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EffRx Obtains Swiss Marketing Authorization for Alkindi® for Paediatric Adrenocortical Insufficiency

FREIENBACH, Switzerland –-BUSINESSWIRE— EffRx Pharmaceuticals SA, a commercial-stage company that commercializes niche and orphan medicines in Switzerland and Europe, today announced that Swissmedic has approved Alkindi® for the treatment of paediatric adrenocortical insufficiency (AI).

Alkindi®, developed by Diurnal Group plc, is the first preparation of hydrocortisone specifically designed for use in children suffering from paediatric adrenocortical insufficiency (AI). Alkindi® is a patented, oral, immediate-release paediatric formulation of hydrocortisone granules in capsules for opening that allows for accurate age-appropriate dosing in children. This therapeutic approach has the potential to help young patients less than eighteen years of age suffering from paediatric AI and the related condition congenital adrenal hyperplasia (CAH).

Alkindi® is approved and marketed in the European Union and is the first preparation of hydrocortisone specifically designed for use in children suffering from AI. On September 29th, 2020 the US Food and Drug Administration (FDA) has also approved Alkindi® for AI. This new treatment approach is expected to be available on the Swiss market by H1 2022. In Switzerland there are approximately 200 patients suffering from paediatric AI.

The marketing authorisation approval in Switzerland is based upon a pivotal open-label Phase III clinical trial conducted in 24 children, requiring replacement therapy for adrenal insufficiency due to congenital adrenal hyperplasia or hypopituitarism. The study successfully met its primary endpoint and no serious adverse events were reported.

"We are delighted to have received Swissmedic approval for Alkindi®. This approval is a major breakthrough for paediatric patients with adrenal insufficiency, with Alkindi® being the first licensed treatment in Switzerland specifically designed for use in infants, children and adolescents (from birth to less than 18 years old)" commented Lorraine Zakin, Senior Director Medical Affairs at EffRx Pharmaceuticals.

Paediatric AI and the related genetic condition CAH is a condition characterised by deficiency in cortisol, an essential hormone in regulating metabolism and the response to stress. The primary symptoms of AI are chronic fatigue and patients are at risk of adrenal crisis and death if they do not have adequate cortisol replacement. AI is either primary or secondary, with primary AI resulting from diseases intrinsic to the adrenal gland and secondary AI resulting from pituitary diseases where there is a failure of stimulation of the adrenal by the pituitary of the signalling hormone ACTH (adrenocorticotropic hormone).

About EffRx Pharmaceuticals SA

EffRx Pharmaceuticals is a commercial-stage pharmaceutical company focused on the late stage development and commercialization of prescription medications for niche and

orphan indications. The business model is centered around providing superior clinical and commercial value propositions for physicians, payers and patients.

EffRx pro-actively seeks in-licensing opportunities for Europe in niche therapeutic areas, with a primary interest for rare diseases, where one of its pipeline assets has received an orphan drug designation (ODD) from the FDA in US.

About Diurnal Group plc

Founded in 2004, Diurnal is a UK-headquartered, European specialty pharma company developing high quality products for the global market for the life-long treatment of chronic endocrine conditions, including congenital adrenal hyperplasia, adrenal insufficiency and hypogonadism. Its expertise and innovative research activities focus on circadian-based endocrinology to yield novel product candidates in the rare and chronic endocrine disease arena.