ORPHAN EUROPE announces that Health Canada has Granted Market Authorization for Carbaglu® (carglumic acid)

PARIS (France) – September 8th, 2015 – Orphan Europe announces that Carbaglu® (carglumic acid) has been authorized for sale by Health Canada as an adjunctive therapy for the treatment of acute hyperammonemia (high blood level of ammonia) or as a maintenance therapy for chronic hyperammonemia due to the deficiency of the hepatic enzyme N-aceytlglutamate synthase (NAGS) in pediatric and adult patients.

A Notice of Compliance was issued for Carbaglu® (carglumic acid) after a priority review by Health Canada. Carbaglu® (carglumic acid) will be imported and distributed in Canada by Recordati Rare Diseases Inc. c/o GMD Distribution Inc., Ontario. Recordati Rare Diseases and Orphan Europe are wholly owned subsidiaries of Recordati S.p.A. (REC IM, RECI.MI), with Recordati Rare Diseases operating in Canada and the United States.

Carbaglu® is currently marketed in the U.S. by Recordati Rare Diseases for pediatric and adult patients as an adjunctive therapy for the treatment of acute hyperammonemia due to the deficiency of the hepatic enzyme N-acetylglutamate synthase (NAGS deficiency) and as maintenance therapy for chronic hyperammonemia due to NAGS deficiency.

Carbaglu® is currently marketed in Europe (European Medicines Agency – EMA – centralized approval) and many other countries in the world by Orphan Europe for pediatric and adult patients as the treatment of hyperammonemia due to N-acetylglutamate synthase primary deficiency and in the treatment of hyperammonemia due to three organic acidemias (propionic academia, methylmalonic academia, and isovaleric academia).

About Carbaglu® and NAGS deficiency

The biochemical and pharmacological characteristics of Carbaglu® make it a specific treatment for NAGS deficiency. Given orally, Carbaglu® acts as a replacement for N-acetylglutamate (NAG), which is an essential activator of carbamoyl phosphate synthetase (CPS1), the first urea cycle enzyme. Carbaglu stimulates CPS1, triggering the urea cycle and normalising blood ammonia concentration.

It is the only available product authorized for sale by Health Canada as an adjunctive therapy for the treatment of acute hyperammonemia or as a maintenance therapy for chronic hyperammonemia due to the deficiency of the hepatic enzyme N-aceytlglutamate synthase (NAGS) in pediatric and adult patients. During acute hyperammonemic episodes, concomitant administration of Carbaglu® with other ammonia lowering therapies such as alternate pathway medications, hemodialysis, and dietary protein restriction are recommended. During maintenance therapy, the concomitant use of other ammonia lowering therapies and protein restriction may be reduced or discontinued based on plasma ammonia levels.

NAGS deficiency is the rarest of the urea cycle disorders (UCD). UCDs are inherited hepatic metabolic disorders resulting from a deficiency in the liver enzymes involved in the transformation of ammonia into urea. Ammonia is a toxic product of protein catabolism. When present at high levels, ammonia is associated with significant toxicity that can lead to cerebral oedema, irreversible brain damage, coma and eventually death. Hyperammonemia is the major cause of mortality and morbidity in many UCDs, including NAGS deficiency. Prompt use of all therapies necessary to reduce plasma ammonia levels is essential.

About Orphan Europe

Orphan Europe, headquartered near Paris, France, is a pharmaceutical company aiming at providing treatments for patients with unmet medical needs suffering from rare diseases. Being part of Recordati Group since December 2007, it continues to be one of the most active players in the field of rare diseases in Europe. Orphan Europe has been recognized by many leading rare diseases organizations as a leader in their field.

For more information, visit www.orphan-europe.com.

About Recordati Rare Diseases Inc.
Recordati Rare Diseases, headquartered in Lebanon, NJ, is a biopharmaceutical company committed to providing urgently needed therapies to people living with rare diseases in the United States and Canada. As part of Recordati Group, Recordati Rare Diseases strives to reduce the impact of these devastating diseases and restore health. Our experienced team works side-by-side with rare disease communities to increase awareness, improve diagnosis, and expand availability of treatments.

For more information, visit www.recordatirarediseases.com.

About 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 around 4,000, dedicated to the research, development, manufacturing and marketing of pharmaceuticals. Headquartered in Milan, Italy, Recordati has operations in the main European countries, in Russia, in other Central and Eastern European countries, in Turkey, in North Africa and in the United States of America. An efficient field force of medical representatives promotes a wide range of innovative pharmaceuticals, both proprietary and under license, in a number of 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 within the urogenital therapeutic area and of treatments for rare diseases. Consolidated revenue for 2014 was € 987.4 million, operating income was € 231.0 million and net income was € 161.2 million.

For further information:
Recordati website: www.recordati.com

Orphan Europe
Bruno Parenti
+33 (1) 47 73 86 09
Email: BPARENTI@orphan-europe.com

Recordati Rare Diseases
Courtney Johnson
+1 (973) 442-1555 X 123
Email: courtney.johnson@smithsolve.com