4	8	8
	Mean	5 42513	0 37463	4 92563	0 22200	5.17538	0 29831
	Median	5.68875	0 24975	5 16150	0 22200	5 60550	0 24975
	SD	0 935714	1 064072	1 050476	1 676676	0 958882	1 302583
	(SE)	(0 467857)	(0.532036)	(0 525238)	(0 838338)	(0.339016)	(0 460533)
	Min,	4 1070,	-0 7770,	3 6075,	-1 7205,	3.6075,	-1 7205,
	Max	6 2160	1 7760	5.7720	2 1645	6 2160	2 1645
Week 44	n	4	4	4	4	8	8
	Mean	5 56388	0 51338	4 45388	-0.24975	5 00888	0 13181
	Median	5 46675	0 52725	4.41225	0 11100	4 99500	0 36075
	SD	0 467377	1 042627	0 340244	1 338536	0 703747	1 183275
	(SE)	(0.233688)	(0.521314)	(0 170122)	(0 669268)	(0 248812)	(0 418351)
	Min,	5 1060,	-0 7770,	4.1070,	-2.1645,	4 1070,	-2 1645,
	Max	6 2160	1 7760	4 8840	0.9435	6 2160	1.7760
Week 48	n	4	4	4	4	8	8
	Mean	5.52225	0.47175	4 67588	-0 02775	5.09906	0 22200
	Median	5 27250	0 33300	4 68975	0.30525	4 91175	0 30525
	SD	0 925925	1 476411	0.294987	0 976653	0 780637	1 189233
	(SE)	(0 462962)	(0 738206)	(0 147493)	(0.488326)	(0 275997)	(0 420457)
	Min,	4 7175,	-1 1655,	4.3290,	-1.4430,	4 3290,	-1.4430,
	Max	6.8265	2 3865	4 9950	0 7215	6 8265	2 3865

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run. 06MAR2015 14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 52	n	4	4	4	4	8	8
	Mean	4 81463	-0 23588	4 62038	-0.08325	4 71750	-0 15956
	Median	4 82850	-0 11100	4 52325	0 27750	4 57875	-0.08325
	SD	0 695228	1.267520	0.559376	1 575327	0 593320	1 326185
	(SE)	(0.347614)	(0.633760)	(0 279688)	(0 787663)	(0 209770)	(0.468877)
	Min,	3 9960,	-1.8870,	4.0515,	-2 2200,	3.9960,	-2 2200,
	Max	5 6055	1 1655	5 3835	1 3320	5 6055	1 3320
Week 56	n	4	4	3	3	7	7
	Mean	6 02175	0 97125	5.03200	0 11100	5 59757	0 60257
	Median	5 71650	0 52725	5.10600	-0 05550	5 38350	0.49950
	SD	0 858009	1 264377	0 178408	1.312197	0.811534	1 258855
	(SE)	(0 429005)	(0 632189)	(0 103004)	(0 757597)	(0.306731)	(0 475802)
	Min,	5 3835,	0.0000,	4 8285,	-1 1100,	4.8285,	-1 1100,
	Max	7 2705	2 8305	5 1615	1 4985	7 2705	2 8305
Week 60	n	4	4	4	4	8	8
	Mean	5 78588	0 73538	4.71750	0 01388	5 25169	0 37463
	Median	5 71650	0 77700	4 52325	0 22200	5 16150	0 61050
	SD	0 774850	1 320678	0 485955	1 389626	0.827430	1 312951
	(SE)	(0 387425)	(0 660339)	(0 242978)	(0 694813)	(0 292541)	(0 464198)
	Min,	4 9950,	-0 8880,	4 3845,	-1 7760,	4 3845,	-1 7760,
	Max	6 7155	2 2755	5 4390	1 3875	6 7155	2.2755

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14:03
Reference listing L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 64	n	4	4	4	4	8	8
	Mean	6 54900	1 49850	4.75913	0 05550	5 65406	0 77700
	Median	6 21600	1 05450	4 68975	0.36075	5 30025	0 83250
	SD	1 185156	1.283319	0 199466	1 236874	1 238689	1 398713
	(SE)	(0 592578)	(0 641659)	(0 099733)	(0.618437)	(0 437943)	(0 494520)
	Min,	5 5500,	0 5550,	4 6065,	-1 5540,	4.6065,	-1.5540,
	Max	8 2140	3.3300	5 0505	1 0545	8.2140	3 3300
Week 68	n	4	4	4	4	8	8
	Mean	6.97913	1 92863	4 60650	-0 09712	5 79281	0 91575
	Median	6 32700	1 22100	4 52325	0 27750	5 57775	0 97125
	SD	1 616423	2 058432	0 485955	1 546964	1 682075	2 003498
	(SE)	(0 808211)	(1 029216)	(0 242978)	(0 773482)	(0 594703)	(0 708344)
	Min,	5 8830,	0 3330,	4 1070,	-2 1645,	4 1070,	-2 1645,
	Max	9 3795	4.9395	5.2725	1 2210	9 3795	4 9395
Week 72	n	3	3	4	4	7	7
	Mean	7.49250	2 42350	4 57875	-0.12487	5 82750	0 96729
	Median	6 21600	1 11000	4 66200	0 36075	5 27250	1 11000
	SD	2 405783	2 973440	0 680488	1 823001	2 141610	2 542493
	(SE)	(1 388979)	(1 716716)	(0 340244)	(0 911501)	(0 809452)	(0.960972)
	Min,	5 9940,	0 3330,	3 7185,	-2.5530,	3 7185,	-2.5530,
	Max	10 2675	5 8275	5 2725	1 3320	10 2675	5 8275

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 73	n	2	2	0	0	2	2
	Mean	5 32800	0 61050			5 32800	0 61050
	Median	5 32800	0 61050			5 32800	0 61050
	SD	0 784889	0 392444			0 784889	0 392444
	(SE)	(0 555000)	(0 277500)			(0.555000)	(0 277500)
	Min,	4 7730,	0 3330,			4 7730,	0 3330,
	Max	5 8830	0 8880			5 8830	0.8880
Week 76	n	4	4	4	4	8	8
	Mean	7 29825	2 24775	4.52325	-0.18038	5 91075	1 03369
	Median	6 71550	1 47075	4 55100	0 24975	5 35575	0 74925
	SD	1 728241	2 167818	0 246126	1.312099	1.872486	2 106273
	(SE)	(0 864120)	(1 083909)	(0 123063)	(0.656049)	(0.662024)	(0 744680)
	Min,	5 9385,	0 6660,	4 2180,	-2 0535,	4 2180,	-2 0535,
	Max	9.8235	5 3835	4 7730	0.8325	9 8235	5 3835
Week 80	n	4	4	4	4	8	8
	Mean	6 31313	1 26263	4.56488	-0 13875	5 43900	0.56194
	Median	6 29925	1 49850	4 32900	0 49950	6 07725	0 97125
	SD	0 183374	0 589756	1 056324	1 763527	1 168705	1 429346
	(SE)	(0 091687)	(0 294878)	(0 528162)	(0 881763)	(0 413200)	(0 505350)
	Min,	6 1050,	0 3885,	3.5520,	-2 7195,	3 5520,	-2 7195,
	Max	6 5490	1.6650	6 0495	1 1655	6 5490	1 6650

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14.03
Reference listing L 16.2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 84	n	4	4	4	4	8	8
	Mean	5 92463	0 87413	5 32800	0 62438	5 62631	0 74925
	Median	5 35575	0 19425	5.38350	0 69375	5.35575	0 19425
	SD	1.428968	1 854827	0 470933	1.159206	1 035313	1 438113
	(SE)	(0 714484)	(0 927413)	(0.235467)	(0 579603)	(0.366038)	(0.508450)
	Min,	4 9395,	-0 4995,	4 7730,	-0 6105,	4.7730,	-0 6105,
	Max	8 0475	3 6075	5 7720	1.7205	8.0475	3 6075
Week 88	n	4	4	4	4	8	8
	Mean	5 64713	0 59663	4.55100	-0 15262	5.09906	0 22200
	Median	5 74425	0 47175	4 68975	0 41625	5.05050	0 47175
	SD	0.523341	0.895341	0 636034	1 768106	0 796264	1 357847
	(SE)	(0 261670)	(0.447670)	(0.318017)	(0.884053)	(0 281522)	(0 480072)
	Min,	4.9395,	-0 2775,	3.6630,	-2 6085,	3 6630,	-2 6085,
	Max	6 1605	1.7205	5 1615	1 1655	6 1605	1 7205
Week 92	n	4	4	4	4	8	8
	Mean	7.06238	2 01188	4 98113	0 27750	6.02175	1 14469
	Median	6.35475	1.41525	4 93950	0 44400	5 30025	0 83250
	SD	2 346530	2 838062	0 349180	1 131075	1 910407	2 204473
	(SE)	(1 173265)	(1.419031)	(0 174590)	(0 565537)	(0 675431)	(0 779399)
	Min,	5 2170,	-0 6660,	4.6620,	-1.1100,	4 6620,	-1 1100,
	Max	10 3230	5 8830	5 3835	1.3320	10 3230	5 8830

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015.14:03
Reference listing L.16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 96	n	4	4	4	4	8	8
	Mean	5 50838	0 45788	5 42513	0.72150	5 46675	0.58969
	Median	5 52225	0 47175	5 41125	0 72150	5 41125	0.47175
	SD	0 478235	0 984897	0.868565	1 722587	0.650626	1.306631
	(SE)	(0.239117)	(0.492448)	(0 434282)	(0.861294)	(0 230031)	(0 461964)
	Min,	4.9950,	-0 6660,	4 3845,	-0 9990,	4 3845,	-0 9990,
	Max	5 9940	1 5540	6 4935	2 4420	6 4935	2 4420
Week 100	n	4	4	4	4	8	8
	Mean	5 39738	0 34688	4 63425	-0 06938	5 01581	0 13875
	Median	5 43900	0 33300	4 66200	-0.02775	4 99500	0 33300
	SD	0 171811	0 568480	0 228832	1 154769	0 448867	0 871494
	(SE)	(0 085906)	(0 284240)	(0 114416)	(0 577385)	(0 158698)	(0.308120)
	Min,	5 1615,	-0.3330,	4 3845,	-1 4430,	4 3845,	-1.4430,
	Max	5 5500	1 0545	4 8285	1.2210	5 5500	1 2210
Week 104	n	4	4	4	4	8	8
	Mean	5.66100	0.61050	5.98012	1 27650	5 82056	0.94350
	Median	5.77200	0 83250	5 52225	1 05450	5 71650	0 99900
	SD	0 792699	0 707852	1.904534	2 912913	1.361226	1 994473
	(SE)	(0 396349)	(0.353926)	(0.952267)	(1.456456)	(0 481266)	(0 705153)
	Min,	4 6065,	-0 3885,	4.2180,	-2 0535,	4 2180,	-2 0535,
	Max	6 4935	1 1655	8 6580	5 0505	8 6580	5 0505

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015 14.03
Reference listing. L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 108	n	4	4	4	4	8	8
	Mean	6.86813	1 81763	4 80075	0 09713	5 83444	0.95738
	Median	6 79875	1 85925	4 68975	0 58275	5.85525	0.94350
	SD	0.643058	1 229693	0 548487	1 430404	1.235843	1.539705
	(SE)	(0 321529)	(0.614847)	(0.274243)	(0.715202)	(0.436937)	(0 544368)
	Min,	6 1605,	0.2775,	4 2735,	-1 9980,	4.2735,	-1 9980,
	Max	7 7145	3 2745	5 5500	1 2210	7 7145	3 2745
Week 112	n	4	4	4	4	8	8
	Mean	7.64513	2.59463	5 24475	0 54113	6 44494	1 56788
	Median	6 41025	1 47075	5.30025	0.55500	5 57775	1 11000
	SD	3 209537	3 681837	0 548487	1 074633	2.487956	2 740334
	(SE)	(1.604768)	(1 840918)	(0.274243)	(0.537317)	(0 879625)	(0 968854)
	Min,	5 4390,	-0 4440,	4 5510,	-0.4440,	4 5510,	-0 4440,
	Max	12.3210	7 8810	5.8275	1 4985	12 3210	7.8810
Week 116	n	4	4	4	4	8	8
	Mean	7 40925	2 35875	5.18925	0 48563	6 29925	1 42219
	Median	6.35475	1 35975	5 24475	1 24875	5.43900	1 24875
	SD	2 932060	3 394889	0 722922	1.662145	2 305756	2 669435
	(SE)	(1 466030)	(1 697444)	(0 361461)	(0 831073)	(0 815208)	(0 943788)
	Min,	5.3280,	-0 4440,	4 2735,	-1.9980,	4 2735,	-1 9980,
	Max	11 5995	7 1595	5 9940	1.4430	11 5995	7 1595

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015.14.03
Reference listing L 16 2 8.1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 120	n	3	3	4	4	7	7
	Mean	5 08750	0 01850	4 80075	0 09712	4 92364	0 06343
	Median	4 82850	-0 05550	4 82850	0 58275	4 82850	0 11100
	SD	0 497440	1 167261	0 474192	1 520183	0 467338	1.269414
	(SE)	(0 287197)	(0 673918)	(0.237096)	(0 760092)	(0 176637)	(0 479793)
	Min,	4 7730,	-1.1100,	4 2180,	-2.0535,	4 2180,	-2 0535,
	Max	5 6610	1 2210	5 3280	1.2765	5 6610	1 2765
Week 124	n	3	3	4	4	7	7
	Mean	7 88100	2 81200	4.81463	0 11100	6 12879	1 26857
	Median	7 99200	2.10900	4 91175	0.66600	5 38350	1 33200
	SD	1 335464	1.669004	0 577440	1 626317	1 856793	2 082157
	(SE)	(0 771031)	(0 963600)	(0.288720)	(0 813159)	(0 701802)	(0 786981)
	Min,	6.4935,	1 6095,	4 0515,	-2 2200,	4 0515,	-2 2200,
	Max	9 1575	4 7175	5 3835	1 3320	9.1575	4 7175
Week 128	n	4	4	4	4	8	8
	Mean	5 49450	0 44400	4 81463	0.11100	5 15456	0.27750
	Median	5 41125	0 33300	4 82850	0.33300	4 91175	0.33300
	SD	0 498471	0 977178	0 027750	1 174714	0.488758	1 016033
	(SE)	(0 249236)	(0.488589)	(0 013875)	(0.587357)	(0 172802)	(0 359222)
	Min,	4 9950,	-0 6105,	4 7730,	-1.4430,	4 7730,	-1 4430,
	Max	6 1605	1 7205	4 8285	1 2210	6 1605	1 7205

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 132	n	4	4	4	4	8	8
	Mean	6 24375	1 19325	4 57875	-0 12488	5 41125	0 53419
	Median	5 43900	0 47175	4 55100	0 08325	4 82850	0 33300
	SD	2 237735	2 709287	0 710024	1 858698	1.776000	2 263369
	(SE)	(1.118868)	(1 354643)	(0 355012)	(0.929349)	(0 627911)	(0 800222)
	Min,	4 6620,	-1.1655,	3 8295,	-2 4420,	3 8295,	-2 4420,
	Max	9 4350	4.9950	5.3835	1 7760	9 4350	4 9950
Week 136	n	4	4	4	4	8	8
	Mean	7 83938	2 78888	5.17538	0 47175	6.50738	1 63031
	Median	7.32600	1 88700	4.85625	0 61050	5 96625	1 24875
	SD	2 672610	2.991985	1 124132	1 980710	2 372863	2 655552
	(SE)	(1 336305)	(1.495993)	(0 562066)	(0 990355)	(0 838934)	(0 938879)
	Min,	5 1615,	0 2775,	4 2180,	-2 0535,	4 2180,	-2 0535,
	Max	11 5440	7.1040	6 7710	2 7195	11 5440	7.1040
Week 140	n	4	4	4	4	8	8
	Mean	6 67388	1 62338	5 43900	0.73538	6 05644	1 17938
	Median	6 18825	1 24875	5.07825	0.58275	5 21700	0 97125
	SD	1 999220	2 496626	1.145507	1.945075	1.646513	2 125574
	(SE)	(0 999610)	(1 248313)	(0.572754)	(0 972538)	(0.582130)	(0 751504)
	Min,	4 9950,	-0.8880,	4 4955,	-1 2765,	4.4955,	-1 2765,
	Max	9 3240	4 8840	7 1040	3 0525	9.3240	4 8840

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing: L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 144	n	4	4	4	4	8	8
	Mean	7 40925	2 35875	5 23088	0 52725	6 32006	1 44300
	Median	6 74325	1 80375	5 10600	0.86025	5.77200	1 30425
	SD	2 272339	2 776665	0 542605	1 553009	1.922219	2 301363
	(SE)	(1.136170)	(1 388332)	(0.271303)	(0.776504)	(0 679607)	(0 813655)
	Min,	5 5500,	-0.3330,	4 7175,	-1 5540,	4 7175,	-1 5540,
	Max	10.6005	6 1605	5.9940	1 9425	10 6005	6.1605
Week 148	n	4	4	4	4	8	8
	Mean	6 25763	1 20713	4 91175	0.20813	5 58469	0 70763
	Median	5 27250	0 33300	4 85625	0 61050	5.18925	0 33300
	SD	2.198330	2.667996	0 622161	1 725788	1.659689	2 147611
	(SE)	(1 099165)	(1 333998)	(0.311081)	(0 862894)	(0 586789)	(0 759295)
	Min,	4 9395,	-0 9435,	4.2735,	-1 9980,	4 2735,	-1.9980,
	Max	9 5460	5 1060	5 6610	1 6095	9 5460	5 1060
Week 152	n	4	4	3	3	7	7
	Mean	6.34088	1 29038	5 03200	0 85100	5 77993	1 10207
	Median	6 43800	1 27650	5 05050	0 72150	5 60550	0 72150
	SD	0.712730	0.924676	0 582970	0 539046	0 925608	0.761267
	(SE)	(0 356365)	(0 462338)	(0 336578)	(0 311218)	(0 349847)	(0.287732)
	Min,	5 3835.	0 3885,	4 4400,	0 3885,	4 4400,	0 3885,
	Max	7 1040	2.2200	5 6055	1 4430	7 1040	2.2200

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 156	n	4	4	4	4	8	8
	Mean	5 89688	0.84638	4 63425	-0 06938	5 26556	0.38850
	Median	5 82750	0 83250	4 49550	0 02775	5.27250	0 38850
	SD	0.529194	0 812369	0 396349	1 493610	0 801771	1 215944
	(SE)	(0 264597)	(0 406185)	(0 198175)	(0 746805)	(0 283469)	(0 429901)
	Min,	5.3280,	0 0000,	4 3290,	-1 9425,	4 3290,	-1 9425,
	Max	6 6045	1 7205	5 2170	1.6095	6.6045	1.7205
Week 160	n	4	4	4	4	8	8
	Mean	5 52225	0.47175	6 32700	1 62338	5 92462	1 04756
	Median	5 41125	0.44400	6.18825	1 94250	5 66100	1 19325
	SD	0 435831	0 978753	1 357199	2 232625	1 027553	1.710480
	(SE)	(0 217916)	(0 489377)	(0 678600)	(1 116313)	(0.363295)	(0 604746)
	Min,	5 1615,	-0.6660,	4 8840,	-1.3875,	4 8840,	-1 3875,
	Max	6 1050	1 6650	8 0475	3 9960	8 0475	3 9960
Week 164	n	4	4	4	4	8	8
	Mean	6 85425	1 80375	5 06438	0 36075	5.95931	1 08225
	Median	6.90975	1 55400	4 82850	0 49950	5 96625	1 11000
	SD	0.821325	1 231467	0.724163	1 669619	1 195484	1 561908
	(SE)	(0 410662)	(0 615733)	(0 362082)	(0 834809)	(0 422667)	(0 552218)
	Min,	5 8275,	0 7770,	4 4955,	-1 6095,	4.4955,	-1 6095,
	Max	7.7700	3 3300	6 1050	2.0535	7 7700	3 3300

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 168	n	4	4	4	4	8	8
	Mean	6 32700	1 27650	5 23087	0 52725	5 77894	0.90188
	Median	6 24375	0 80475	4 71750	0 47175	5 66100	0 80475
	SD	1 069486	1 401856	1 271564	2 136330	1.235487	1 720052
	(SE)	(0.534743)	(0.700928)	(0.635782)	(1.068165)	(0.436811)	(0 608130)
	Min,	5 1060,	0.2220,	4.3845,	-1 8870,	4 3845,	-1 8870,
	Max	7 7145	3 2745	7 1040	3 0525	7 7145	3 2745

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2 8.1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Creatinine (umol/L)							
Baseline [1]	n	4		4		8	
	Mean	23 8680		26.5200		25 1940	
	Median	21 6580		26 9620		24.7520	
	SD	5.35289		2.16535		4 03719	
	(SE)	(2 67644)		(1 08267)		(1.42736)	
	Min,	20.332,		23 868,		20 332,	
	Max	31 824		28 288		31.824	
Week 2	n	4	4	4	4	8	8
	Mean	27 4040	3 5360	33.3710	6 8510	30 3875	5 1935
	Median	27 4040	3 9780	30 0560	3 0940	29 1720	3.9780
	SD	2 04151	4 73305	11.82436	10 39405	8 47821	7.68387
	(SE)	(1 02076)	(2.36652)	(5 91218)	(5 19703)	(2 99750)	(2 71666)
	Min,	25 636,	-2 652,	22 984,	-0 884,	22 984,	-2 652,
	Max	29 172	8 840	50.388	22 100	50 388	22 100
Week 4	n	4	4	4	4	8	8
	Mean	24 0890	0 2210	27 4040	0 8840	25 7465	0 5525
	Median	24 3100	-0 8840	28 2880	0 4420	27.4040	0 4420
	SD	4 69852	4 41263	2 39388	1.25016	3 88033	3.02328
	(SE)	(2.34926)	(2 20631)	(1.19694)	(0 62508)	(1.37190)	(1.06889)
	Min,	18 564,	-3.536,	23 868,	0.000,	18.564,	-3 536,
	Max	29 172	6 188	29.172	2 652	29 172	6.188

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run, 06MAR2015 14 03
Reference listing L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	25 1940	1 3260	28 0670	1 5470	26 6305	1.4365
	Median	25 1940	0 8840	24.7520	0 0000	24 7520	0 0000
	SD	3 10451	5 12923	7 26728	6 38992	5 39659	5.36547
	(SE)	(1 55225)	(2.56462)	(3 63364)	(3.19496)	(1 90798)	(1 89698)
	Min,	22 100,	-4 420,	23.868,	-4 420,	22 100,	-4 420,
	Max	28.288	7 956	38 896	10 608	38 896	10 608
Week 8	n	4	4	4	4	8	8
	Mean	24.7520	0 8840	24.9730	-1 5470	24.8625	-0.3315
	Median	24 3100	0 4420	24 7520	-1 7680	24 7520	-0 4420
	SD	3 14618	5 44934	0 44200	1 96014	2 08324	4 00771
	(SE)	(1 57309)	(2.72467)	(0.22100)	(0.98007)	(0.73654)	(1 41694)
	Min.	22 100,	-5 304,	24 752,	-3.536,	22.100,	-5 304,
	Max	28.288	7 956	25.636	0 884	28 288	7 956
Week 10	n	4	4	4	4	8	8
	Mean	26 2990	2.4310	27.1830	0 6630	26 7410	1 5470
	Median	26 9620	3.5360	27 4040	1 3260	26 9620	2 6520
	SD	1 67338	4 91528	2 63969	3 84481	2 09992	4 19318
	(SE)	(0 83669)	(2.45764)	(1 31985)	(1 92240)	(0 74243)	(1 48251)
	Min.	23.868,	-4 420,	23 868,	-4 420,	23 868,	-4.420,
	Max	27 404	7.072	30 056	4 420	30 056	7 072

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run: 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	24 3100	0.4420	26 9620	0 4420	25 6360	0.4420
	Median	22 5420	0 4420	27.4040	0.4420	23 8680	0 4420
	SD	5 88596	2 55189	5 22981	4.70545	5 34593	3 50429
	(SE)	(2.94298)	(1.27594)	(2.61491)	(2 35272)	(1.89007)	(1 23895)
	Min.	19 448,	-2 652,	21 216,	-4 420,	19 448,	-4.420,
	Max	32.708	3 536	31 824	5 304	32 708	5.304
Week 15	n	4	4	4	4	8	8
	Mean	24 7520	0 8840	28.0670	1 5470	26 4095	1 2155
	Median	22 9840	0 0000	28 2880	0 0000	25 6360	0 0000
	SD	5 00066	2.39388	4 91528	4 58630	4 92049	3.40533
	(SE)	(2.50033)	(1 19694)	(2 45764)	(2 29315)	(1 73965)	(1 20396)
	Min.	21 216,	-0 884,	22 100,	-1 768,	21 216,	-1.768,
	Max	31 824	4 420	33 592	7 956	33 592	7 956
Week 18	n	4	4	4	4	8	8
	Mean	24 3100	0.4420	29 1720	2.6520	26 7410	1 5470
	Median	22 9840	0 4420	28 7300	1.7680	26 0780	0 4420
	SD	6 14576	3 26801	4 14633	6 25082	5 50539	4 76634
	(SE)	(3.07288)	(1 63401)	(2 07316)	(3 12541)	(1 94645)	(1 68516)
	Min.	18.564,	-3 536,	24.752,	-3 536,	18 564,	-3 536,
	Max	32 708	4 420	34 476	10 608	34 476	10.608

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 21	n	4	4	4	4	8	8
	Mean	23.6470	-0.2210	24.0890	-2.4310	23 8680	-1 3260
	Median	20 7740	-0 4420	24 3100	-3 0940	22.9840	-3 0940
	SD	8 14608	3 84481	3 09400	3 00863	5 70946	3 40737
	(SE)	(4.07304)	(1 92240)	(1 54700)	(1 50432)	(2.01860)	(1 20469)
	Min,	17 680,	-3 536,	20 332,	-5.304,	17 680,	-5 304,
	Max	35.360	3 536	27 404	1 768	35.360	3.536
Week 24	n	4	4	4	4	8	8
	Mean	22 7630	-1 1050	26 0780	-0.4420	24 4205	-0 7735
	Median	21.2160	-2 2100	27 4040	0 0000	25 1940	-1.3260
	SD	4 58630	2 53910	3 92029	2.33884	4 32909	2 28757
	(SE)	(2 29315)	(1 26955)	(1 96014)	(1 16942)	(1.53056)	(0 80878)
	Min,	19 448,	-2 652,	20 332,	-3 536,	19.448,	-3 536,
	Max	29 172	2 652	29 172	1 768	29 172	2.652
Week 25	n	4	4	4	4	8	8
	Mean	26 2990	2 4310	26 5200	0 0000	26 4095	1 2155
	Median	26 0780	1 3260	27 8460	0 4420	27 8460	1 3260
	SD	5 65461	4 52915	4 27013	2 79545	4 64025	3 71873
	(SE)	(2 82730)	(2 26458)	(2.13506)	(1 39773)	(1.64058)	(1 31477)
	Min,	20 332,	-1 768,	20 332,	-3 536,	20 332,	-3.536,
	Max	32 708	8.840	30.056	2.652	32.708	8 840

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 28	n	4	4	4	4	8	8
	Mean	23 2050	-0.6630	33.1500	6 6300	28 1775	2 9835
	Median	22.5420	-2 6520	32.2660	5.3040	28 7300	0.4420
	SD	6 47094	5.27322	6.31302	8 37082	7 95512	7.55936
	(SE)	(3 23547)	(2 63661)	(3.15651)	(4 18541)	(2 81256)	(2.67264)
	Min,	17.680,	-4 420,	26 520,	-1 768,	17 680,	-4 420,
	Max	30 056	7 072	41 548	17 680	41 548	17 680
Week 32	n	4	4	3	3	7	7
	Mean	16 7960	-7 0720	19 7427	-7 6613	18 0589	-7 3246
	Median	15 0280	-6 6300	20 3320	-7 9560	16 7960	-7.9560
	SD	6 96062	3 46155	2 70066	3 98618	5 39788	3 37445
	(SE)	(3 48031)	(1 73077)	(1 55923)	(2.30142)	(2 04021)	(1 27542)
	Min,	10 608,	-11 492,	16 796,	-11 492,	10 608,	-11.492,
	Max	26.520	-3 536	22.100	-3 536	26.520	-3.536
Week 36	n	4	4	4	4	8	8
	Mean	17 9010	-5 9670	19 0060	-7 5140	18.4535	-6 7405
	Median	16.7960	-5 7460	19 8900	-7.0720	18 1220	-6.6300
	SD	3 56352	2.21000	3 57264	1 53113	3 35579	1 94466
	(SE)	(1 78176)	(1 10500)	(1 78632)	(0.76557)	(1.18645)	(0 68754)
	Min,	15 028,	-8 840,	14 144,	-9 724,	14 144,	-9 724,
	Max	22 984	-3.536	22 100	-6.188	22 984	-3.536

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run, 06MAR2015 14:03
Reference listing L.16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 40	n	4	4	4	4	8	8
	Mean	20 1110	-3 7570	20 7740	-5.7460	20 4425	-4 7515
	Median	18 5640	-3 5360	20 3320	-6 1880	19.0060	-3 9780
	SD	4 41263	1 32600	5 27939	4 23952	4 51836	3 09625
	(SE)	(2 20631)	(0.66300)	(2 63969)	(2.11976)	(1 59748)	(1 09469)
	Min,	16 796,	-5 304,	15 028,	-9.724,	15.028,	-9 724,
	Max	26 520	-2 652	27.404	-0 884	27 404	-0.884
Week 44	n	4	4	4	4	8	8
	Mean	20 5530	-3 3150	22 1000	-4 4200	21 3265	-3 8675
	Median	17 6800	-3 0940	22 1000	-4 8620	21 2160	-3 9780
	SD	6 97464	2 21000	1 44357	1 90966	4 73550	2 00124
	(SE)	(3 48732)	(1 10500)	(0 72178)	(0 95483)	(1 67425)	(0 70755)
	Min,	15 912,	-6.188,	20 332,	-6 188,	15 912,	-6 188,
	Max	30 940	-0 884	23 868	-1 768	30 940	-0 884
Week 48	n	4	4	4	4	8	8
	Mean	16 7960	-7 0720	23 6470	-2 8730	20 2215	-4 9725
	Median	15 9120	-6 6300	23.4260	-3 5360	19.0060	-5.7460
	SD	5.15456	2 79545	6.59059	5 22358	6.58883	4 48114
	(SE)	(2 57728)	(1 39773)	(3.29530)	(2.61179)	(2 32950)	(1 58432)
	Min,	11 492,	-10.608,	15.912,	-7 956,	11.492,	-10 608,
	Max	23.868	-4 420	31 824	3 536	31 824	3 536

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 52	n	4	4	4	4	8	8
	Mean	18.3430	-5.5250	23 4260	-3 0940	20 8845	-4.3095
	Median	16.7960	-6 6300	20.7740	-4 4200	19 8900	-5 3040
	SD	5 22358	2.53910	6 22995	5 27939	5.97576	4 04927
	(SE)	(2 61179)	(1 26955)	(3 11498)	(2 63969)	(2 11275)	(1 43163)
	Min,	14 144,	-7 072,	19 448,	-7 956,	14 144,	-7.956,
	Max	25.636	-1 768	32 708	4 420	32 708	4.420
Week 56	n	4	4	3	3	7	7
	Mean	16.5750	-7 2930	27 1093	0 2947	21 0897	-4 0411
	Median	15 0280	-7.9560	20 3320	-5 3040	18 5640	-7.9560
	SD	4 41263	1 96014	13 29923	12 06695	10 01990	8.17970
	(SE)	(2 20631)	(0 98007)	(7 67831)	(6.96686)	(3 78717)	(3 09164)
	Min,	13.260,	-8 840,	18.564,	-7 956,	13 260,	-8 840,
	Max	22 984	-4 420	42 432	14 144	42.432	14 144
Week 60	n	4	4	4	4	8	8
	Mean	16.5750	-7 2930	19 0060	-7.5140	17 7905	-7 4035
	Median	15 4700	-8 8400	18.1220	-6 6300	18 1220	-7 9560
	SD	5.17348	3 70683	2 10434	2 10434	3.88033	2 79296
	(SE)	(2 58674)	(1 85342)	(1 05217)	(1 05217)	(1 37190)	(0 98746)
	Min,	12 376,	-9 724,	17 680,	-10 608,	12 376,	-10 608,
	Max	22 984	-1.768	22.100	-6 188	22.984	-1 768

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run. 06MAR2015:14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 64	n	4	4	4	4	8	8
	Mean	15.2490	-8 6190	15 6910	-10 8290	15 4700	-9.7240
	Median	13 7020	-9 7240	17 2380	-10 6080	16 3540	-9 7240
	SD	5 02015	2 83018	4.04295	2 63969	4.22633	2 79545
	(SE)	(2.51008)	(1.41509)	(2.02148)	(1 31985)	(1.49423)	(0 98834)
	Min,	11 492,	-10 608,	9 724,	-14.144,	9 724,	-14 144,
	Max	22 100	-4 420	18 564	-7.956	22 100	-4 420
Week 68	n	4	4	4	4	8	8
	Mean	17 0170	-6 8510	17 4590	-9 0610	17 2380	-7 9560
	Median	15 9120	-7.5140	16 7960	-8 8400	16 7960	-8 3980
	SD	3 77645	3 00863	2 63969	1.82241	3.02559	2 58809
	(SE)	(1.88822)	(1 50432)	(1.31985)	(0 91121)	(1 06971)	(0 91503)
	Min,	14 144,	-9 724,	15 028,	-11 492,	14 144,	-11 492,
	Max	22 100	-2 652	21 216	-7 072	22 100	-2 652
Week 72	n	3	3	4	4	7	7
	Mean	14 4387	-10 6080	19.4480	-7.0720	17.3011	-8 5874
	Median	11 4920	-10 6080	18.1220	-8.3980	17 6800	-9 7240
	SD	5 88596	0 00000	4 94829	3 60891	5 56422	3.17561
	(SE)	(3 39826)	(0 00000)	(2 47415)	(1 80446)	(2 10308)	(1 20027)
	Min,	10 608,	-10 608,	15.028,	-9 724,	10 608,	-10 608,
	Max	21.216	-10 608	26.520	-1.768	26 520	-1 768

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 73	n	2	2	0	0	2	2
	Mean	21 2160	-4 8620			21 2160	-4 8620
	Median	21 2160	-4 8620			21 2160	-4 8620
	SD	5.00066	3 12541			5 00066	3 12541
	(SE)	(3 53600)	(2 21000)			(3 53600)	(2 21000)
	Min,	17 680,	-7.072,			17.680,	-7.072,
	Max	24 752	-2.652			24 752	-2 652
Week 76	n	4	4	4	4	8	8
	Mean	18.7850	-5 0830	19 4480	-7 0720	19 1165	-6 0775
	Median	16 3540	-4.4200	19 4480	-7 5140	18 1220	-5 3040
	SD	5.79116	1 32600	2.16535	2 16535	4 06304	1.97315
	(SE)	(2 89558)	(0 66300)	(1 08267)	(1 08267)	(1 43650)	(0.69761)
	Min,	15 028,	-7 072,	16 796,	-8.840,	15 028,	-8.840,
	Max	27 404	-4 420	22.100	-4 420	27 404	-4 420
Week 80	n	4	4	4	4	8	8
	Mean	16 3540	-7 5140	17.6800	-8.8400	17 0170	-8.1770
	Median	15 9120	-7 0720	18 5640	-9.7240	16 7960	-8 3980
	SD	2 93190	3 01943	3 14618	2 50033	2 90320	2 66250
	(SE)	(1 46595)	(1 50972)	(1 57309)	(1 25016)	(1 02644)	(0 94134)
	Min,	13 260,	-11 492,	13.260,	-10 608,	13 260,	-11 492,
	Max	20.332	-4.420	20.332	-5 304	20 332	-4 420

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015 14.03
Reference listing L.16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 84	n	4	4	4	4	8	8
	Mean	18 5640	-5 3040	18 3430	-8.1770	18 4535	-6 7405
	Median	17 6800	-6 1880	20 3320	-7.5140	19 8900	-7 0720
	SD	4.01872	3 14618	4 58630	3.00863	3.99375	3 23726
	(SE)	(2 00936)	(1.57309)	(2 29315)	(1.50432)	(1 41201)	(1 14455)
	Min,	15 028,	-7 956,	11.492,	-12 376,	11 492,	-12.376,
	Max	23 868	-0 884	21 216	-5.304	23 868	-0 884
Week 88	n	4	4	4	4	8	8
	Mean	17 0170	-6 8510	41 5480	15 0280	29 2825	4 0885
	Median	16 7960	-8 3980	22 1000	-4 8620	20.3320	-7 0720
	SD	4 52915	4 16982	40 68962	42 51173	29 83769	30 31097
	(SE)	(2 26458)	(2 08491)	(20 34481)	(21 25587)	(10.54922)	(10 71655)
	Min,	12 376,	-9 724,	19 448,	-8 840,	12 376,	-9 724,
	Max	22 100	-0 884	102 544	78.676	102.544	78 676
Week 92	n	4	4	4	4	8	8
	Mean	17 4590	-6 4090	19 6690	-6 8510	18 5640	-6 6300
	Median	16 3540	-7 0720	20.3320	-6 6300	19 0060	-7 0720
	SD	4 64275	2 73660	3 33703	1 82241	3.92503	2 16535
	(SE)	(2 32138)	(1 36830)	(1.66851)	(0 91121)	(1 38771)	(0 76557)
	Min,	13 260,	-8 840,	15 028,	-8.840,	13.260,	-8 840,
	Max	23 868	-2 652	22 984	-5.304	23 868	-2 652

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 96	n	4	4	4	4	8	8
	Mean	17 6800	-6 1880	20 3320	-6 1880	19 0060	-6 1880
	Median	17 2380	-6 1880	19.8900	-6 1880	19 0060	-6 1880
	SD	2 79545	3.60891	2.60243	1 44357	2 87421	2.54459
	(SE)	(1 39773)	(1 80446)	(1 30121)	(0 72178)	(1 01619)	(0 89965)
	Min,	15 028,	-10.608,	17.680,	-7 956,	15.028,	-10 608,
	Max	21 216	-1 768	23.868	-4.420	23 868	-1 768
Week 100	n	4	4	4	4	8	8
	Mean	16 5750	-7 2930	19 4480	-7 0720	18 0115	-7.1825
	Median	15 4700	-7 9560	18 5640	-7 9560	17 2380	-7 9560
	SD	5 51468	3 33703	4.01872	2 39388	4 72370	2 69117
	(SE)	(2 75734)	(1 66851)	(2 00936)	(1 19694)	(1.67008)	(0.95147)
	Min,	11 492,	-10 608,	15 912,	-8 840,	11 492,	-10 608,
	Max	23 868	-2.652	24 752	-3.536	24 752	-2 652
Week 104	n	4	4	4	4	8	8
	Mean	19 2270	-4 6410	17.9010	-8 6190	18 5640	-6 6300
	Median	18 1220	-6 1880	18.1220	-8 8400	18 1220	-7.0720
	SD	6 14046	4 41263	1.50972	3 56352	4.19983	4 27883
	(SE)	(3 07023)	(2 20631)	(0 75486)	(1 78176)	(1 48486)	(1 51280)
	Min,	14 144,	-7 956,	15 912,	-12 376,	14 144,	-12.376,
	Max	26.520	1 768	19.448	-4 420	26 520	1 768

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 108	n	4	4	4	4	8	8
	Mean	18 1220	-5 7460	17 9010	-8 6190	18 0115	-7 1825
	Median	18 1220	-8 3980	18 5640	-8.8400	18 5640	-8 8400
	SD	7 16349	6 86643	1.96014	1 11234	4 86343	4.80571
	(SE)	(3 58175)	(3 43321)	(0 98007)	(0 55617)	(1 71948)	(1 69907)
	Min,	11 492,	-10 608,	15 028,	-9.724,	11 492,	-10 608,
	Max	24 752	4 420	19 448	-7 072	24 752	4 420
Week 112	n	4	4	4	4	8	8
	Mean	19 2270	-4 6410	20.3320	-6.1880	19.7795	-5.4145
	Median	19 4480	-7.0720	19 4480	-6 6300	19.4480	-7.0720
	SD	6 93719	6 74684	3 75049	2.60243	5.19635	4 80571
	(SE)	(3 46860)	(3.37342)	(1.87525)	(1 30121)	(1 83719)	(1.69907)
	Min,	12 376,	-9 724,	16 796,	-8 840,	12 376,	-9 724,
	Max	25 636	5 304	25 636	-2 652	25 636	5 304
Week 116	n	4	4	4	4	8	8
	Mean	17 4590	-6.4090	16.7960	-9 7240	17 1275	-8 0665
	Median	17.2380	-7 9560	16 7960	-10.1660	16 7960	-8 3980
	SD	6 01185	5 02015	3 60891	2 16535	4 60402	3.99375
	(SE)	(3.00592)	(2 51008)	(1 80446)	(1 08267)	(1 62777)	(1 41201)
	Min,	11 492,	-10 608,	12 376,	-11 492,	11 492,	-11 492,
	Max	23.868	0 884	21 216	-7.072	23 868	0.884

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run: 06MAR2015 14.03
Reference listing: L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 120	n	3	3	4	4	7	7
	Mean	15 0280	-10 0187	16 1330	-10.3870	15 6594	-10 2291
	Median	12 3760	-9 7240	16 3540	-10 6080	15 0280	-10.6080
	SD	6.18800	1 35033	2 43435	0.44200	4.00945	0 86270
	(SE)	(3.57264)	(0 77961)	(1 21717)	(0.22100)	(1 51543)	(0.32607)
	Min,	10 608,	-11 492,	13 260,	-10.608,	10.608,	-11.492,
	Max	22 100	-8 840	18 564	-9.724	22.100	-8 840
Week 124	n	3	3	4	4	7	7
	Mean	15 6173	-9 4293	16 5750	-9 9450	16 1646	-9.7240
	Median	12 3760	-8 8400	16.3540	-10.1660	15 9120	-8 8400
	SD	6.39501	1.02076	0.84637	2.83018	3.77522	2 10434
	(SE)	(3.69216)	(0.58933)	(0 42318)	(1 41509)	(1 42690)	(0 79537)
	Min,	11 492,	-10 608,	15.912,	-12 376,	11 492,	-12 376,
	Max	22 984	-8 840	17 680	-7 072	22 984	-7.072
Week 128	n	4	4	4	4	8	8
	Mean	15 4700	-8 3980	17 6800	-8 8400	16 5750	-8 6190
	Median	14.5860	-10 1660	19.0060	-8.3980	18.5640	-8.8400
	SD	6 39501	4 59340	3.60891	1 90966	4 95017	3 26516
	(SE)	(3 19751)	(2.29670)	(1 80446)	(0 95483)	(1 75015)	(1 15441)
	Min,	9 724,	-11 492,	12.376,	-11 492,	9 724,	-11 492,
	Max	22.984	-1 768	20 332	-7 072	22 984	-1 768

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run 06MAR2015.14 03
Reference listing: L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 132	n	4	4	4	4	8	8
	Mean	16 3540	-7.5140	19 2270	-7 2930	17 7905	-7 4035
	Median	16.3540	-10 1660	17.2380	-9 7240	17.2380	-10.1660
	SD	6 14576	6.22995	4 91528	6.89954	5 37586	6 08682
	(SE)	(3 07288)	(3 11498)	(2 45764)	(3 44977)	(1 90066)	(2 15202)
	Min,	10 608,	-11 492,	15 912,	-12 376,	10 608,	-12.376,
	Max	22 100	1 768	26 520	2 652	26 520	2 652
Week 136	n	4	4	4	4	8	8
	Mean	15 6910	-8.1770	16.3540	-10 1660	16.0225	-9 1715
	Median	14 5860	-9.7240	16 7960	-10 1660	16 7960	-9 7240
	SD	5 17348	3 70683	3 01943	1.14124	3 93745	2 75270
	(SE)	(2 58674)	(1 85342)	(1 50972)	(0 57062)	(1 39210)	(0 97322)
	Min,	11 492,	-10 608,	12 376,	-11.492,	11 492,	-11.492,
	Max	22.100	-2 652	19 448	-8 840	22 100	-2 652
Week 140	n	4	4	4	4	8	8
	Mean	14 1440	-9 7240	15 6910	-10.8290	14.9175	-10 2765
	Median	14 1440	-11 9340	15.9120	-11.0500	15.9120	-11 4920
	SD	5 10378	5 35289	2.63969	4 35320	3 85146	4 55527
	(SE)	(2 55189)	(2.67644)	(1 31985)	(2 17660)	(1.36170)	(1.61053)
	Min,	9 724,	-13 260,	12 376,	-15 912,	9.724,	-15 912,
	Max	18 564	-1 768	18 564	-5 304	18.564	-1 768

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015·14·03
Reference listing. L 16 2 8.1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 144	n	4	4	4	4	8	8
	Mean	16 5750	-7 2930	19 2270	-7 2930	17 9010	-7 2930
	Median	14 1440	-7 5140	17 6800	-7 9560	15 9120	-7 5140
	SD	5 51468	1.50972	5 07178	4.10688	5 10560	2 86449
	(SE)	(2.75734)	(0 75486)	(2 53589)	(2.05344)	(1 80510)	(1 01275)
	Min,	13 260,	-8 840,	15 028,	-11 492,	13 260,	-11 492,
	Max	24.752	-5 304	26 520	-1.768	26 520	-1 768
Week 148	n	4	4	4	4	8	8
	Mean	16 3540	-7.5140	19 4480	-7.0720	17 9010	-7 2930
	Median	14 5860	-8 3980	18 5640	-7 9560	18 1220	-7 9560
	SD	7 23585	4 47855	3 81932	2.39388	5 60586	3.33284
	(SE)	(3 61793)	(2 23927)	(1.90966)	(1.19694)	(1.98197)	(1 17834)
	Min,	10 608,	-11 492,	15 912,	-8.840,	10.608,	-11 492,
	Max	25 636	-1 768	24.752	-3 536	25.636	-1.768
Week 152	n	4	4	3	3	7	7
	Mean	15 0280	-8 8400	15.0280	-12 3760	15 0280	-10 3554
	Median	13 7020	-9 2820	17.6800	-10 6080	15 0280	-9 7240
	SD	5 05248	2 60243	5.37716	6 37461	4 73305	4.52813
	(SE)	(2 52624)	(1 30121)	(3.10451)	(3 68039)	(1 78892)	(1 71147)
	Min,	10 608,	-11 492,	8.840,	-19 448,	8 840,	-19 448,
	Max	22 100	-5 304	18.564	-7 072	22 100	-5 304

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing L 16 2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 156	n	4	4	4	4	8	8
	Mean	15 6910	-8.1770	16 3540	-10 1660	16 0225	-9 1715
	Median	14 5860	-9 2820	17 2380	-10 1660	17.2380	-9.2820
	SD	5 79116	3 84481	3 42372	2.10434	4.41842	3.05999
	(SE)	(2 89558)	(1 92240)	(1 71186)	(1 05217)	(1 56215)	(1.08187)
	Min.	10 608,	-11 492,	11 492,	-12.376,	10 608,	-12 376,
	Max	22 984	-2 652	19 448	-7 956	22.984	-2 652
Week 160	n	4	4	4	4	8	8
	Mean	14 8070	-9 0610	15 4700	-11 0500	15.1385	-10 0555
	Median	14 1440	-10.6080	16 7960	-11 4920	16 3540	-10 6080
	SD	6 55095	5 07178	5 07819	4.23952	5 43781	4 45616
	(SE)	(3 27548)	(2 53589)	(2.53910)	(2 11976)	(1 92255)	(1.57549)
	Min.	8.840,	-13 260,	8 840,	-15 028,	8 840,	-15 028,
	Max	22 100	-1 768	19 448	-6.188	22.100	-1.768
Week 164	n	4	4	4	4	8	8
	Mean	13.4810	-10 3870	15.2490	-11 2710	14 3650	-10 8290
	Median	12 3760	-11.9340	16.3540	-11 4920	15 9120	-11.9340
	SD	5.65461	4 04295	3 17708	1 96014	4 34999	2 97911
	(SE)	(2 82730)	(2 02148)	(1 58854)	(0 98007)	(1 53795)	(1 05328)
	Min.	8 840,	-13 260,	10 608,	-13.260,	8.840,	-13 260,
	Max	20 332	-4 420	17.680	-8.840	20 332	-4.420

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 168	n	4	4	4	4	8	8
	Mean	14 1440	-9 7240	18 7850	-7 7350	16 4645	-8 7295
	Median	13 7020	-11.9340	17.6800	-9 2820	17.6800	-10 6080
	SD	5 72897	4 73305	5.56171	4 16982	5.78593	4.26413
	(SE)	(2 86449)	(2 36652)	(2 78086)	(2 08491)	(2 04564)	(1.50760)
	Min.	8 840.	-12 376.	13 260.	-10.608.	8 840.	-12 376.
	Max	20.332	-2 652	26 520	-1.768	26.520	-1 768

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015.14 03
Reference listing L 16 2 8 1.1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4.1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Blood Urea Nitrogen (mmol/L)							
Baseline [1]	n	4		4		8	
	Mean	2 9453		3 0345		2 9899	
	Median	2 6775		3 2130		3 2130	
	SD	1 40928		0 61834		1 00862	
	(SE)	(0 70464)		(0 30917)		(0.35660)	
	Min,	1 785,		2 142,		1 785,	
	Max	4 641		3 570		4.641	
Week 2	n	4	4	4	4	8	8
	Mean	3 3915	0 4463	4 7303	1 6958	4.0609	1 0710
	Median	2.8560	0 3570	4 4625	1.7850	4 1055	0.7140
	SD	1 68712	0.44922	0 93889	1 06603	1 45249	1 00975
	(SE)	(0 84356)	(0.22461)	(0.46945)	(0 53302)	(0 51353)	(0 35700)
	Min,	2 142,	0 000,	3.927,	0 357,	2 142,	0 000,
	Max	5 712	1 071	6 069	2 856	6 069	2.856
Week 4	n	4	4	4	4	8	8
	Mean	3.7485	0 8033	3 9270	0 8925	3 8378	0 8479
	Median	3 5700	0 7140	3.7485	0 5355	3.5700	0.5355
	SD	0 94453	0 53550	1 39793	1 10996	1 10859	0 80819
	(SE)	(0.47227)	(0 26775)	(0 69897)	(0 55498)	(0 39195)	(0 28574)
	Min,	2 856,	0 357,	2.499,	0 000,	2.499,	0 000,
	Max	4 998	1.428	5 712	2 499	5.712	2 499

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14 03
Reference listing L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	3 4808	0 5355	3 5700	0.5355	3.5254	0 5355
	Median	3 0345	0.8925	3 3915	0.7140	3 0345	0.8925
	SD	1 60650	1 38266	1.27057	0 94453	1.34172	1 09620
	(SE)	(0 80325)	(0.69133)	(0 63529)	(0.47227)	(0.47437)	(0.38757)
	Min,	2 142,	-1 428,	2.499,	-0.714,	2.142,	-1 428,
	Max	5.712	1 785	4 998	1 428	5 712	1 785
Week 8	n	4	4	4	4	8	8
	Mean	3.3023	0.3570	4.4625	1 4280	3 8824	0 8925
	Median	3 2130	0 1785	4 4625	1 6065	3 9270	0 8925
	SD	0 73597	0 87447	0.74315	0.87447	0 92383	0 99155
	(SE)	(0 36799)	(0.43723)	(0 37158)	(0 43723)	(0 32662)	(0 35057)
	Min,	2 499,	-0 357,	3 570,	0.357,	2.499,	-0 357,
	Max	4.284	1.428	5 355	2 142	5 355	2 142
Week 10	n	4	4	4	4	8	8
	Mean	3 6593	0 7140	4.4625	1 4280	4 0609	1 0710
	Median	3 3915	0 7140	4 6410	1.7850	3 9270	0 8925
	SD	1 28305	0 29149	1 58319	1 51462	1.40146	1 07947
	(SE)	(0 64152)	(0 14574)	(0 79160)	(0.75731)	(0 49549)	(0 38165)
	Min,	2 499,	0 357,	2 499,	-0 714,	2.499,	-0 714,
	Max	5 355	1.071	6 069	2 856	6.069	2 856

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run: 06MAR2015 14 03
Reference listing L 16 2 8.1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	4 1055	1 1603	4 6410	1 6065	4 3733	1.3834
	Median	4 1055	1 4280	4.6410	1.6065	4 4625	1 6065
	SD	1 03057	1.46834	0.58298	0.84983	0.82629	1 13597
	(SE)	(0.51529)	(0 73417)	(0 29149)	(0 42491)	(0 29214)	(0.40163)
	Min,	2.856,	-0 714,	3 927,	0.714,	2 856,	-0 714,
	Max	5 355	2 499	5.355	2 499	5.355	2 499
Week 15	n	4	4	4	4	8	8
	Mean	3 5700	0 6248	4 7303	1 6958	4.1501	1 1603
	Median	3 5700	0 3570	4 6410	1 7850	4 4625	0 7140
	SD	1 69966	0 84356	1 63273	1.82908	1 66289	1.43753
	(SE)	(0 84983)	(0 42178)	(0 81637)	(0 91454)	(0 58792)	(0 50824)
	Min,	1 785,	0 000,	2.856,	-0 357,	1 785,	-0 357,
	Max	5 355	1 785	6 783	3 570	6.783	3 570
Week 18	n	4	4	4	4	8	8
	Mean	3 7485	0 8033	4 9088	1 8743	4 3286	1 3388
	Median	3 3915	0.8925	4 6410	1 7850	4.2840	1 0710
	SD	1 44280	0 34180	0 84356	0 79160	1.25767	0 80396
	(SE)	(0 72140)	(0 17090)	(0.42178)	(0 39580)	(0 44465)	(0 28424)
	Min,	2 499,	0 357,	4 284,	1 071,	2.499,	0.357,
	Max	5 712	1 071	6.069	2.856	6 069	2 856

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015 14.03
Reference listing: L 16.2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 21	n	4	4	4	4	8	8
	Mean	3.6593	0.7140	5.0873	2 0527	4 3733	1 3834
	Median	3 5700	0 5355	4.6410	1.7850	3.5700	1 0710
	SD	1 02540	0 77121	1 89748	1 65855	1 60508	1.39495
	(SE)	(0 51270)	(0 38560)	(0 94874)	(0 82927)	(0 56748)	(0 49319)
	Min.	2.499,	0.000,	3.570,	0.357,	2 499,	0 000,
	Max	4 998	1 785	7.497	4 284	7 497	4 284
Week 24	n	4	4	4	4	8	8
	Mean	3 3915	0 4463	4.9088	1 8743	4 1501	1.1603
	Median	3 2130	0 5355	4.6410	1 7850	4 2840	0 7140
	SD	1 07100	0.34180	1.34765	1 43911	1 38840	1.23300
	(SE)	(0.53550)	(0 17090)	(0.67382)	(0.71956)	(0.49087)	(0 43593)
	Min.	2 499,	0 000,	3 570,	0 357,	2.499,	0 000,
	Max	4 641	0 714	6.783	3 570	6 783	3.570
Week 25	n	4	4	4	4	8	8
	Mean	3.4808	0 5355	4 9980	1 9635	4.2394	1.2495
	Median	3.2130	0.5355	5 1765	1 9635	4 4625	1 0710
	SD	0 84356	0.61834	0 50487	0 74315	1 03534	0 99155
	(SE)	(0 42178)	(0 30917)	(0 25244)	(0 37158)	(0.36605)	(0 35057)
	Min.	2.856,	0 000,	4 284,	1 071,	2.856,	0 000,
	Max	4 641	1 071	5 355	2 856	5.355	2 856

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 28	n	4	4	4	4	8	8
	Mean	3 4808	0 5355	4 3733	1 3388	3 9270	0 9371
	Median	3 0345	0 5355	4.6410	1 2495	3 9270	0 8925
	SD	1 60650	0 46089	0.84356	1 17954	1 28009	0 93363
	(SE)	(0 80325)	(0 23044)	(0.42178)	(0 58977)	(0 45258)	(0 33009)
	Min,	2 142,	0 000,	3 213,	0 000,	2 142,	0 000,
	Max	5.712	1 071	4.998	2 856	5.712	2.856
Week 32	n	4	4	3	3	7	7
	Mean	3 8378	0 8925	4.2840	1 4280	4 0290	1 1220
	Median	3.7485	0.8925	4 6410	1 4280	3 9270	1.4280
	SD	0.60969	0 84983	0.61834	1 07100	0 60845	0 90851
	(SE)	(0 30485)	(0 42491)	(0.35700)	(0.61834)	(0 22997)	(0 34338)
	Min,	3 213,	0 000,	3 570,	0 357,	3 213,	0 000,
	Max	4 641	1 785	4 641	2 499	4 641	2 499
Week 36	n	4	4	4	4	8	8
	Mean	4 0163	1 0710	5 0873	2 0528	4 5518	1 5619
	Median	4.1055	0 8925	4 9980	1 9635	4 4625	1 6065
	SD	0.60969	1 12893	0 73597	0 60969	0 84804	0 99041
	(SE)	(0.30485)	(0.56447)	(0 36799)	(0 30485)	(0 29983)	(0 35016)
	Min,	3 213,	0.000,	4 284,	1 428,	3 213,	0 000,
	Max	4 641	2 499	6 069	2 856	6 069	2 856

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14.03
Reference listing. L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 40	n	4	4	4	4	8	8
	Mean	4 3733	1 4280	5 0872	2 0527	4 7303	1 7404
	Median	4.1055	1 4280	5 1765	2 3205	4 6410	1 6065
	SD	1.21502	0 87447	1 17954	1.28305	1.17245	1 06994
	(SE)	(0 60751)	(0 43723)	(0.58977)	(0 64152)	(0.41452)	(0 37828)
	Min,	3 213,	0 357,	3 570,	0 357,	3 213,	0 357,
	Max	6.069	2 499	6 426	3 213	6 426	3 213
Week 44	n	4	4	4	4	8	8
	Mean	3 8378	0 8925	5 2658	2.2313	4 5518	1 5619
	Median	3 7485	0 8925	4 8195	1 4280	4.4625	1 0710
	SD	1 37881	0.20611	2 14942	1 87496	1 83777	1 42720
	(SE)	(0 68940)	(0 10306)	(1.07471)	(0 93748)	(0 64975)	(0 50459)
	Min,	2.499,	0 714,	3 213,	1 071,	2 499,	0 714,
	Max	5 355	1.071	8 211	4 998	8.211	4 998
Week 48	n	4	4	4	4	8	8
	Mean	3 8378	0 8925	4 9980	1.9635	4 4179	1 4280
	Median	3.5700	0 8925	4 2840	1.4280	4 1055	1 0710
	SD	1.21502	0 20611	1 69966	1.60980	1 50180	1 20688
	(SE)	(0 60751)	(0 10306)	(0.84983)	(0 80490)	(0.53097)	(0 42670)
	Min,	2.856,	0 714,	3 927,	0 714,	2 856,	0 714,
	Max	5 355	1 071	7 497	4 284	7 497	4 284

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14:03
Reference listing: L 16 2 8 1 1.1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 52	n	4	4	4	4	8	8
	Mean	4.3733	1 4280	4 5518	1 5173	4.4625	1 4726
	Median	3 9270	1 6065	4 1055	0 8925	3 9270	1 2495
	SD	1 17954	0 50487	1 87496	1 63273	1 45328	1.11982
	(SE)	(0 58977)	(0 25244)	(0 93748)	(0.81637)	(0.51381)	(0 39592)
	Min,	3 570,	0 714,	2 856,	0 357,	2 856,	0.357,
	Max	6.069	1 785	7 140	3 927	7 140	3 927
Week 56	n	4	4	3	3	7	7
	Mean	3 9270	0.9818	5 8310	2 4990	4.7430	1 6320
	Median	3 7485	1.0710	5 3550	1.7850	4 6410	1 0710
	SD	1 27057	0.17850	2 88560	2 92217	2.14907	1 87617
	(SE)	(0 63529)	(0 08925)	(1.66600)	(1 68712)	(0 81227)	(0 70913)
	Min,	2.856,	0 714,	3 213,	0 000,	2 856,	0 000,
	Max	5 355	1 071	8 925	5.712	8 925	5 712
Week 60	n	4	4	4	4	8	8
	Mean	4 2840	1 3388	4 6410	1.6065	4 4625	1 4726
	Median	4 1055	1.4280	4.6410	1 7850	4.6410	1 4280
	SD	1 27057	0 17850	1 16596	1 31977	1 14495	0 88353
	(SE)	(0 63529)	(0 08925)	(0 58298)	(0 65989)	(0 40480)	(0 31238)
	Min,	3 213,	1 071,	3 213,	0.000,	3 213,	0 000,
	Max	5 712	1 428	6 069	2 856	6 069	2.856

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source: P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14 03
Reference listing: L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 64	n	4	4	4	4	8	8
	Mean	4.4625	1 5173	4 8195	1 7850	4 6410	1 6511
	Median	4 4625	1.6065	4.6410	1.7850	4 6410	1 6065
	SD	1 50053	0 34180	0.94453	0 92177	1.17632	0.65931
	(SE)	(0 75027)	(0.17090)	(0.47227)	(0.46089)	(0 41589)	(0 23310)
	Min,	2.856,	1 071,	3.927,	0 714,	2 856,	0 714,
	Max	6 069	1 785	6.069	2 856	6.069	2 856
Week 68	n	4	4	4	4	8	8
	Mean	4 8195	1 8743	4 9980	1 9635	4 9088	1 9189
	Median	4 4625	1 7850	4 8195	1 9635	4 6410	1 9635
	SD	1 71211	0 53550	1 12893	1 03057	1 34596	0 76181
	(SE)	(0 85606)	(0 26775)	(0.56447)	(0 51529)	(0.47587)	(0.26934)
	Min,	3 213,	1 428,	3 927,	0 714,	3.213,	0 714,
	Max	7 140	2 499	6 426	3 213	7.140	3 213
Week 72	n	3	3	4	4	7	7
	Mean	3.8080	1 4280	4.6410	1 6065	4.2840	1 5300
	Median	3 5700	1 4280	4 4625	1 6065	3 5700	1.4280
	SD	0 74315	0 35700	1 77306	1.60980	1 39793	1.16074
	(SE)	(0 42906)	(0 20611)	(0.88653)	(0.80490)	(0 52837)	(0 43872)
	Min,	3 213,	1 071,	2 856,	-0 357,	2 856,	-0.357,
	Max	4 641	1 785	6 783	3.570	6 783	3.570

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run. 06MAR2015:14.03
Reference listing: L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 73	n	2	2	0	0	2	2
	Mean	6 2475	2 1420			6 2475	2 1420
	Median	6.2475	2 1420			6 2475	2.1420
	SD	2 27193	1 51462			2 27193	1 51462
	(SE)	(1 60650)	(1 07100)			(1 60650)	(1 07100)
	Min,	4.641,	1 071,			4 641,	1 071,
	Max	7 854	3 213			7 854	3 213
Week 76	n	4	4	4	4	8	8
	Mean	4 2840	1 3388	4 9088	1 8743	4 5964	1.6065
	Median	4 1055	1 4280	4 9980	2.1420	4.1055	1 6065
	SD	0 87447	0 53550	1.75802	1 46834	1 32808	1 06247
	(SE)	(0 43723)	(0 26775)	(0 87901)	(0 73417)	(0.46955)	(0 37564)
	Min,	3 570,	0 714,	3 213,	0.000,	3.213,	0 000,
	Max	5 355	1.785	6 426	3 213	6 426	3 213
Week 80	n	4	4	4	4	8	8
	Mean	4.1948	1 2495	4 9088	1 8743	4 5518	1 5619
	Median	4.1055	1 0710	4 9980	2 3205	4 1055	1 6065
	SD	0 89250	0 94453	1.57983	1 34765	1.24768	1 12792
	(SE)	(0 44625)	(0 47227)	(0 78992)	(0 67382)	(0 44112)	(0 39878)
	Min,	3 213,	0 357,	3.213,	0 000,	3 213,	0 000,
	Max	5 355	2 499	6 426	2.856	6 426	2 856

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 84	n	4	4	4	4	8	8
	Mean	4 1948	1 2495	4 6410	1 6065	4.4179	1.4280
	Median	3 9270	1 2495	4 2840	1 4280	4 2840	1 4280
	SD	1 60650	0 20611	1.62294	1 47195	1 51387	0 99155
	(SE)	(0.80325)	(0.10306)	(0 81147)	(0 73597)	(0 53523)	(0 35057)
	Min,	2 856,	1 071,	3 213,	0.000,	2 856,	0 000,
	Max	6 069	1.428	6 783	3 570	6 783	3 570
Week 88	n	4	4	4	4	8	8
	Mean	4 5518	1.6065	8.3003	5 2658	6 4260	3 4361
	Median	4 9980	0 8925	6.9615	3 7485	5 7120	2 4990
	SD	1 52510	1 55613	4 48150	4 04294	3.69037	3.44510
	(SE)	(0 76255)	(0 77806)	(2 24075)	(2 02147)	(1 30474)	(1.21803)
	Min,	2 499,	0 714,	4 641,	2 499,	2 499,	0 714,
	Max	5 712	3 927	14 637	11 067	14 637	11 067
Week 92	n	4	4	4	4	8	8
	Mean	4 6410	1.6958	4 9980	1.9635	4 8195	1 8296
	Median	3 9270	1 4280	4 6410	1 7850	4 6410	1 4280
	SD	2 01950	0.79160	1.23668	1.31977	1 56197	1 01761
	(SE)	(1 00975)	(0 39580)	(0 61834)	(0 65989)	(0 55224)	(0 35978)
	Min,	3 213,	1 071,	3 927,	0 714,	3 213,	0 714,
	Max	7 497	2 856	6 783	3 570	7 497	3 570

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab.sas, Date/time of run. 06MAR2015:14:03
Reference listing. L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 96	n	4	4	4	4	8	8
	Mean	3 8378	0 8925	5.8013	2 7668	4.8195	1 8296
	Median	3 3915	1.0710	4 9980	2 3205	4 2840	1 6065
	SD	1 02540	0 94453	2.18860	2 10952	1 89868	1.81472
	(SE)	(0.51270)	(0 47227)	(1 09430)	(1 05476)	(0 67128)	(0 64160)
	Min,	3 213,	-0 357,	4 284,	0 714,	3.213,	-0 357,
	Max	5 355	1.785	8.925	5 712	8.925	5.712
Week 100	n	4	4	4	4	8	8
	Mean	3.9270	0 9818	4.3733	1 3388	4 1501	1 1603
	Median	3 3915	0 8925	3.7485	0 7140	3 7485	0 8925
	SD	1 77306	0 60969	1 63273	1 52510	1.59584	1 09204
	(SE)	(0 88653)	(0 30485)	(0.81637)	(0 76255)	(0.56421)	(0.38610)
	Min,	2 499,	0 357,	3 213,	0 357,	2 499,	0 357,
	Max	6.426	1 785	6 783	3 570	6 783	3 570
Week 104	n	4	4	4	4	8	8
	Mean	4 1947	1 2495	4 2840	1 2495	4 2394	1 2495
	Median	4 2840	1 0710	4.2840	1 4280	4 2840	1 2495
	SD	0 73597	0.94453	1 23668	0 94453	0 94333	0 87447
	(SE)	(0 36799)	(0 47227)	(0 61834)	(0 47227)	(0 33352)	(0 30917)
	Min,	3 213,	0 357,	3.213,	0 000,	3 213,	0 000,
	Max	4 998	2.499	5 355	2 142	5 355	2 499

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 108	n	4	4	4	4	8	8
	Mean	4 1948	1 2495	4 7303	1 6958	4.4625	1 4726
	Median	4.1055	1.0710	4.6410	1 7850	4 6410	1.2495
	SD	1.55271	0 35700	1 34765	1 34765	1 37606	0 94333
	(SE)	(0 77636)	(0 17850)	(0 67382)	(0 67382)	(0.48651)	(0 33352)
	Min,	2 856,	1 071,	3 213,	0 000,	2 856,	0.000,
	Max	5.712	1.785	6 426	3 213	6 426	3 213
Week 112	n	4	4	4	4	8	8
	Mean	4 6410	1 6958	5 2658	2 2313	4 9534	1 9635
	Median	3 9270	1 7850	4 8195	1 9635	4.2840	1.7850
	SD	1 69966	0 73597	1 60650	1 49699	1 56706	1 12893
	(SE)	(0 84983)	(0 36799)	(0 80325)	(0 74849)	(0 55404)	(0 39914)
	Min,	3 570,	0 714,	3 927,	0 714,	3 570,	0 714,
	Max	7 140	2 499	7 497	4 284	7 497	4.284
Week 116	n	4	4	4	4	8	8
	Mean	4 6410	1.6958	4 8195	1 7850	4 7303	1.7404
	Median	4.2840	1 6065	4 9980	2 1420	4 9980	2 1420
	SD	2 38594	0 98310	1 58319	1 51462	1 87698	1 18307
	(SE)	(1 19297)	(0 49155)	(0.79160)	(0 75731)	(0.66361)	(0 41828)
	Min,	2 499,	0 714,	2 856,	-0.357,	2 499,	-0 357,
	Max	7.497	2 856	6.426	3 213	7 497	3 213

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen.
Source P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run. 06MAR2015 14 03
Reference listing L 16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 120	n	3	3	4	4	7	7
	Mean	3 3320	0 9520	4 6410	1 6065	4.0800	1 3260
	Median	2.8560	1 0710	4.6410	1 7850	4.2840	1 0710
	SD	0 82446	0 20611	0.65179	0 35700	0 96362	0 44752
	(SE)	(0.47600)	(0 11900)	(0.32589)	(0 17850)	(0 36421)	(0 16915)
	Min.	2 856,	0.714,	3 927,	1 071,	2 856,	0.714,
	Max	4 284	1 071	5 355	1 785	5.355	1 785
Week 124	n	3	3	4	4	7	7
	Mean	3 5700	1 1900	5.3550	2 3205	4 5900	1 8360
	Median	2 8560	1 0710	5 8905	2 6775	5 3550	1 7850
	SD	1 55613	0 54533	1 45745	0 84983	1 66721	0.90851
	(SE)	(0 89843)	(0 31484)	(0 72872)	(0 42491)	(0.63015)	(0 34338)
	Min.	2 499,	0 714,	3.213,	1 071,	2 499,	0 714,
	Max	5 355	1 785	6.426	2 856	6 426	2 856
Week 128	n	4	4	4	4	8	8
	Mean	4 1948	1.2495	5.2657	2 2313	4.7303	1 7404
	Median	3 9270	1 2495	4 4625	1 2495	4 1055	1 2495
	SD	1 10516	0 46089	2 54741	2 33874	1 90586	1 64638
	(SE)	(0.55258)	(0.23044)	(1 27370)	(1 16937)	(0 67382)	(0 58208)
	Min.	3.213,	0 714,	3 213,	0 714,	3 213,	0 714,
	Max	5.712	1 785	8.925	5.712	8 925	5.712

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14.03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc.
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 132	n	4	4	4	4	8	8
	Mean	3 8378	0.8925	5.3550	2 3205	4 5964	1 6065
	Median	3 3915	0 8925	5 5335	2 6775	4 2840	1 2495
	SD	1 60650	0 46089	1.27057	1 10996	1 56706	1 09620
	(SE)	(0 80325)	(0 23044)	(0 63529)	(0.55498)	(0 55404)	(0 38757)
	Min,	2 499,	0 357,	3.927,	0 714,	2 499,	0.357,
	Max	6 069	1 428	6.426	3 213	6 426	3 213
Week 136	n	4	4	4	4	8	8
	Mean	4 4625	1 5173	5 3550	2.3205	4.9088	1.9189
	Median	3.9270	1.6065	5 3550	2 1420	4 4625	1 7850
	SD	1 60980	0 60969	1 30358	0 94453	1 43753	0.85206
	(SE)	(0.80490)	(0 30485)	(0 65179)	(0 47227)	(0 50824)	(0.30125)
	Min,	3 213,	0.714,	3.927,	1.428,	3 213,	0 714,
	Max	6 783	2 142	6 783	3 570	6 783	3 570
Week 140	n	4	4	4	4	8	8
	Mean	4 1055	1.1603	4 1055	1 0710	4 1055	1 1156
	Median	3 3915	0 7140	4 2840	1 0710	4 2840	0 8925
	SD	2 44747	1 14296	0 61834	0 29149	1.65259	0.77366
	(SE)	(1 22373)	(0.57148)	(0 30917)	(0 14574)	(0 58428)	(0.27353)
	Min,	2 142,	0 357,	3.213,	0 714,	2.142,	0.357,
	Max	7 497	2 856	4 641	1.428	7.497	2 856

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run: 06MAR2015.14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4.1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 144	n	4	4	4	4	8	8
	Mean	4 6410	1 6958	4 8195	1 7850	4 7303	1 7404
	Median	4 2840	1 7850	4 2840	1.4280	4 2840	1 7850
	SD	1.36721	1 02540	1 60980	1 74894	1 38594	1.32808
	(SE)	(0.68360)	(0.51270)	(0 80490)	(0 87447)	(0 49000)	(0 46955)
	Min,	3 570,	0 357,	3.570,	0 357,	3 570,	0 357,
	Max	6 426	2 856	7 140	3 927	7.140	3 927
Week 148	n	4	4	4	4	8	8
	Mean	3 4808	0 5355	4 8195	1 7850	4 1501	1.1603
	Median	3 3915	0 8925	5.1765	1 7850	4 1055	1 0710
	SD	0 73597	0 84983	1.18404	0 65179	1.15976	0 96833
	(SE)	(0.36799)	(0 42491)	(0 59202)	(0 32589)	(0 41004)	(0 34236)
	Min,	2 856,	-0 714,	3.213,	1 071,	2 856,	-0.714,
	Max	4.284	1 071	5 712	2 499	5 712	2 499
Week 152	n	4	4	3	3	7	7
	Mean	3 6593	0 7140	4.8790	2 0230	4 1820	1.2750
	Median	3 7485	0 8925	4 2840	2 1420	4 2840	1 0710
	SD	1 14296	0 50487	1.35158	1 25374	1.29892	1 06816
	(SE)	(0 57148)	(0 25244)	(0 78034)	(0 72385)	(0 49094)	(0 40373)
	Min,	2 499,	0 000,	3 927,	0 714,	2.499,	0 000,
	Max	4.641	1 071	6 426	3 213	6 426	3 213

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing L 16 2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 156	n	4	4	4	4	8	8
	Mean	3 3023	0 3570	5.4443	2.4098	4 3733	1 3834
	Median	3 2130	0 7140	5 3550	2 4990	4 4625	1.0710
	SD	0 93889	0 71400	0 93889	0.73597	1 43753	1 28630
	(SE)	(0 46945)	(0.35700)	(0 46945)	(0 36799)	(0 50824)	(0 45477)
	Min,	2.499,	-0 714,	4 641,	1.428,	2 499,	-0 714,
	Max	4 284	0.714	6 426	3 213	6 426	3 213
Week 160	n	4	4	4	4	8	8
	Mean	3 2130	0 2678	5 3550	2 3205	4.2840	1 2941
	Median	3 0345	0 7140	5 5335	2 6775	3 9270	1 0710
	SD	0 50487	1.17954	1 27057	1 10996	1 45328	1 52585
	(SE)	(0 25244)	(0.58977)	(0 63529)	(0.55498)	(0 51381)	(0 53947)
	Min,	2.856,	-1 428,	3 927,	0 714,	2 856,	-1 428,
	Max	3 927	1 071	6 426	3.213	6 426	3.213
Week 164	n	4	4	4	4	8	8
	Mean	3 3023	0 3570	5 7120	2.6775	4 5071	1.5173
	Median	3 3915	0.5355	6.0690	3.2130	3 9270	0 8925
	SD	0 73597	0 77121	1 82035	1 87779	1 81972	1 81785
	(SE)	(0 36799)	(0 38560)	(0.91017)	(0 93889)	(0 64337)	(0 64271)
	Min,	2 499,	-0 714,	3 213,	0.000,	2 499,	-0.714,
	Max	3 927	1 071	7 497	4.284	7 497	4 284

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14:03
Reference listing. L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14.3.4 1 1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 168	n	4	4	4	4	8	8
	Mean	3 4808	0 5355	5.9798	2 9453	4 7303	1 7404
	Median	3 3915	0 8925	5 7120	2 8560	4 8195	1 4280
	SD	0 98310	0 84983	1.21502	1.02540	1 68261	1 55540
	(SE)	(0 49155)	(0 42491)	(0.60751)	(0 51270)	(0.59489)	(0.54992)
	Min,	2 499,	-0 714,	4.998,	1 785,	2.499,	-0.714,
	Max	4 641	1 071	7 497	4 284	7.497	4 284

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P:\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14.03
Reference listing L 16 2.8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3.4 1.1 1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Protein (g/dL)							
Baseline [1]	n	4		4		8	
	Mean	6 95.		6 90		6 93	
	Median	7 05		6 80		7 00	
	SD	0 387		0.529		0 430	
	(SE)	(0.194)		(0 265)		(0.152)	
	Min.	6 4.		6 4.		6 4.	
	Max	7.3		7 6		7.6	
Week 2	n	4	4	4	4	8	8
	Mean	6 75	-0.20	6 63	-0 28	6.69	-0 24
	Median	6 55	-0 20	6 65	-0 20	6 60	-0 20
	SD	0 580	0 523	0.330	0 486	0 442	0 469
	(SE)	(0 290)	(0 261)	(0 165)	(0 243)	(0 156)	(0 166)
	Min.	6.3.	-0.7.	6 2.	-0.9.	6 2.	-0 9.
	Max	7.6	0 3	7.0	0 2	7 6	0 3
Week 4	n	4	4	4	4	8	8
	Mean	6 63	-0 33	7 13	0 23	6 88	-0 05
	Median	6 65	-0 35	7 20	0 20	6 80	-0.20
	SD	0 250	0.411	0 624	0.512	0 515	0 521
	(SE)	(0 125)	(0 206)	(0 312)	(0 256)	(0 182)	(0 184)
	Min.	6 3.	-0 8.	6 3.	-0 3.	6.3.	-0 8.
	Max	6 9	0 2	7 8	0.8	7 8	0 8

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source. P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015.14:03
Reference listing L.16.2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4 1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 6	n	4	4	4	4	8	8
	Mean	6 25	-0.70	6.68	-0 23	6 46	-0 46
	Median	6 35	-0.70	6 60	-0 30	6.45	-0 55
	SD	0 311	0.183	0.310	0.411	0 366	0.389
	(SE)	(0 155)	(0.091)	(0 155)	(0 206)	(0.129)	(0 138)
	Min,	5 8,	-0 9,	6.4,	-0 6,	5 8,	-0 9,
	Max	6 5	-0 5	7 1	0 3	7 1	0.3
Week 8	n	4	4	4	4	8	8
	Mean	6 60	-0 35	6 75	-0 15	6.68	-0.25
	Median	6 35	-0 40	6 80	-0 05	6 50	-0 05
	SD	0.606	0.526	0 370	0 238	0 471	0 393
	(SE)	(0 303)	(0 263)	(0 185)	(0.119)	(0 167)	(0 139)
	Min,	6 2,	-0 8,	6.3,	-0.5,	6 2,	-0.8,
	Max	7 5	0.2	7 1	0 0	7 5	0.2
Week 10	n	4	4	4	4	8	8
	Mean	6 40	-0.55	6 68	-0 23	6 54	-0 39
	Median	6 50	-0 45	6 70	-0 10	6 70	-0 35
	SD	0 383	0 311	0.450	0 499	0 414	0 422
	(SE)	(0 191)	(0.155)	(0.225)	(0.250)	(0.146)	(0 149)
	Min,	5 9,	-1 0,	6 1,	-0 9,	5 9,	-1 0,
	Max	6.7	-0 3	7 2	0 2	7 2	0.2

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source P \Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015:14 03
Reference listing L 16 2 8 1 1 1

Sarepta, Inc
4658-us-201 & 4658-us-202
Week 168 Analysis

Table 14 3 4.1 1.1
Summary and Change from Baseline of Serum Chemistry Laboratory Parameters
Eteplirsen for 168 Weeks
Safety Population

		Eteplirsen					
		30 mg/kg		50 mg/kg		Eteplirsen for 168 Weeks	
Time Point		Observed	Change from Baseline	Observed	Change from Baseline	Observed	Change from Baseline
Week 12	n	4	4	4	4	8	8
	Mean	6 63	-0 33	6 95	0 05	6 79	-0 14
	Median	6 55	-0 35	6 70	-0 05	6.55	-0 25
	SD	0 419	0 206	1 028	0.507	0.747	0 410
	(SE)	(0.210)	(0 103)	(0 514)	(0.253)	(0.264)	(0 145)
	Min,	6 2,	-0.5,	6 1,	-0 4,	6.1,	-0 5,
	Max	7 2	-0.1	8 3	0 7	8 3	0.7
Week 15	n	4	4	4	4	8	8
	Mean	6 28	-0 68	6 60	-0 30	6.44	-0 49
	Median	6 35	-0 70	6 60	-0 40	6.35	-0 55
	SD	0 359	0 150	0 808	0 432	0 605	0 360
	(SE)	(0.180)	(0 075)	(0 404)	(0 216)	(0 214)	(0 127)
	Min,	5 8,	-0 8,	5 9,	-0 7,	5 8,	-0 8,
	Max	6.6	-0 5	7 3	0.3	7.3	0 3
Week 18	n	4	4	4	4	8	8
	Mean	6 50	-0.45	6 68	-0.23	6.59	-0 34
	Median	6 65	-0.45	6 55	-0 20	6 65	-0 35
	SD	0 337	0 129	0.655	0 150	0 491	0 177
	(SE)	(0 168)	(0 065)	(0 328)	(0 075)	(0 174)	(0 062)
	Min,	6 0,	-0.6,	6 1,	-0 4,	6 0,	-0.6,
	Max	6 7	-0 3	7 5	-0 1	7 5	-0 1

[1]Baseline is the last non-missing assessment prior to dosing with Eteplirsen
Source: P.\Sarepta\DMD\202\Programs\Analysis\Production\Tables\t_lab sas, Date/time of run 06MAR2015 14.03
Reference listing L 16 2 8 1 1 1