

# Risk Evaluation and Mitigation Strategy (REMS) Document

## Myalept (metreleptin) REMS Program

### I. Administrative Information

Application Number: BLA 125390  
Application Holder: Aegerion Pharmaceuticals, Inc.  
Initial REMS Approval: 02/2014  
Most Recent REMS Update: 07/2020

### II. REMS Goal

The goal of the Myalept REMS is to mitigate (1) the risks of serious adverse sequelae (such as severe infections, excessive weight gain, glucose intolerance, diabetes mellitus) due to the development of anti-metreleptin antibodies that neutralize endogenous leptin and/or Myalept, and (2) the risk of lymphoma by:

1. Educating prescribers about the development of neutralizing anti-metreleptin antibodies, the serious adverse sequelae that may result from these antibodies, and the risk for lymphoma associated with Myalept
2. Limiting the population exposed to Myalept by requiring prescriber certification, pharmacy certification, and prescriber attestation that each patient has a diagnosis consistent with the approved indication

### III. REMS Requirements

**Aegerion Pharmaceuticals, Inc. must ensure that healthcare providers, patients, outpatient pharmacies, and wholesalers-distributors comply with the following requirements:**

---

#### 1. Healthcare Providers who prescribe Myalept must:

- 
- |  |  |
|--|--|
| To become certified to prescribe         | <ol style="list-style-type: none"><li>1. Review the drug's Prescribing Information.</li><li>2. Review the following: <a href="#">Prescriber Training Module</a>.</li><li>3. Enroll in the REMS by completing the <a href="#">Prescriber Enrollment Form</a> and submitting it to the REMS Program.</li></ol> |
| Before treatment initiation (first dose) | <ol style="list-style-type: none"><li>4. Assess the patient to confirm the clinical diagnosis is consistent with the approved indication.</li><li>5. Counsel the patient on the risks of Myalept.</li><li>6. Order the prescription using the <a href="#">Prescription Authorization Form</a>.</li></ol>     |
-

---

**1. Healthcare Providers who prescribe Myalept must:**

---

During treatment;  
before each  
prescription

7. Order the prescription using the [Prescription Authorization Form](#).

---

During treatment

8. Assess the patient's condition for the presence of neutralizing antibodies if a patient experiences severe infections or if you suspect Myalept treatment is no longer working (e.g., loss of glycemic control, increased triglycerides).

---

**2. Patients who are prescribed Myalept:**

---

Before treatment  
initiation

1. Receive counseling from the prescriber on the risks of Myalept.

---

During treatment

2. Be monitored for neutralizing antibodies

---

**3. Outpatient Pharmacies that dispense Myalept must:**

---

To become  
certified to  
dispense

1. Designate an authorized representative to carry out the certification process and oversee implementation and compliance with the REMS Program on behalf of the pharmacy.

2. Train all relevant staff involved in dispensing of Myalept on the REMS Program requirements.

3. Establish processes and procedures to verify that the prescriber is certified and a completed [Prescription Authorization Form](#) is received for each new prescription.

---

Before dispensing

4. Verify that the prescriber is certified and a completed [Prescription Authorization Form](#) is received for each new prescription through the processes and procedures established as a requirement of the REMS Program.

---

At all times

5. Not distribute, transfer, loan, or sell Myalept.

6. Maintain and submit records of prescription data to the REMS program.

7. Maintain records that all REMS processes and procedures are in place and are being followed.

8. Comply with audits carried out by Aegerion or a third party acting on behalf of Aegerion, to ensure that all processes and procedures are in place and are being followed.

---

---

**4. Wholesalers-distributors that distribute Myalept must:**

To be able to distribute	<ol style="list-style-type: none"><li>1. Establish processes and procedures to ensure the drug is distributed only to certified outpatient pharmacies, or to inpatient pharmacies authorized by the REMS Program call center.</li><li>2. Train all relevant staff involved in distributing on the program requirements.</li></ol>
At all times	<ol style="list-style-type: none"><li>3. Distribute only to outpatient certified pharmacies, or to inpatient pharmacies authorized by the REMS Program call center.</li><li>4. Maintain records of drug distribution.</li><li>5. Comply with audits carried out by Aegerion Pharmaceuticals, Inc. or a third party acting on behalf of Aegerion Pharmaceuticals, Inc., to ensure that all processes and procedures are in place and are being followed.</li></ol>

---

**Aegerion Pharmaceuticals, Inc. must provide training to healthcare providers who prescribe Myalept.**

The training includes the following educational material: [Prescriber Training Module](#). The training must be available online or by contacting the REMS Program call center.

**To inform healthcare providers about the REMS Program and the risks and safe use of Myalept, Aegerion Pharmaceuticals, Inc. must disseminate REMS communication materials according to the table below:**

Target Audience	Communication Materials and Dissemination Plans
Healthcare providers who prescribed Myalept within the 12 months prior to approval of the 12/11/2017 REMS modification and newly identified healthcare providers who prescribed or are likely to prescribe Myalept	<p>REMS Letter: <a href="#">REMS Letter for Health Care Providers (Infections)</a></p> <ol style="list-style-type: none"><li>1. Mail within 30 calendar days of the approval of the REMS modification (12/11/2017) with a copy or link to the Prescribing Information.<ol style="list-style-type: none"><li>a. Send by email within 30 calendar days after the initial letter was mailed if the letter is undeliverable.</li><li>b. Send a second email within 30 calendars days of the date the first email was sent if the first email is marked as unopened.</li></ol></li><li>2. Disseminate through field-based sales and medical representatives upon request for 12 months after approval of the 12/11/2017 REMS modification with a copy or link to the Prescribing Information.</li></ol>

**To support REMS Program operations, Aegerion Pharmaceuticals, Inc. must:**

1. Establish and maintain a REMS Program website, [www.MYALEPTREMS.com](http://www.MYALEPTREMS.com). The REMS Program website must include the option to print the Prescribing Information, Medication Guide, and REMS materials. All product websites for consumers and health care providers must include prominent REMS-specific links to the REMS Program website. The REMS Program website must not link back to the promotional product website(s).
  2. Make the REMS Program website fully operational and all REMS materials available through the REMS website or call center.
-

3. Establish and maintain a REMS Program call center for REMS participants at 1-855-6MYALEPT (1-855-669-2537).
4. Establish and maintain a validated, secure database of all REMS participants who are enrolled and/or certified in the Myalept REMS Program.
5. Ensure prescribers are able to enroll in the REMS Program by fax.
6. Ensure outpatient pharmacies are able to certify with the manufacturer and agreeing to comply with the requirements of the REMS Program.
7. Ensure inpatient pharmacies are able to contact the REMS Program call center for assistance in obtaining Myalept for a specific inpatient who has a certified prescriber and a [Prescription Authorization Form](#) on file with a certified pharmacy.
8. Provide the [Prescriber Training Module, Prescriber Enrollment Form, Myalept REMS Program: An Introduction](#), and the Prescribing Information to REMS participants who (1) attempt to prescribe Myalept and are not yet certified or (2) inquire about how to become certified.
9. Provide for 12 months after the approval of the 12/2017 REMS modification the [REMS Letter for Health Care Providers \(Infections\)](#) to REMS participants who (1) attempt to prescribe Myalept and are not yet certified or (2) inquire about how to become certified.
10. Notify prescribers and pharmacies within 48 hours after they become certified in the REMS Program.
11. Provide certified pharmacies access to the database of certified prescribers.

**To ensure REMS participants' compliance with the REMS Program, Aegerion Pharmaceuticals, Inc. must:**

1. Maintain adequate records to demonstrate that REMS requirements have been met, including, but not limited to records of: Myalept distribution and dispensing; certification of prescribers and pharmacies; and audits of REMS participants. These records must be readily available for FDA inspections.
  2. Establish a plan for addressing noncompliance with REMS Program requirements.
  3. Monitor certified prescribers and pharmacies on an ongoing basis to ensure the requirements of the REMS are being met. Take corrective action if non-compliance is identified, including de-certification.
  4. Audit certified pharmacies no later than 180 days after they become certified, to ensure that all REMS processes and procedures are in place, functioning, and support the REMS Program requirements. Thereafter, Aegerion Pharmaceuticals, Inc. must include the certified pharmacies in their annual audit plan.
  5. Take reasonable steps to improve implementation of and compliance with the requirements in the Myalept REMS Program based on monitoring and evaluation of the Myalept REMS Program.
-

## **IV. REMS Assessment Timetable**

Aegerion Pharmaceuticals, Inc. must submit REMS Assessments at 6 months, 12 months, and annually thereafter from the date of the initial approval of the REMS. To facilitate inclusion of as much information as possible while allowing reasonable time to prepare the submission, the reporting interval covered by each assessment should conclude no earlier than 60 calendar days before the submission date for that assessment. Aegerion Pharmaceuticals, Inc. must submit each assessment so that it will be received by the FDA on or before the due date.

## **V. REMS Materials**

The following materials are part of the Myalept REMS:

### **Enrollment Forms**

Prescriber:

1. [Prescriber Enrollment Form](#)

### **Training and Educational Materials**

Prescriber:

2. [Prescriber Training Module](#)
3. [Myalept REMS Program: An Introduction](#)

### **Patient Care Forms**

4. [Prescription Authorization Form](#)

### **Communication Materials**

5. [REMS Letter for Health Care Providers \(Infections\)](#)

### **Other Materials**

6. [REMS Program Website \(www.MYALEPTREMS.com\)](http://www.MYALEPTREMS.com)

**APPENDIX 1**  
**PRESCRIBER TRAINING MODULE**