

## **RECORDATI: CARBAGLU® RECEIVES ORPHAN DRUG DESIGNATION IN THE U.S. FOR ORGANIC ACIDEMIAS**

*Milan, 3 July 2014* – Recordati announces that the U.S. Food and Drug Administration (FDA) has granted approval of Orphan Europe's request for orphan drug designation for the use of Carbaglu® (carglumic acid) in the treatment of organic acidemias (OAs).

The term organic acidemias (OAs) applies to a group of serious life-threatening disorders characterized by excretion of non-amino organic acids in urine. Most organic acidemias (OAs) result from dysfunction of a specific step in amino acid catabolism usually the result of deficient enzyme activity. Classic organic acidemias (OAs) such as isovaleric acidemia (IVA), propionic acidemia (PA) and methylmalonic acidemia (MMA) are autosomal recessive disorders of branched-chain amino acid metabolism (leucine, isoleucine, valine).

Orphan drug designation applies to drugs that seek to treat rare diseases or conditions affecting fewer than 200,000 patients in the U.S. while providing significant therapeutic advantage over existing therapies. The designation provides the opportunity for seven years marketing exclusivity upon approval for the designated indication, tax credits to offset clinical research expenses, the ability to apply for annual grant funding, and the potential waiver of prescription drug user fees.

Carbaglu® is currently marketed in the U.S. by Recordati Rare Diseases for pediatric and adult patients as the only specific treatment for 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.

"We are very pleased to receive FDA orphan drug designation, which confirms the need for developing novel therapies for organic acidemias (OAs) and is an important step in the further development of Carbaglu® in this indication for patients in the U.S." stated Giovanni Recordati, Chairman and CEO. "This also represents a significant step in the development of our business dedicated to the treatment of rare and orphan diseases."

*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 2013 was € 941.6 million, operating income was € 195.4 million and net income was € 133.7 million.*

For further information:

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