Recordati Rare Diseases to Present New Data in Cushing's Disease and Cushing's Syndrome at ENDO 2024

FDA Orphan Drug Designation Granted to Osilodrostat for the Treatment of Endogenous Cushing's Syndrome Excluding Cushing's Disease

Bridgewater, NJ, May 16, 2024 – Recordati Rare Diseases Inc. announced today that five poster presentations will be featured, including two late-breaking presentations, from the company's endocrinology portfolio at the Endocrine Society's annual meeting, ENDO 2024, June 1-4, 2024 in Boston.

"We are excited to share our clinical data with the ENDO 2024 community. These findings underscore our commitment to understanding and addressing Cushing's disease and our dedication to pursuing novel treatment options for people who desperately need them," said Mohamed Ladha, President and General Manager, Recordati Rare Diseases, North America. "In line with our commitment to this patient community and following positive interaction with the FDA and the recent confirmation of Orphan Drug Designation, Recordati plans to submit the supplemental New Drug Application (sNDA) during the third quarter of 2024 for the potential label expansion of osilodrostat (Isturisa®) into Cushing's syndrome in the U.S."

Earlier this month, Recordati was granted FDA Orphan Drug Designation for osilodrostat, a cortisol synthesis inhibitor, for the treatment of endogenous Cushing's syndrome excluding Cushing's disease. Osilodrostat, marketed currently as ISTURISA®, maintains its Orphan Drug Designation which was granted September 13, 2013, and its indication for the treatment of adult patients with Cushing's disease for whom pituitary surgery is not an option or has not been curative.

Key Data Presentations

Title: 671 – SAT-671 / SAT-671 – Safety And Effectiveness Of Osilodrostat In Patients With Non-Pituitary Cushing's Syndrome: Results From The Retrospective Observational LINC 7 Study
Session: P106 – Late-Breaking Poster Presentation Day 1
Date/Time: Saturday, June 1, 2024/12:15-1:45pm EDT
Location: ENDOExpo Poster Area – BCEC

Title 670 – SAT-670 / SAT-670 – Effect Of Osilodrostat On Cardiovascular And Metabolic Manifestations Of Hypercortisolism in Patients With Non-Pituitary Cushing's Syndrome: Findings From A Retrospective Observational Study (LINC 7)
Session: P106 – Late-Breaking Poster Presentation Day 1
Date/Time: Saturday, June 1, 2024/12:15-1:45pm EDT Location: ENDOExpo Poster Area – BCEC

Title: 071 – SAT-071 / SAT-071 – Long-Term Effect of Subcutaneous Pasireotide on Clinical and Quality of Life Endpoints in Patients with Cushing's Disease: Results from a Non-Interventional Study
Session: P003 – Neuroendocrinology and Pituitary: Pituitary Tumors I
Date/Time: Saturday, June 1, 2024/12:15-1:45pm EDT
Location: ENDOExpo Poster Area – BCEC

Title: 059 – SUN-059 / SUN-059 – Continued Improvements in Hypertension and Diabetes During Long-Term Osilodrostat Therapy in Patients with Cushing's Disease: A Pooled Analysis for the Phase III LINC 3 and LINC 4 Studies
Session: P036 – Neuroendocrinology and Pituitary: Pituitary Tumors III
Date/time: Sunday, June 2, 2024/12:00-1:30pm EDT
Location: ENDOExpo Poster Area – BCEC

Title: 153 – MON-153 / MON-153 – A Non-interventional, Multinational, Phase IV Study to Evaluate the Long-Term Safety and Efficacy of Osilodrostat in Patients with Endogenous Cushing's Syndrome (LINC6): 1-Year Real-World Interim Analysis
Session: P072 – Neuroendocrinology and Pituitary: Pituitary Tumors IV
Date/Time: Monday, June 3, 2024/12:00-1:30pm EDT
Location: ENDOExpo Poster Area – BCEC

Recordati Rare Diseases is exhibiting at Booth #715.

About Rare Diseases
Rare diseases, designated as conditions occurring in fewer than 200,000 people in the U.S. and 5 per 10,000 people in the European Union, have an extensive impact on public health. Despite their relatively low prevalence, development of safe and effective treatments for rare diseases are increasingly recognized as a global priority.[1]

About Cushing's disease
Cushing's disease is a form of Cushing's syndrome, in which chronically elevated cortisol levels are triggered by a pituitary adenoma secreting excess adrenocorticotropic hormone (ACTH). It is a rare, serious and difficult-to-treat disease that affects approximately one to two patients per million per year. Prolonged exposure to elevated cortisol levels is associated with considerable morbidity, mortality and impaired Quality of Life due to complications and comorbidities.[2, 3]

About Cushing's syndrome
Cushing's syndrome is caused by an inappropriate and chronic exposure to excessive levels of cortisol. The source of this excess of cortisol can be endogenous or exogenous (i.e., medication). Cushing's syndrome affects 40 to 70 people out of every one million living in the U.S.[4] Earlier this month, Recordati was granted FDA Orphan Drug Designation for osilodrostat, a cortisol synthesis inhibitor, for the treatment of endogenous Cushing's syndrome excluding Cushing's disease.

INDICATION(S) AND IMPORTANT SAFETY INFORMATION

What is ISTURISA?

ISTURISA (osilodrostat) is a prescription medicine that is used to treat adults with Cushing's disease:

- who cannot have pituitary surgery, or
- who have had pituitary surgery, but the surgery did not cure their Cushing's disease

It is not known if ISTURISA is safe and effective in children.

IMPORTANT SAFETY INFORMATION

Before starting ISTURISA tell your healthcare provider about all your medical conditions, including if you:

- have or had heart problems, such as an irregular heartbeat, including a condition called prolonged QT syndrome (QT internal prolongation). Your healthcare provider will check the electrical signal of your heart (called an electrocardiogram) before you start taking ISTURISA, 1 week after starting ISTURISA, and as needed after that.
- have a history of low levels of potassium or magnesium in your blood.
- have liver problems.
- are breastfeeding or plan to breastfeed. It is not known if ISTURISA passes into your breast milk. You should not breastfeed if you take ISTURISA and for 1 week after stopping treatment.

Tell your healthcare provider about all the medicines you take, including any prescription and over-the-counter medicines, vitamins, or herbal supplements.

Especially tell your healthcare provider if you take medicines used to treat certain heart problems. Ask your healthcare provider if you are not sure whether your medicine is used to treat heart problems.

ISTURISA can cause serious side effects including:

- Low cortisol levels in your blood (hypocortisolism). Tell your healthcare provider right away if you experience more than one of the following symptoms, as these may be symptoms of very low cortisol level, known as adrenal insufficiency: nausea, vomiting, tiredness (fatigue), low blood pressure, stomach (abdominal) pain, loss of appetite, dizziness.
  If you get symptoms of hypocortisolism while taking ISTURISA, your healthcare provider may change your dose or ask you to stop taking it.
- Heart problem or a heart rhythm problem, such as an irregular heartbeat which could be a sign of a heart problem called QT prolongation. Call your healthcare provider right away if you have irregular heartbeats.
- Increase in other adrenal hormone levels. Your other adrenal hormones may increase when you take ISTURISA. Your healthcare provider may monitor you for the symptoms associated with these hormonal changes while you are taking ISTURISA:

- Low potassium (hypokalemia).
- High blood pressure (hypertension).
- Swelling (edema) in the legs, ankles or other signs of fluid retention.
- Excessive facial or body hair growth (hirsutism).
- Acne (in women).

Call your healthcare provider if you have any of these side effects.

The most common side effects of ISTURISA include very low cortisol levels (adrenal insufficiency), tiredness (fatigue), nausea, headache, and swelling of the legs, ankles or other signs of fluid retention (edema).

These are not all the possible side effects of ISTURISA. Call your healthcare provider for medical advice about side effects. You are encouraged to report side effects of prescription drugs to the FDA. Call 1-800-FDA-1088 or visit www.fda.gov/medwatch.

ISTURISA® (osilodrostat) tablets, for oral use, are available as 1 mg and 5 mg tablets.

Please see the accompanying full Prescribing Information, including Patient Information.

About Recordati Rare Diseases Inc.
Recordati Rare Diseases Inc. is a U.S.-based biopharmaceutical company committed to providing often-overlooked orphan therapies to the underserved rare disease communities. The company's U.S. corporate headquarters is located in Bridgewater, NJ, with global headquarter offices located in Milan.

Recordati Rare Diseases is a part of the Recordati Group, an international pharmaceutical group listed on the Italian Stock Exchange (ISIN IT 0003828271) uniquely structured to bring treatment across specialty and primary care, consumer healthcare, and rare diseases. We believe that health, and the opportunity to live life to the fullest, is a right, not a privilege. We want to support people in unlocking the full potential of their lives. We have fully integrated operations across research & development, chemical and finished product manufacturing through to commercialization and licensing. Established in 1926, Recordati operates in approximately 150 countries across EMEA, Americas and APAC regions. At the end of 2023, Recordati employed over 4,450 people and consolidated revenue of €2,082.3 million. For more information, please visit www.recordati.com.

At Recordati Rare Diseases, we are "focused on the few" with a mission to reduce the impact of rare and devastating diseases by providing urgently needed therapies. Recordati Rare Diseases is focused on providing the most effective treatment for people living with rare diseases and has formed part of the Recordati Group since 2007. https://www.recordatirarediseases.com/us

For a full list of products, please click here: www.recordatirarediseases.com/us/products.

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This document contains forward-looking statements relating to future events and future operating, economic and financial results of the Recordati group. By their nature, forward-looking statements involve risk and uncertainty because they depend on the occurrence of future events and circumstances. Actual results may therefore differ materially from those forecast as a result of a variety of reasons, most of which are beyond the Recordati group's control. The information on the pharmaceutical specialties and other products of the Recordati group contained in this document is intended solely as information on the Recordati group's activities and therefore, as such, it is not intended as medical scientific indication or recommendation, nor as advertising.

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[1] Burton A et al. Drug Discovery and Development in Rare Diseases: Taking a Closer Look at the Tafamidis Story. *Drug Des Devel Ther.* 2021 Mar 18;15:1225-1243. Doi; 10.2147/DDDT.S289772.PMID:33776421; PMCID: PMC7987260. pp 1225-1227.
[2] Feelders RA, Pulgar SJ, Kempel A, Pereira AM. The burden of Cushing's disease: clinical and health-related quality of life aspects. *Eur J Endocrinol.* 2012;167(3):311-326.
[3] Pivonello R, De Leo M, Cozzolino A, Colao A. The treatment of Cushing's disease. *Endocr Rev.* 2015;36(4):385-486.
[4] Lacroix A, Feelders RA, Stratakis CA, Nieman LK. Cushing's syndrome. *Lancet.* 2015;386(9996):913-927.