DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration
Silver Spring MD 20993

NDA 203858/S-001

SUPPLEMENT APPROVAL

Aegerion Pharmaceuticals, Inc.
Attention: Martha J. Carter
Chief Regulatory Officer and Senior Vice President
101 Main Street, Suite 1850
Cambridge, MA 02142

Dear Ms. Carter:

Please refer to your Supplemental New Drug Application (sNDA) dated and received February 7, 2013, submitted under section 505(b) of the Federal Food, Drug, and Cosmetic Act (FDCA) for Juxtapid (lomitapide) capsules 5 mg, 10 mg, and 20 mg.

We acknowledge receipt of your amendments dated July 2 and August 5 and 7, 2013, and your risk evaluation and mitigation strategy (REMS) assessment dated June 19, 2013.

This Prior Approval supplemental new drug application modifies the existing REMS to allow prescribers to complete the REMS-required training and enrollment online.

We have completed our review of this supplemental application, as amended. It is approved, effective on the date of this letter.

RISK EVALUATION AND MITIGATION STRATEGY REQUIREMENTS

The REMS for Juxtapid (lomitapide) was originally approved on December 21, 2012. The REMS consists of elements to assure safe use, an implementation system, and a timetable for submission of assessments of the REMS. Your proposed modification to the REMS allows prescribers to complete the REMS-required training and enrollment online.

The timetable for submission of assessments of the REMS will remain the same as that approved on December 21, 2012.

The revised REMS assessment plan should include, but is not limited to, the following:

1. A survey study to evaluate healthcare providers' knowledge of the risk of hepatotoxicity associated with the use of Juxtapid (lomitapide), the need to monitor liver-related laboratory tests before and during treatment with Juxtapid (lomitapide) as described in product labeling, and prescribers' knowledge that FDA's determination of the safety and

efficacy of Juxtapid (lomitapide) is limited to patients diagnosed with homozygous familial hypercholesterolemia.

a. The target level of healthcare provider knowledge for each educational goal of the REMS

b. If the target levels for healthcare provider knowledge are not met, provide possible causes for the deficiencies and proposed measures to improve knowledge.

2. An assessment of enrollment in the Juxtapid (lomitapide) REMS Program, including the following:

   a. Number of healthcare providers certified during the reporting period and cumulatively.

      i. Prescriber information, including degree, specialty, and practice setting (i.e., type of practice, geographic location)
      ii. Volume of prescriptions for each prescriber and each specialty
      iii. A summary of the method prescribers used to enroll (fax, email, online); and

   b. Number of pharmacies certified during the reporting period and cumulatively.

   c. Number of healthcare providers and pharmacies that had their certification revoked during the reporting period and cumulatively and the reason for the revocation.

3. Metrics regarding Juxtapid (lomitapide) distribution and dispensing to assess pharmacy compliance with the Juxtapid REMS:

   a. The number of Juxtapid (lomitapide) orders shipped to pharmacies during the reporting period and cumulatively, including number of bottles, bottle size, and dosage strength.

   b. Pharmacy compliance with Juxtapid (lomitapide) REMS Program requirements (e.g., shipped to a Juxtapid (lomitapide) REMS certified pharmacy versus a non-certified pharmacy).

   c. The number of prescriptions dispensed for Juxtapid (lomitapide), including quantity of tablets (mean, minimum, maximum) and dosage strength during the reporting period and cumulatively, overall and subset by compliance with the Juxtapid (lomitapide) REMS Program requirements (e.g., received from Juxtapid REMS certified versus non-certified healthcare providers, number of initial prescriptions dispensed without a signed attestation on the Juxtapid Prescription Authorization Form). Dispensing details are to be obtained from the pharmacies.

d. The number and demographics (e.g., gender, age, geographic location) of patients who received Juxtapid (lomitapide) during the reporting period and annually. The number is to be calculated by reconciling orders dispensed to unique patients.

e. Duration of therapy for patients (mean, median, range).

f. Report of number, length, and reasons for shipment delays to patients.

g. Detailed description of root cause of noncompliance with Juxtapid (lomitapide) REMS Program-required dispensing and any corrective and/or preventive actions taken to address noncompliance during the reporting period and cumulatively.

4. Summary of issues and complaints received by Juxtapid (lomitapide) REMS call center; summary of resolution of the issues and complaints.

The requirements for assessments of an approved REMS under section 505-1(g)(3) include, with respect to each goal included in the strategy, an assessment of the extent to which the approved strategy, including each element of the strategy, is meeting the goal or whether 1 or more such goals or such elements should be modified.

In addition to the assessments submitted according to the timetable included in the approved REMS, you must submit a REMS assessment and may propose a modification to the approved REMS when you submit a supplemental application for a new indication for use as described in section 505-1(g)(2)(A) of the FDCA.

If the assessment instruments and methodology for your REMS assessments are not included in the REMS supporting document, or if you propose changes to the submitted assessment instruments or methodology, you should update the REMS supporting document to include specific assessment instrument and methodology information at least 90 days before the assessments will be conducted. Updates to the REMS supporting document may be included in a new document that references previous REMS supporting document submission(s) for unchanged portions. Alternatively, updates may be made by modifying the complete previous REMS supporting document, with all changes marked and highlighted. Prominently identify the submission containing the assessment instruments and methodology with the following wording in bold capital letters at the top of the first page of the submission:

NDA 203858 REMS CORRESPONDENCE
(insert concise description of content in bold capital letters, e.g.,
UPDATE TO REMS SUPPORTING DOCUMENT - ASSESSMENT METHODOLOGY)

An authorized generic drug under this NDA must have an approved REMS prior to marketing. Should you decide to market, sell, or distribute an authorized generic drug under this NDA, contact us to discuss what will be required in the authorized generic drug REMS submission.

Prominently identify the submission containing the REMS assessments or proposed modifications of the REMS with the following wording in bold capital letters at the top of the first page of the submission as appropriate:

NDA 203858 REMS ASSESSMENT

NEW SUPPLEMENT FOR NDA 203858
PROPOSED REMS MODIFICATION

NEW SUPPLEMENT (NEW INDICATION FOR USE)
FOR NDA 203858
REMS ASSESSMENT
PROPOSED REMS MODIFICATION (if included)

If you do not submit electronically, please send 5 copies of REMS-related submissions.

PROMOTIONAL MATERIALS

You may request advisory comments on proposed introductory advertising and promotional labeling. To do so, submit the following, in triplicate, (1) a cover letter requesting advisory comments, (2) the proposed materials in draft or mock-up form with annotated references, and (3) the package insert(s) to:

Food and Drug Administration
Center for Drug Evaluation and Research
Office of Prescription Drug Promotion (OPDP)
5901-B Ammendale Road
Beltsville, MD 20705-1266

You must submit final promotional materials and package insert(s), accompanied by a Form FDA 2253, at the time of initial dissemination or publication (21 CFR 314.81(b)(3)(i)). Form FDA 2253 is available at http://www.fda.gov/opacom/morechoices/fdaforms/cder.html; instructions are provided on page 2 of the form. For more information about submission of promotional materials to the Office of Prescription Drug Promotion (OPDP), see http://www.fda.gov/AboutFDA/CentersOffices/CDER/ucm090142.htm.

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, call Kati Johnson, Regulatory Project Manager, at (301) 796-1234.

Sincerely,

{See appended electronic signature page}

Amy G. Egan, MD, MPH
Deputy Director for Safety
Division of Metabolism and Endocrinology Products
Office of Drug Evaluation II
Center for Drug Evaluation and Research

Enclosure: REMS

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