NDA 214860/S-007

SUPPLEMENT APPROVAL
RELEASE FROM POSTMARKETING REQUIREMENT
NEW POSTMARKETING REQUIREMENT
FULFILLMENT OF POSTMARKETING REQUIREMENTS

Acer Therapeutics Inc.
Attention: Gerald Orehostky
SVP Regulatory Affairs and Quality
1180 Celebration Blvd, Suite 103
Celebration, FL 34747

Dear Gerald Orehostky:

Please refer to your supplemental new drug application (sNDA) dated and received December 12, 2024, and your amendments, submitted pursuant to section 505(b)(2) of the Federal Food, Drug, and Cosmetic Act (FDCA) for Olpruva (sodium phenylbutyrate) for oral suspension.

This Prior Approval sNDA provides for updates to the Olpruva labeling for administration to patients 1 year of age and older who weigh 7 kg or greater, new 0.5 g and 1 g dosage strengths, and updated administration instructions, including for use via gastrostomy tubes.

APPROVAL & LABELING

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

Olpruva (sodium phenylbutyrate) is also approved for use in pediatric patients ages 1 years and older who weigh 7 kg or greater as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). This supplement provides for pediatric labeling text pursuant to the Pediatric Research Equity Act (PREA). This approval is in response to a PREA postmarketing requirement (PMR).

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.[1] Content of labeling must be identical to the enclosed labeling (text for the Prescribing Information, Patient Package Insert, and Instructions for Use), 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*.[2]

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).

CARTON AND CONTAINER LABELING

Submit final printed carton and container labeling that are identical to the enclosed carton and container labeling as soon as they are available, but no more than 30 days after they are printed. Please submit these labeling electronically according to the guidance for industry *Providing Regulatory Submissions in Electronic Format — Certain Human Pharmaceutical Product Applications and Related Submissions Using the eCTD Specifications*. For administrative purposes, designate this submission "**Final Printed Carton and Container Labeling for approved NDA 214860/S-007**." Approval of this submission by FDA is not required before the labeling is used.

RELEASE FROM POSTMARKETING REQUIREMENT

We have received your submissions dated March 27, July 30, and December 12, 2024, reporting on the following postmarketing requirement listed in our December 22, 2022, approval letter:

[1] http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm
[2] 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.

4380-1 Develop dosage strength(s) to accommodate the recommended dosing for pediatric patients who weigh <20 kg and patients who weigh ≥20 kg with a body surface area <1.2 m^2.

The original timetable you submitted on December 12, 2022, states that you will conduct this study according to the following schedule:

Draft Protocol Submission:	03/2023
Final Protocol Submission:	05/2023
Study Completion:	09/2023
Final Report Submission:	10/2023

We have reviewed your submissions and have determined that you are released from the above postmarketing requirement because it is not safe to administer Olpruva oral suspension to pediatric patients <12 months of age and pediatric patients <7 kg because use of the drug product would be unsafe in this pediatric group.

The above postmarketing requirement will be replaced by the new postmarketing requirement as described below:

REQUIRED PEDIATRIC ASSESSMENT

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.

Your deferred pediatric study required under section 505B(a) of the FDCA is a required postmarketing study. This required study is listed below.

4380-4 Develop dosage strength(s) to accommodate pediatric patients 12 months of age and older who weigh between 7 kg to less than 20 kg.

We are waiving the pediatric study requirement for pediatric patients <12 months of age and pediatric patients <7 kg for this application because there is evidence strongly suggesting that the drug product would be unsafe in this pediatric group due to amount of free water required for product administration.

FULFILLMENT OF POSTMARKETING REQUIREMENTS

We have received your submissions dated March 27, 2024, June 10, 2024, and December 12, 2024, reporting on and containing the final reports for the following

postmarketing requirements listed in the December 22, 2022, approval letter and in this sNDA approval letter.

4380-3 Conduct in vitro studies to determine the feasibility of administering Olpruva (sodium phenylbutyrate) for oral suspension through enteral feeding tubes.

4380-4 Develop dosage strength(s) to accommodate pediatric patients 12 months of age and older who weigh between 7 kg to less than 20 kg.

We have reviewed your submission and conclude that the above requirements were fulfilled.

We remind you that there is a postmarketing requirement (PMR 4380-2) listed in the December 22, 2022, approval letter that is still open.

PROMOTIONAL MATERIALS

You may request advisory comments on proposed introductory advertising and promotional labeling. For information about submitting promotional materials, see the final guidance for industry *Providing Regulatory Submissions in Electronic and Non-Electronic Format-Promotional Labeling and Advertising Materials for Human Prescription Drugs.*[3]

You must submit final promotional materials and Prescribing Information, 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 FDA.gov.[4] Information and Instructions for completing the form can be found at FDA.gov.[5]

PATENT LISTING REQUIREMENTS

Pursuant to 21 CFR 314.53(d)(2) and 314.70(f), certain changes to an approved NDA submitted in a supplement require you to submit patent information for listing in the Orange Book upon approval of the supplement. You must submit the patent information required by 21 CFR 314.53(d)(2)(i)(A) through (C) and 314.53(d)(2)(ii)(A) and (C), as applicable, to FDA on Form FDA 3542 within 30 days after the date of approval of the supplement for the patent information to be timely filed (see 21 CFR 314.53(c)(2)(ii)). You also must ensure that any changes to your approved NDA that require the submission of a request to remove patent information from the Orange Book

[3] For the most recent version of a guidance, check the FDA guidance web page at https://www.fda.gov/media/128163/download.
[4] http://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Forms/UCM083570.pdf
[5] http://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Forms/UCM375154.pdf

are submitted to FDA at the time of approval of the supplement pursuant to 21 CFR 314.53(d)(2)(ii)(B) and 314.53(f)(2)(iv).

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 Diego Diaz, Regulatory Project Manager, at (301) 796-7182 or Diego.Diaz@fda.hhs.gov .
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Sincerely,

{See appended electronic signature page}

Yuliya Yasinskaya, M.D.
Deputy Director
Division of Rare Diseases and Medical Genetics (DRDMG)
Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM)
Center for Drug Evaluation and Research

ENCLOSURES:

- Content of Labeling
  - Prescribing Information
  - Patient Package Insert
  - Instructions for Use
- Carton and Container Labeling

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.

/s/

YULIYA I YASINSKAYA
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