

# Endocrinology / Rare Diseases

In the endocrinology / rare diseases field, our ambition is to make meaningful health solutions broadly accessible to patients suffering from rare diseases. We reach our goal by incubating emerging ideas, repurposing dormant compounds into actively growing medicines, giving wings to innovative projects through first-in-class development, regulatory, medical and marketing expertise in the field of orphan drugs.

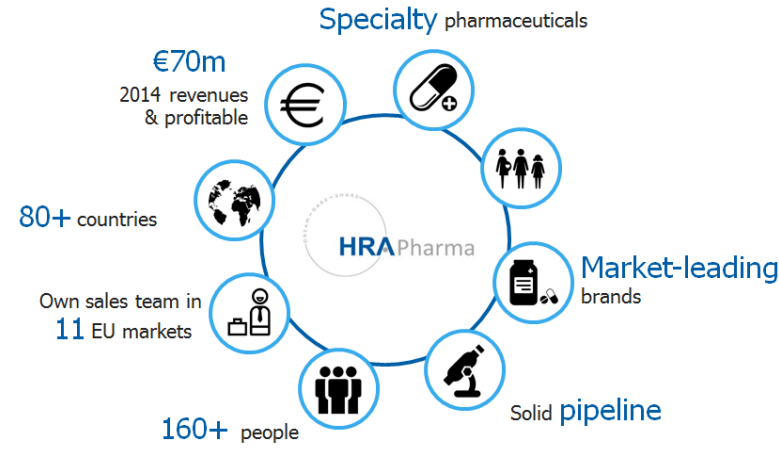
## PRODUCTS

In the field of endocrine and rare diseases, HRA Pharma commercializes Lysodren®, for the symptomatic treatment of advanced adrenal cortical carcinoma. In order to assist healthcare professionals in the treatment of patients with this disease, HRA Pharma has developed LYSSAFE® Service, a free therapeutic monitoring service\* that helps physicians optimize Lysodren® treatment.

Moreover, HRA Pharma portfolio includes two treatments options for endogenous Cushing's syndrome patients:

- Metopirone® (also called Metopiron®, Cormeto®, Metycor™) a steroidogenesis inhibitor that reduces cortisol levels, indicated\*\* for the management of endogenous Cushing's syndrome and/or diagnostic test for ACTH insufficiency and/or diagnostic test for the differential diagnosis of ACTH-dependent Cushing's syndrome depending on the country
- Ketoconazole HRA™, belonging to the same class, indicated for the treatment of adults and adolescents above 12 years old with endogenous Cushing's syndrome. This is the only compound with a dedicated indication in this rare disease

## HRA PHARMA QUICK FACTS



**OUR PRODUCTS**  
 Lysodren® (mitotane)  
 Metopirone® (metyrapone)  
 Ketoconazole HRA™ (ketoconazole)

**LYSSAFE® SERVICE**  
 A unique and free-of-charge\* mitotane plasma level testing

**CURRENTLY TARGETED INDICATIONS**  
 Advanced adrenal cortical carcinoma  
 All causes of Cushing's syndrome

**INDICATIONS UNDER PROSPECTION**  
 Rare tumour  
 Rare endocrine diseases  
 Rare inborn errors of metabolism  
 Rare neurologic diseases  
 Rare renal diseases

**PARTNERING**  
 HRA Pharma is attentive to physicians and patients' needs. We are the ideal go-to-partner for the companies who want to develop, register or market their products in Europe and we are always interested in hearing from partners who wish to manufacture or distribute our own products in their countries.

\* Free of charge transport in EU only  
 \*\*Indications vary by country and not all indications are available in every country