Zevra Therapeutics Transitions to Orsini as the Specialty Pharmacy Provider for OLPRUVA® (sodium phenylbutyrate), a Treatment for Certain Urea Cycle Disorders

June 18, 2024

CELEBRATION, Fla. and ELK GROVE VILLAGE, Ill., June 18, 2024 /PRNewswire/ -- Orsini Specialty Pharmacy (Orsini), and Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra) today announced that Orsini is now the pharmacy partner for OLPRUVA® (sodium phenylbutyrate) for oral suspension. OLPRUVA® is a prescription medicine used along with certain therapies, including changes in diet, for long-term management of certain adult and pediatric patients with urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC) or argininosuccinic acid synthetase (AS). Visit OLPRUVA-Prescribing-Information.pdf (olpruva.com) to view the full Prescribing Information, including Important Safety Information.

UCDs are rare genetic disorders that impair the body's ability to remove excess ammonia. People living with UCDs often suffer from ammonia buildup in the blood, potentially resulting in brain damage and neurocognitive impairments, coma and even death, if untreated. OLPRUVA is a nitrogen scavenger that removes excess ammonia.

"Those living with UCDs can face tremendous long-term challenges including developmental delay and neurocognitive impairment, and we are committed to ensuring that they have access to OLPRUVA," said Joshua Schafer, Zevra's Chief Commercial Officer.

"Orsini is built to serve rare disease communities, and we're excited to partner with Zevra to connect UCD patients with this important treatment option," said Brandon Tom, Orsini's Chief Executive Officer.

About Urea Cycle Disorders

UCDs are a group of rare, genetic disorders that can cause harmful ammonia to build up in the blood, potentially resulting in brain damage and neurocognitive impairments if ammonia levels are not controlled.[i] Any increase in ammonia over time is serious. Therefore, it is important to adhere to any dietary protein restrictions and have alternative medication options to help control ammonia levels.

About OLPRUVA®

OLPRUVA (sodium phenylbutyrate) was approved for the treatment of certain UCDs in December 2022 and is marketed under the brand name, OLPRUVA®. OLPRUVA (sodium phenylbutyrate) for oral suspension is a prescription medicine used along with certain therapies, including changes in diet, for the long-term management of adults and children weighing 44 pounds (20 kg) or greater and with a body surface area (BSA) of 1.2 m^2 or greater, with UCDs, involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). OLPRUVA is not used to treat rapid increase of ammonia in the blood (acute hyperammonemia), which can be life-threatening and requires emergency medical treatment. For more information, please visit www.OLPRUVA.com.

Important Safety Information

Certain medicines may increase the level of ammonia in your blood or cause serious side effects when taken during treatment with OLPRUVA. Tell your doctor about all the medicines you or your child take, especially if you or your child take corticosteroids, valproic acid, haloperidol, and/or probenecid.

OLPRUVA can cause serious side effects, including: 1) nervous system problems (neurotoxicity). Symptoms include sleepiness, tiredness, lightheadedness, vomiting, nausea, headache, confusion, 2) low potassium levels in your blood (hypokalemia) and 3) conditions related to swelling (edema). OLPRUVA contains salt (sodium), which can cause swelling from salt and water retention. Tell your doctor right away if you or your child get any of these symptoms. Your doctor may do certain blood tests to check for side effects during treatment with OLPRUVA. If you have certain medical conditions such as heart, liver or kidney problems, are pregnant/planning to get pregnant or breast-feeding, your doctor will decide if OLPRUVA is right for you.

The most common side effects of OLPRUVA include absent or irregular menstrual periods, decreased appetite, body odor, bad taste or avoiding foods you ate prior to getting sick (taste aversion). These are not all of the possible side effects of OLPRUVA. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

[i] Ah Mew N, et al. Urea cycle disorders overview [updated June 22, 2017]. In: Adam MP, Ardinger HH, Pagon RA, et al, eds.

GeneReviews® [Internet]. University of Washington; 1993-2022. Accessed March 20, 2022.

About Orsini

Providing patients with comprehensive and compassionate care since 1987, Orsini is a leader in rare diseases and gene therapies. Orsini partners with biopharma innovators, healthcare providers and payors to support patients and their families in accessing revolutionary treatments for rare diseases. Through integrated rare disease pharmacy solutions including pharmacy distribution, patient services, clinical management and convenient home infusion services, Orsini simplifies how patients connect to advanced therapies. Orsini's high-touch care model centers on experienced and trained therapy care teams that provide personalized patient care to ensure that *No Patient is Left Behind*™.

Orsini Specialty Pharmacy holds accreditations with the Accreditation Commission for Health Care (ACHC), The Joint Commission, URAC and NABP. Orsini has earned URAC's Rare Disease Pharmacy Center of Excellence Designation and ACHC's Distinction in Rare Diseases and Orphan Drugs. For more information, contact Orsini at 847-734-7373 ext. 505, email us at media@orsinihc.com, or visit www.orsinispecialtypharmacy.com.

About Zevra Therapeutics

Zevra Therapeutics is a rare disease company combining science, data, and patient needs to create transformational therapies for diseases with limited or no treatment options. Our mission is to bring life-changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, the Company is overcoming complex drug development challenges to make new therapies available to the rare disease community.

Expanded access programs are made available by Zevra Therapeutics and its affiliates and are subject to the Company's Expanded Access Program (EAP) policy as published on its website. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the treating physician's discretion.

For more information, please visit www.zevra.com or follow us on X (formerly Twitter) and LinkedIn.

Cautionary Note Concerning Forward-Looking Statements

This press release may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, including without limitation statements regarding the promise and potential impact of our preclinical or clinical trial data, the initiation, timing, design, or results of any clinical trials or readouts, the potential benefits of any of our products or product candidates for any specific disease indication or at any dosage. Forward-looking statements are based on information currently available to Zevra and its current plans or expectations. They are subject to several known and unknown uncertainties, risks, and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the "Risk Factors" section of Zevra's Annual Report on Form 10-K for the year ended December 31, 2023, Zevra's quarterly report for the three months ended March 31, 2024, and Zevra's other filings with the Securities and Exchange Commission. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we cannot assure that such expectations will prove correct. These forward-looking statements should not be relied upon as representing our views as of any date after the date of this press release.

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