RECORDATI RARE DISEASES GROUP

NEWS RELEASE

Recordati Rare Diseases Announces Availability of CYSTADROPS® (Cysteamine Ophthalmic Solution) 0.37% in the U.S.

Lebanon, NJ, September 15, 2020 – Recordati Rare Diseases Inc., today announced that CYSTADROPS® (cysteamine ophthalmic solution) 0.37% is now available for prescription and distribution across the United States (U.S.). CYSTADROPS is a new, viscous eye drop solution that depletes cystine crystal deposits in the cornea of the eyes of people living with cystinosis. Cystinosis is a rare genetic condition present from birth that leads to the build-up of cystine crystals throughout the body, causing widespread tissue and organ damage and significant impact on the eyes.

CYSTADROPS is available exclusively through Anovo, a specialty pharmacy that focuses on providing medical treatments to people living with rare and chronic diseases. Recordati and Anovo have partnered together to establish patient support services including insurance benefit investigations, educational references, resources for financial aid, and more. Clinicians can complete a prescription form here.

CYSTADROPS is the first and only U.S. Food and Drug Administration (FDA) approved cysteamine eye drop formulation with four times a day dosing. The FDA approval of CYSTADROPS was supported by data from two clinical trials, both in which patients received CYSTADROPS at a median frequency of four times per day. A Phase 3 open-label, randomized, controlled, two-arm multicenter trial, with 15 patients in the CYSTADROPS arm, investigated the reduction in corneal cystine crystal density as assessed by *in vivo* confocal microscopy (IVCM). In the CYSTADROPS arm, the trial showed a 40 percent reduction in the IVCM total score across all corneal layers from baseline to 90 days.

A Phase 1/2a open-label, adaptive dose-response clinical trial of eight cystinosis patients showed that treatment with CYSTADROPS resulted in a 30 percent decrease in IVCM total score that was maintained for the five-year study period.

The safety of CYSTADROPS was evaluated in two clinical trials. The most commonly observed adverse reactions were eye pain (stinging), blurred vision, eye irritation (burning), eye redness, discomfort at instillation site (sticky eyes or sticky eyelids), eye itching, watery eyes, and medicine deposit on the eye lashes or around the eyes.

Please click here for full Prescribing Information and Instructions For Use.

What is CYSTADROPS (cysteamine ophthalmic solution) 0.37%?

CYSTADROPS is a viscous, or thick, cystine-depleting ophthalmic solution indicated for the treatment of corneal cystine crystal deposits in adults and children living with cystinosis. Cystinosis is a complex, rare disease requiring patients and caregivers to manage multiple different medications every day.

CYSTADROPS is the first and only FDA-approved cysteamine eye drop formulation applied four times a day during waking hours. CYSTADROPS can be stored at room temperature for up to seven days after opening.

Indications and Usage

CYSTADROPS (cysteamine ophthalmic solution) 0.37% is a cystine-depleting agent indicated for the treatment of corneal cystine crystal deposits in adults and children with cystinosis.

Important Safety Information

- To minimize the risk of contamination, do not touch the dropper tip to any surface. Keep bottle tightly closed when not in use.
- A condition where the pressure inside the skull increases for unknown reasons has been reported with cysteamine taken by mouth or cysteamine eye drops (used at the same time as cysteamine taken by mouth). This condition went away with the addition of medicine that increases the production of urine.
- Contains the preservative benzalkonium chloride. Contact with soft contact lenses should be avoided. Remove contact lenses prior to application. Lenses may be reinserted 15 minutes following administration.
- The most common side effects are eye pain (stinging), blurred vision, eye irritation (burning), eye redness, discomfort at instillation site (sticky eyes or sticky eyelids), eye itching, watery eyes, medicine deposit on the eye lashes or around the eyes.
- **To report SUSPECTED SIDE EFFECTS, contact Recordati Rare Diseases Inc. at 1-888-575-8344, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.**

For more information, visit www.cystadrops.com.

About Cystinosis

Cystinosis is a rare genetic disorder affecting multiple organs and systems that most frequently begins in infancy. Cystinosis requires patients and caregivers to manage multiple different medications every day. It is estimated that approximately 600 people in the U.S. have cystinosis. Cystinosis is caused by a mutation in the *CTNS* gene that results in impaired transport of the amino acid cystine out of lysosomes in cells. This, in turn, leads to formation and accumulation of cystine crystals in cells, causing damage to organs throughout the body and significant impact on the eyes.

The cornea, or front layer of the eye, is the part of the eye that may be most affected. The first and most frequently reported ocular symptom is photophobia -- sensitivity to light that results in discomfort. It is thought that photophobia is mainly due to the presence of corneal cystine crystals that cause light entering the eye to scatter. As the disease progresses, ocular symptoms increase in number and intensity, daily activities become more difficult to carry out, and severe complications may develop, including visual impairment and potential corneal transplant.

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.

Media Contact:
Elissa Johnsen
EJJ Communications, LLC for Recordati
312-285-3203
ellijohnsen@ejjcommunications.com

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.

###