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Media and Public Relations

Press release

First paediatric medicine to treat rare hormonal disorder

CHMP gives positive opinion to Alkindi for paediatric-use marketing authorisation

The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended granting a paediatric-use marketing authorisation (PUMA) for Alkindi (hydrocortisone) for the treatment of primary adrenal insufficiency, a rare hormonal disorder, in infants, children and adolescents.

Primary adrenal insufficiency is a condition where the adrenal glands (located just above the kidneys) do not produce enough of a steroid hormone called cortisol (also known as the stress hormone because it is released in response to stress). Symptoms include weight loss, muscle weakness, fatigue, low blood pressure, low blood sugar, disturbances in sodium and potassium balance and sometimes darkening of the skin. Adrenal insufficiency can be life-threatening and usually requires life-long treatment to replace the missing cortisol.

Alkindi is a paediatric-specific formulation of hydrocortisone, a glucocorticoid which has been used routinely as a replacement therapy for the treatment of adrenal insufficiency in adults and children for more than 50 years.

Currently, children are usually treated using crushed tablets of an adult formulation of hydrocortisone prepared by a pharmacist, leading to a risk of under- or over-dosing when parents try to administer the bitter-tasting crushed tablet as best as they can. Alkindi's formulation is meant to allow a more accurate dosing of hydrocortisone in children, with a better masking of the bitter taste, which could be helpful in particular in younger children.

PUMAs can be granted for medicines which are already authorised, but no longer under patent protection, and have been developed specifically for children. Prerequisite for a PUMA is a paediatric investigation plan (PIP) which sets out the development of the medicine in children and has to be approved by the EMA's Paediatric Committee (PDCO). As an incentive to stimulate research of existing products for treatment in children, medicines that have been granted a PUMA benefit from ten years of market protection.

The opinion adopted by the CHMP at its December 2017 meeting is an intermediary step on Alkindi's path to patient access. The CHMP opinion will now be sent to the European Commission for the

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 8427 **Facsimile** +44 (0)20 3660 5555
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Alkindi is Diurnal LTD.
3. More information on paediatric-use marketing authorisations (PUMAs) is available on the Agency's website here.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

Contact our press officers

Tel. +44 (0)20 3660 8427
E-mail: press@ema.europa.eu
Follow us on Twitter @EMA_News