



NDA 213535/S-001

**SUPPLEMENT APPROVAL**  
**FULFILLMENT OF POSTMARKETING**  
**REQUIREMENT**

Genentech, Inc.  
Attention: Anna Gruzman, PharmD  
Regulatory Program Management  
1 DNA Way  
South San Francisco, CA 94080

Dear Dr. Gruzman:

Please refer to your supplemental new drug application (sNDA) dated November 13, 2020, received November 13, 2020, submitted under section 505(b) of the Federal Food, Drug, and Cosmetic Act (FDCA) for Evrysdi (risdiplam) for oral solution.

This Prior Approval supplemental new drug application provides for updates to section 8 (Use in Specific Populations) and subsection 12.3 (Pharmacokinetics) of the Evrysdi prescribing information and updates to the patient information based on results from the completed clinical trial entitled "An Open-Label, Single-Dose, Parallel-Group, Two-Part Study to Evaluate the Pharmacokinetics And Safety Of Risdiplam In Subjects With Mild Or Moderate Hepatic Impairment Compared To Subjects With Normal Hepatic Function".

**APPROVAL & LABELING**

We have completed our review of this supplemental application, as amended. It is approved, effective on the date of this letter, for use as recommended in the enclosed agreed-upon labeling.

## **CONTENT OF LABELING**

As soon as possible, but no later than 14 days from the date of this letter, submit the content of labeling [21 CFR 314.50(l)] in structured product labeling (SPL) format using the FDA automated drug registration and listing system (eLIST), as described at FDA.gov.<sup>1</sup> Content of labeling must be identical to the enclosed labeling (text for the Prescribing Information, Patient Package Insert, Instructions for Use, and Instructions for Constitution), with the addition of any labeling changes in pending “Changes Being Effected” (CBE) supplements, as well as annual reportable changes not included in the enclosed labeling.

Information on submitting SPL files using eList may be found in the guidance for industry *SPL Standard for Content of Labeling Technical Qs and As*.<sup>2</sup>

The SPL will be accessible from publicly available labeling repositories.

Also within 14 days, amend all pending supplemental applications that include labeling changes for this NDA, including CBE supplements for which FDA has not yet issued an action letter, with the content of labeling [21 CFR 314.50(l)(1)(i)] in Microsoft Word format, that includes the changes approved in this supplemental application, as well as annual reportable changes. To facilitate review of your submission(s), provide a highlighted or marked-up copy that shows all changes, as well as a clean Microsoft Word version. The marked-up copy should provide appropriate annotations, including supplement number(s) and annual report date(s).

## **REQUIRED PEDIATRIC ASSESSMENTS**

Under the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c), all applications for new active ingredients (which includes new salts and new fixed combinations), new indications, new dosage forms, new dosing regimens, or new routes of administration are required to contain an assessment of the safety and effectiveness of the product for the claimed indication in pediatric patients unless this requirement is waived, deferred, or inapplicable.

Because none of these criteria apply to your application, you are exempt from this requirement.

## **FULFILLMENT OF POSTMARKETING REQUIREMENT**

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<sup>1</sup> <http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm>

<sup>2</sup> We update guidances periodically. For the most recent version of a guidance, check the FDA Guidance Documents Database <https://www.fda.gov/RegulatoryInformation/Guidances/default.htm>.

We have received your submission dated November 13, 2020, containing the final report for the following postmarketing requirement listed in the August 7, 2020, approval letter.

3886-4            Conduct a clinical pharmacokinetic trial to determine an appropriate dose of risdiplam to minimize toxicity in patients with different degrees of hepatic impairment. Design and conduct the trial in accordance with the FDA Guidance for Industry entitled “Pharmacokinetics in Patients with Impaired Hepatic Function: Study Design, Data Analysis, and Impact on Dosing and Labeling”.

We have reviewed your submission and conclude that the above requirement was fulfilled.

We remind you that there are postmarketing requirement(s) listed in the August 7, 2020, approval letter that are still open.

### **REPORTING REQUIREMENTS**

We remind you that you must comply with reporting requirements for an approved NDA (21 CFR 314.80 and 314.81).

If you have any questions, contact Annie Nguyen, Regulatory Project Manager, by email at [Anhtu.Nguyen@fda.hhs.gov](mailto:Anhtu.Nguyen@fda.hhs.gov) or by phone at (240) 402-4460.

Sincerely,

*{See appended electronic signature page}*

Teresa Buracchio, MD  
Deputy Director  
Division of Neurology 1  
Office of Neuroscience  
Center for Drug Evaluation and Research

### **ENCLOSURE(S):**

- Content of Labeling
  - Prescribing Information
  - Patient Package Insert
  - Instructions for Use
  - Instructions for Constitution

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**This is a representation of an electronic record that was signed electronically. Following this are manifestations of any and all electronic signatures for this electronic record.**  
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/s/  
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TERESA J BURACCHIO  
04/30/2021 01:02:04 PM