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Press release

Two new paediatric-use marketing authorisations recommended by CHMP

Kigabeq and Slenyto developed specifically for children based on existing medicines

The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended granting two new paediatric-use marketing authorisations (PUMAs), for Kigabeq (vigabatrin) and Slenyto (melatonin).

PUMAs can be granted for authorised medicines which have been developed specifically for children and are no longer under patent protection. PUMAs aim to stimulate research of existing medicines for better treatments for children by giving the medicines ten years of market protection.

Slenyto is to be used for the treatment of insomnia in children from 2 years of age with autism spectrum disorder and Smith-Magenis syndrome (a disorder with a variety of features including intellectual disability, speech and language delay, distinctive facial features, difficulty sleeping and behavioural problems). Sleep disorders are common in children with developmental disabilities and are often difficult to treat. There are currently no approved medicines to treat insomnia in children. However, in practice doctors have been prescribing medicines off-label, including melatonin.

Slenyto has been developed specifically for children and is available in an age-appropriate form, in small tablets. A clinical trial provided scientific data on its efficacy and safety in this population. Its active substance, melatonin, is a naturally-occurring hormone which is normally produced by a gland in the brain called the pineal gland and is involved in coordinating the body's sleep cycle. Data from the clinical trial and from the scientific literature suggest the medicine is associated with a significant increase in total sleep time, a shortened sleep latency (the time it takes to fall asleep after the lights have been turned out) and a longer duration of uninterrupted sleep. The main side effects observed in the clinical trial were somnolence (sleepiness), headache and fatigue.

Kigabeq, the other medicine recommended by the CHMP at this meeting, is meant for the treatment of infantile spasms (West's syndrome), an uncommon and severe form of epilepsy associated with a highly-resistant seizure type (epileptic spasms) and a rapid psychomotor regression, and in resistant partial epilepsy, in infants and children from 1 month to 7 years of age.



Vigabatrin, the active substance in Kigabeq, is an antiepileptic agent that was first authorised in European countries almost thirty years ago and is commonly used to treat adult and paediatric patients, in particular patients with forms of epilepsy which are difficult to manage. Currently vigabatrin is available in the European Union (EU) as 500 mg film-coated tablets or granules for oral solution sachets, which need to be split and/or diluted to make them appropriate for children.

The CHMP recommended a PUMA for a child-friendly formulation of the medicine that is much easier to administer and thus reduces the risk of medication errors. Kigabeq has been developed as 100 mg and 500 mg soluble tablets, with incremental unitary doses of 50 mg, to allow better adjustment of the dose to the patient's body weight. The medicine is to be given as an oral solution and can also be administered via nasogastric tube. The safety profile of the medicine is well known. The most common treatment-related adverse events were visual field defects, marked sedation, as well as agitation and excitation.

The need for paediatric-specific formulations of vigabatrin and melatonin has been identified by EMA's expert committee for medicines for children, the Paediatric Committee (PDCO), in [its inventory of the needs for paediatric medicines](#), which aims to help medicine developers identify opportunities. The medicines were developed according to a paediatric investigation plan (PIP) agreed by the PDCO.

The opinion adopted by the CHMP is an intermediary step on Slenyto and Kigabeq's path to patient access. The CHMP opinions will now be sent to the European Commission for the adoption of decisions on EU-wide marketing authorisations. Once marketing authorisations have 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 the medicines 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 Slenyto is RAD Neurim Pharmaceuticals EEC Ltd.
3. The applicant for Kigabeq is Orphelia Pharma SAS.
4. More information on paediatric-use marketing authorisations (PUMAs) is available on the EMA website [here](#).
5. One medicine containing melatonin, [Circadin](#), is centrally approved in the EU. It is used for the short-term treatment of primary insomnia (poor quality of sleep) in patients aged 55 years or over.
6. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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