HRA Pharma Receives European CHMP Positive Opinion for Ketoconazole in the Treatment of Endogenous Cushing's Syndrome

Paris, FRANCE, September 26, 2014 - HRA Pharma today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has issued a positive opinion recommending the approval of its Ketoconazole HRA, 200 mg tablets, for patients suffering from endogenous Cushing's syndrome.

Ketoconazole is a steroidogenesis inhibitor that blocks cortisol synthesis. It was originally marketed to treat fungal infections but was withdrawn from the EU market in October 2013 following a referral procedure. Ketoconazole has been found to be effective for treating endogenous Cushing's syndrome from all causes in patients unwilling or unable to have surgery or in whom surgery has failed or while awaiting radiotherapy efficacy.

Ketoconazole HRA was designated as an orphan medicinal product in April 2012 and will be the first ever ketoconazole product registered and labelled for the treatment of endogenous Cushing's syndrome in adults and adolescents above the age of 12 years. Clinical endocrinology guidelines globally recognize ketoconazole as an essential therapy in the management of Cushing's syndrome*.

"Ketoconazole formal approval and registration as a Cushing's syndrome medication will simplify and speed up its supply while improving its proper use with a dedicated labelling for optimal patient management" said Matthieu Leuwers, Endocrinology Director at HRA Pharma. "We believe Cushing's syndrome patients and their healthcare providers will be the immediate beneficiaries of this status change".

"At HRA Pharma, we are strongly committed to offering patients with rare diseases access to the most effective treatments," adds Erin Gainer CEO of HRA Pharma. "With three products in our portfolio essential in the treatment of rare endocrine disorders, we continue to invest to offer adapted solutions for healthcare professionals and patients all around the world".

The EU marketing authorization application for Ketoconazole was reviewed via an accelerated assessment process. The CHMP positive opinion will be now reviewed by the European Commission. If approved, Ketoconazole will be granted a centralized marketing authorization with unified labelling that is valid in 31 European countries including the 28 countries of the European Union, as well as member of the European Economic Area - Iceland, Lichtenstein and Norway. The European Commission usually issues a final legally binding decision within three months of a CHMP opinion. Pending approval, first launches of Ketoconazole HRA Pharma in Europe are expected in first half of 2015.

** Biller BM et al, Treatment of ACTH-Dependent Cushing's Syndrome: A Consensus Statement. Journal of Clinical Endocrinology & Metabolism. 2008;93(7):2454-2462*

About endogenous Cushing's syndrome

Endogenous Cushing's syndrome is a heterogeneous disorder of diverse origins caused by prolonged exposure to inappropriately high levels of the hormone cortisol. Cushing's syndrome is associated with increased mortality and multisystem morbidity. Endogenous Cushing's syndrome is caused by tumors secreting adrenocorticotropin hormone (ACTH) whether from pituitary (Cushing's disease) or extra-pituitary origin (ectopic Cushing's syndrome). Cushing's syndrome is also caused by cortisol-secreting adrenal tumors or hyperplasia.

Estimated maximal prevalence of endogenous Cushing's syndrome is 0,9/ 10 000 and, in the EU, it has an annual incidence of 1/1,400,000-1/400,000, with a peak incidence at 25-40 years of age (Orphanet). Of these, 65-80 % have pituitary dependent Cushing's syndrome, 20-25% have adrenal Cushing's and 5% have ectopic ACTH production from a non-pituitary tumor Definitive therapy of endogenous Cushing's syndrome optimally involves tumor resection. Indications for medical therapy include acutely ill patients, preparation for surgery, those for whom surgery is not indicated, or patients who remain hypercortisolemic postoperatively or while awaiting the effectiveness of radiotherapy.

About HRA Pharma

HRA Pharma is a privately-held European pharmaceutical company that designs products, devices and supporting services in niche areas of health and makes them available to doctors and patients worldwide. The company targets therapeutic gaps in the areas of reproductive health and endocrinology, and uses innovative marketing solutions and socially-conscious programs to promote healthy management of drugs and diseases. Headquartered in Paris, France and with subsidiaries across Western Europe, HRA Pharma has built a strong network of R&D, manufacturing, distribution and NGO partners which enables it to satisfy critical patient needs and improve patient health in over 50 countries across the globe. Visit www.hra-pharma.comfor more information.

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