FOR IMMEDIATE RELEASE

Recordati Rare Diseases Inc. Acquires North American Marketing Rights to CYSTADANE® (Betaine Anhydrous for Oral Solution)

LEBANON, N.J. (April 13, 2017)—Recordati Rare Diseases Inc., a biopharmaceutical company committed to providing orphan therapies to underserved rare disease communities in the U.S., today announced the acquisition of North American marketing rights to CYSTADANE® (betaine anhydrous for oral solution). CYSTADANE, the only betaine anhydrous prescription medication approved by the U.S. Food & Drug Administration (FDA), is indicated for the treatment of homocystinuria to decrease elevated homocysteine blood levels.

Homocystinuria is the term used for several rare genetic disorders (diseases passed down through families) that cause levels of homocysteine to build up in blood and urine. This build-up occurs when the body cannot properly process methionine. Methionine and homocysteine are two very important amino acids. Amino acids are the building blocks that the body uses to make proteins. There are multiple forms of homocystinuria, which are distinguished by their signs and symptoms and genetic cause. High levels of homocysteine and abnormal levels of methionine can cause many different symptoms in the body and lead to chronic, serious, and potentially life-threatening health issues.

“Patients suffering from homocystinuria are treated with CYSTADANE to decrease elevated blood levels of homocysteine, as betaine treatment is an important component of disease management,” said Renata Sklodowska, M.D., Director, Medical Affairs, Recordati Rare Diseases Inc. “We are committed to meeting the needs of the homocystinuria community by continuing to supply this treatment. The distribution process will remain exactly the same as before, so there will be no interruptions or changes to patients’ access to this important drug.”

CYSTADANE is a prescription pharmaceutical product that helps to remove homocysteine from the blood by converting excess homocysteine to methionine. CYSTADANE has been evaluated in observational studies without concurrent controls in patients with homocystinuria and in a double-blind, placebo-controlled study, however, no formal statistical analyses were performed. Plasma levels of homocystine decreased by 83-88% regardless of pre-treatment level, and plasma homocysteine decreased by 71-83%, regardless of the pre-treatment level.

In an effort to facilitate patient access to CYSTADANE, Recordati Rare Diseases Inc. offers a **Copay Assistance Program** to help eligible patients with their insurance copayments or co-insurance, and a **Patient Assistance Program** to help eligible uninsured or underinsured patients receive CYSTADANE.

For more information about CYSTADANE or the company's Copay Assistance Program and Patient Assistance Program, visit www.cystadane.com.

CYSTADANE® (betaine anhydrous for oral solution)

Indications and Usage
CYSTADANE (betaine anhydrous for oral solution) is indicated for the treatment of homocystinuria to decrease elevated homocysteine blood levels. Included within the category of homocystinuria are:

- Cystathionine beta-synthase (CBS) deficiency
- 5,10-methylenetetrahydrofolate reductase (MTHFR) deficiency
- Cobalamin cofactor metabolism (cbl) defect

Important Safety Information

- Hypermethioninemia: CYSTADANE may worsen elevated plasma methionine concentrations in patients with CBS deficiency. Cerebral edema has been reported in patients receiving CYSTADANE.
- Monitoring: Monitor plasma methionine concentrations in patients with CBS deficiency. Keep plasma methionine concentrations below 1,000 µmol/L through dietary modification and, if necessary, a reduction of CYSTADANE dose.
- Most common adverse reactions (incidence > 2%) were nausea and gastrointestinal distress, based on physician survey.
- **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.**
- Pregnancy: Animal reproduction studies have not been conducted with CYSTADANE. Use only if clearly needed.
- Nursing women: It is not known whether CYSTADANE is excreted in human milk. Use only if clearly needed.
- Pediatrics: Pediatric patients ranging in age from 24 days to 17 years have been treated with CYSTADANE. Children younger than 3 years of age may benefit from dose titration.

Please see accompanying FULL PRESCRIBING INFORMATION for CYSTADANE.

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 and our sister company, Orphan Europe, are part of the rare disease business within 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.

Recordati Rare Diseases focuses on inborn errors of metabolism and pediatrics. We began marketing products to treat rare diseases in 2013. The U.S. Food and Drug Administration (FDA) has granted four of our products orphan drug designation. For a full list of our products please click here.

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

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