RECORDATI RARE DISEASES GROUP

NEWS RELEASE

RECORDATI RARE DISEASES: CARBAGLU® (Carglumic Acid) Tablets 200mg Receives U.S. FDA Approval for a New Indication to Treat Acute Hyperammonemia Associated with Propionic Acidemia and Methylmalonic Acidemia

CARBAGLU is First and Only FDA-Approved Medication for Hyperammonemia Associated with These Rare Conditions

Lebanon, NJ, January 26, 2021 – Recordati Rare Diseases Inc. today announced the U.S. Food and Drug Administration (FDA) has approved a new indication for CARBAGLU® (carglumic acid) tablets 200mg as adjunctive therapy to standard of care for the treatment of acute hyperammonemia due to propionic acidemia (PA) or methylmalonic acidemia (MMA) in pediatric and adult patients.

CARBAGLU is the first and only FDA approved medication for the treatment of acute hyperammonemia due to PA and MMA. CARBAGLU was initially approved by the FDA for N-acetylglutamate synthase (NAGS) deficiency, another rare metabolic disorder, as adjunctive therapy to standard of care for the treatment of acute hyperammonemia due to NAGS deficiency, and maintenance therapy for the treatment of chronic hyperammonemia due to NAGS deficiency.

PA and MMA are rare inherited metabolic disorders that result in the dysfunction of a specific step of amino acid catabolism, or breaking down of certain fatty acids, due to deficient enzyme activity. As a result, toxic metabolites accumulate, which can cause hyperammonemia, a potentially life-threatening condition. CARBAGLU acts as a replacement for N-acetylglutamate (NAG) in NAGS deficiency, PA, and MMA patients by activating carbamoyl phosphate synthetase (CPS 1), improves or restores the function of the urea cycle, and facilitates ammonia detoxification and urea production.

“There are few approved drugs that treat hyperammonemia, and none that are indicated for the treatment of acute hyperammonemia in PA and MMA patients,” said Mendel Tuchman, MD, Medical Geneticist and Professor Emeritus of Pediatrics at The George Washington University School of Medicine and Health Science. “Carbaglu has the potential to impact these patients by reducing high plasma ammonia levels during critical situations.”

FDA approval of the new indication was supported by a randomized, double-blind, placebo-controlled, multicenter clinical trial comparing the effectiveness of CARBAGLU to placebo in the treatment of hyperammonemic episodes in patients with PA or MMA. The efficacy evaluation, based on 90 hyperammonemic episodes occurring in 24 patients, showed that patients receiving CARBAGLU demonstrated a quicker reduction of ammonia compared to patients receiving placebo. The primary endpoint was the time from the first dose to the earlier of blood ammonia level below 50 micromol/L or hospital discharge. Throughout the first three days of treatment, a higher proportion of CARBAGLU-treated episodes reached the primary endpoint compared to placebo-treated episodes.

In the clinical trial, at least 1 adverse reaction was reported in 42.2% of the 90 hyperammonemic episodes that occurred. The most common adverse events were (≥5%) neutropenia, anemia, vomiting, electrolyte imbalance, decreased appetite, hypoglycemia, lethargy/stupor, encephalopathy and pancreatitis/lipase increased.

"People living with these conditions face potentially serious complications from hyperammonemia that, without treatment, can lead to coma or even death," said Andrea Recordati, CEO of Recordati S.p.A.. "We are pleased to receive this new indication for Carbaglu which will enable Recordati to address an unmet medical need in these patients."

About Propionic Acidemia (PA) and Methylmalonic Acidemia (MMA)
Propionic acidemia (PA) and methylmalonic acidemia (MMA) are rare inherited metabolic disorders. PA results from a deficiency of the enzyme propionyl-CoA carboxylase. Most PA patients present with symptoms in the first few days of life.

MMA results from different types of enzyme deficiencies or defects. The most common cause is a deficiency of the enzyme methylmalonyl-CoA mutase. In both disorders, complete lack of enzyme or very limited enzyme causes more severe symptoms.

Hyperammonemia is one of the most severe and life-threatening events that can occur in patients with PA or MMA. Hyperammonemia is a medical emergency that if left untreated, can progress to irreversible brain damage, coma, or death.

Please click here for Full Prescribing Information and Instructions for Use.

Important Safety Information

- Contraindications: None.
- NAGS deficiency: Most common adverse reactions (≥13%) are: vomiting, abdominal pain, pyrexia, tonsillitis, anemia, diarrhea, ear infection, infections, nasopharyngitis, hemoglobin decreased, and headache.
- PA and MMA: Most common adverse reactions (≥5%) are: neutropenia, anemia, vomiting, electrolyte imbalance, decreased appetite, hypoglycemia, lethargy/stupor, encephalopathy and pancreatitis/lipase increased.
- **To report SUSPECTED ADVERSE REACTIONS, contact Recordati Rare Diseases Inc. at 1-888-575-8344, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.**
- If CARBAGLU is administered during pregnancy to women with NAGS deficiency, health care providers should report CARBAGLU exposure to the pregnancy pharmacovigilance program by calling 1-888-575-8344.

About Recordati Rare Diseases Inc.
Recordati Rare Diseases Inc. is a biopharmaceutical company committed to providing often-overlooked orphan therapies to the underserved rare disease communities of the United States. Recordati Rare Diseases is part of the Recordati Group, a public international pharmaceutical company

committed to the research and development of new specialties with a focus on treatments for rare diseases.

Recordati Rare Diseases' mission is to reduce the impact of extremely rare and devastating diseases by providing urgently needed therapies. We work side-by-side with rare disease communities to increase awareness, improve diagnosis and expand availability of treatments for people with rare diseases.

The company's U.S. corporate headquarters is located in Lebanon, NJ, with global headquarter offices located in Milan, Italy.

For a full list of products please click here: http://www.recordatirarediseases.com/us/products.
For additional information, please visit our website: www.recordatirarediseases.com/us.

Recordati, established in 1926, is an international pharmaceutical group, listed on the Italian Stock Exchange (Reuters RECI.MI, Bloomberg REC IM, ISIN IT 0003828271), with a total staff of more than 4,300, dedicated to the research, development, manufacturing and marketing of pharmaceuticals. Headquartered in Milan, Italy, Recordati has operations throughout the whole of Europe, including Russia, Turkey, North Africa, the United States of America, Canada, Mexico, some South American countries, Japan and Australia. An efficient field force of medical representatives promotes a wide range of innovative pharmaceuticals, both proprietary and under license, in several therapeutic areas including a specialized business dedicated to treatments for rare diseases. Recordati is a partner of choice for new product licenses for its territories. Recordati is committed to the research and development of new specialties with a focus on treatments for rare diseases. Consolidated revenue for 2019 was € 1,481.8 million, operating income was € 465.3 million and net income was € 368.9 million.

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Forward Looking Statement
Statements contained in this release, other than historical facts, are "forward-looking statements" (as such term is defined in the Private Securities Litigation Reform Act of 1995). These statements are based on currently available information, on current best estimates, and on assumptions believed to be reasonable. This information, these estimates and assumptions may prove to be incomplete or erroneous, and involve numerous risks and uncertainties, beyond the Company's control. Hence, actual results may differ materially from those expressed or implied by such forward-looking statements. All mentions and descriptions of Recordati products are intended solely as information on the general nature of the company's activities and are not intended to indicate the advisability of administering any product in any particular instance.