EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

31 May 2018
EMA/352612/2018
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Myalepta

metreleptin

On 31 May 2018, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Myalepta, intended for the treatment of complications of leptin deficiency in patients with generalised or partial lipodystrophy which is often associated with severe metabolic abnormalities, including hypertriglyceridaemia, insulin resistance and/or diabetes.

Myalepta was designated as an orphan medicinal product on 17 July 2012. The applicant for this medicinal product is Aegerion Pharmaceuticals B.V.

Myalepta will be available as an 11.3 mg powder for solution for injection. The active substance of Myalepta is metreleptin, a recombinant human leptin analogue (ATC code: A16AA07). Metreleptin mimics the physiological effects of leptin by binding to and activating the human leptin receptor, thus decreasing various types of fat in the body and reducing their accumulation in tissues such as liver and muscle.

The benefits with Myalepta are its ability to reduce the levels of glycated haemoglobin and triglycerides in patients with lipodystrophy. The most common side effects are hypoglycaemia, decrease in weight, injection site reactions and formation of neutralising antibodies.

The full indication is:

“Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients:

- with confirmed congenital generalised LD (*Berardinelli-Seip syndrome*) or acquired generalised LD (*Lawrence syndrome*) in adults and children 2 years of age and above
- with confirmed familial partial LD or acquired partial LD (*Barraquer-Simons syndrome*), in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control.”

It is proposed that Myalepta be prescribed by physicians experienced in the diagnosis and management of metabolic disorders.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5520
Send a question via our website www.ema.europa.eu/contact

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.