18 Dec 2024

Yuliya Yasinskaya, MD
Deputy Director
Division of Rare Diseases and Medical Genetics
Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine
Center for Drug Evaluation and Research
Food and Drug Administration

RE: **NDA 214860: Sequence Number 0127**
**OLPRUVA™ (sodium phenylbutyrate) for oral suspension**
**RESPONSE TO PREA NON-COMPLIANCE LETTER**

Dear Dr. Yasinskaya,

Reference is made to our 505(b)(2) New Drug Application (NDA) for OLPRUVA™ (sodium phenylbutyrate) for oral suspension (hereafter, OLPRUVA) for use as adjunctive therapy in the chronic management of patients with urea cycle disorders involving deficiencies of carbamylphosphate synthetase, ornithine transcarbamylase, or argininosuccinic acid synthetase submitted on 05 Aug 2021 and approved on 22 Dec 2022.

Reference is also made to the Notification of Non-Compliance with PREA dated 14 November 2024 in which FDA notifies Acer Therapeutics Inc. of the failure to meet the postmarketing requirement (PMR) of the Pediatric Equity Act (PREA) for NDA 214860 for not submitting the pediatric assessment for PMR 4380-1.

The purpose of this submission is to inform the Agency that Acer Therapeutics Inc. was delayed in submitting the labeling in parallel to the Final Report to PMR 4380-1 (SQ0089) dated 21 Mar 2024. Upon receiving the Notification of Non-Compliance Letter, further clarification was received 03 Dec 2024 via FDA Email Correspondence confirming that PREA requires the results of all required pediatric studies in the label (whether negative, positive or nonconclusive data); therefore, a labeling or efficacy supplement is required as a condition of fulfillment of a PREA PMR.

Subsequently, the Prior Approval Supplement (PAS) to include the information for the additional unit-dosage strengths (0.5 g and 1 g) and the alternate route of administration via gastrostomy tubes was submitted 12 Dec 2024 (SQ0125). Upon anticipated approval of the PAS (SQ0125), all obligations and requirements set forth under Post Marketing Requirement PMR-4380-1 and the Pediatric Research Equity Act (PREA) have been met.

This submission is certified virus free using Windows Defender Antivirus. Technical questions related to this electronic submission should be directed to Monica Celso at mcelso@zevra.com.

If you have questions or comments, please contact me by FDA-secure email at gorehostky@zevra.com, by phone or facsimile at (321) 250-3699 should you have any questions regarding the enclosed information or require additional information.

Sincerely,

Gerald Orehostky
SVP, Regulatory Affairs and Quality
Acer Therapeutics Inc., a wholly owned subsidiary of Zevra Therapeutics, Inc.
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Celebration, FL 34747