Recordati Rare Diseases is now the European Marketing Authorization holder of Isturisa® (osilodrostat) indicated for Adult patients with Endogenous Cushing's Syndrome

Paris, 8 April, 2020 – Recordati Rare Diseases today announces the transfer of the European Marketing Authorization of Isturisa® (osilodrostat). The transfer includes all EU member states plus the UK, Norway, Iceland and Liechtenstein.

Isturisa® is authorized for the treatment of adult patients with Cushing's syndrome. Isturisa® is a potent inhibitor of 11β-hydroxylase (CYP11B1), the enzyme responsible for the final step of cortisol biosynthesis[1]. Isturisa® has demonstrated rapid and sustained normalization of cortisol levels in a significant proportion of adult patients with a manageable safety profile, making this a novel oral treatment option for patients with Cushing's syndrome[2, 3]

Cushing's syndrome is an endocrine disorder caused by excessive cortisol, a vital hormone that regulates metabolism, maintains cardiovascular function and helps the body respond to stress. It is a rare but serious disease that most commonly affects adults as young as 20 to 50 years and affects women three times more often than men. It may present with weight gain, central obesity, a round, red full face, severe fatigue and weakness, striae (purple stretch marks), high blood pressure, depression and anxiety. Cushing's syndrome can cause severe illness and death with mortality up to four times higher than in the healthy population[4, 5, 6].

''Isturisa® (osilodrostat) is an important and welcome new treatment option in the management of patients with Cushing's syndrome, a severe, potentially life-threatening rare disease," said Rosario Pivonello, M.D., Professor at the Department of Molecular and Clinical Endocrinology and Oncology of the Frederico II University of Naples, Italy. "Cushing's syndrome results in an increased risk of cardiovascular and cerebrovascular diseases, as well as hypercoagulability, diabetes, infections, depression, and decreased quality of life. If not appropriately treated, Cushing's syndrome has increased mortality. The primary treatment goal is the normalization of cortisol levels. Until now, patients have had few approved options, either with limited efficacy or with too many adverse effects. With this new oral treatment, having shown efficacy and safety in a prospective long-term setting, we have a therapeutic option that will help address patients' needs in this underserved patient population."

In the phase 3 pivotal LINC-3 study, a significantly higher proportion of patients with Cushing's disease treated with Isturisa® maintained normal mean urinary free cortisol (mUFC) at the end of the 8-week randomized withdrawal period (week 34) versus placebo (86% vs 29%). Cortisol level control is the primary objective in the treatment of patients with Cushing's disease. Adverse drug reactions associated with Isturisa® and occurring in greater than 20% of patients are adrenal insufficiency, fatigue, nausea, headache, and edema.[7]

RECORDATI RARE DISEASES

SIEGE SOCIAL
Immeuble « Le Wilson »
70, avenue du Général de Gaulle
92800 Puteaux, France
Tél. : 33 (0)1 47 73 64 58
Fax : 33 (0)1 49 00 18 00

Capital : 320 000.00 Euros
RCS : Nanterre B 379 088 115
Siret : 379 088 115 00058
TVA intracommunautaire : FR
79 379 088 115
www.recordatirarediseases.com

DISTRIBUTION PHARMACEUTIQUE
Eco River Parc
30 rue des Peupliers
92000 Nanterre, France
Tél. : 33 (0)1 47 73 64 58
Fax : 33 (0)1 47 84 69 43

"As a company with a strong commitment to Rare Diseases, we are excited to provide Isturisa®, an effective treatment option to patients with Cushing's Syndrome", said Mr Massimo Mineo, General Manager EMEA. "Rare pituitary disorders, like Cushing's and the unmet needs of patients with this special subset of diseases, are at the heart of our efforts in endocrinology. Today, April 8th, is Cushing's Awareness day. In memory of Harvey Cushing, who first described the syndrome in 1932, we would like to draw attention to the importance of improving awareness and encouraging accurate and early diagnosis of Cushing's syndrome and other rare pituitary diseases.'

The European Commission granted Isturisa® a European Marketing Authorisation as an Orphan Drug. Recordati Rare Diseases expects commercialisation to initiate in Q3 2020.

Recordati Rare Diseases, part of the Recordati group, recently launched an endocrinology business unit and is actively building its commercial, medical, and market access teams. The company is developing a comprehensive distribution model that will support patients and healthcare providers.

1. Bertagna X et al. J Clin Endocrinol Metab 2014;99:1375–83.
2. Fleseriu M et al. Pituitary 2016;19:138–48.
3. Biller BMK et al. Abstract OR16-2. Oral presentation at the Endocrine Society Annual Congress 2019.
4. Nieman LK. Endocrinol Metab 2018;33:139–46.
5. Lonser RR et al. J Neurosurg 2017;126:404
6. Dekkers O et al J Clin Endocrinol Metab, 2013, 98(6):2277-84
7. Isturisa® Summary of Product Characteristics. January 2020.

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 more than 4,300, dedicated to the research, development, manufacturing and marketing of pharmaceuticals. Headquartered in Milan, Italy, Recordati has operations throughout the whole of Europe, including Russia, Turkey, North Africa, the United States of America, Canada, Mexico, some South American countries, Japan and Australia. 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 with a focus on treatments for rare diseases. Consolidated revenue for 2019 was € 1,481.8 million, operating income was € 465.3 million and net income was € 368.9 million.

For further information:
Recordati Rare Diseases website: www.recordatirarediseases.com
Head of International Marketing
Gordon J Daniels
(33)0607531337
e-mail: daniels.g@recordati.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

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