EMA/386898/2018
EMEA/H/C/004218

Myalepta (*metreleptin*)

An overview of Myalepta and why it is authorised in the EU

What is Myalepta and what is it used for?

Myalepta is a medicine used in addition to diet to treat lipodystrophy, where patients have loss of fatty tissue under the skin and build-up of fat elsewhere in the body such as in the liver and muscles. The medicine is used in:

- adults and children above the age of 2 years with generalised (throughout the body) lipodystrophy (Berardinelli-Seip syndrome and Lawrence syndrome);
- adults and children above the age of 12 years with partial (localised) lipodystrophy (including Barraquer-Simons syndrome), when standard treatments have failed.

Myalepta contains the active substance metreleptin.

Because the number of patients with the various forms of lipodystrophy is low, the diseases are considered 'rare', and Myalepta was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 July 2012. Further information on the orphan designations can be found on the European Medicines Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation (Bernardinelli-Seip syndrome: 17/07/12; Lawrence syndrome: 17/07/12; familial partial lipodystrophy: 17/07/12; Barraquer-Simons syndrome: 17/07/12).

How is Myalepta used?

Myalepta can only be obtained with a prescription and treatment should be started and monitored by a doctor experienced in the diagnosis and management of patients with metabolic disorders.

Myalepta is given as a daily injection under the skin of the abdomen (belly), thigh or upper arm, at the same time every day. The recommended daily dose depends on the patient's bodyweight and is adjusted based on the patient's response to treatment. Patients or carers can inject the medicine themselves once they have been trained.

For more information about using Myalepta, see the package leaflet or contact your doctor or pharmacist.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

How does Myalepta work?

Patients with lipodystrophy have low levels of a human hormone called leptin, which plays a key role in regulating the break-down of fats and sugars in the body. This leads to loss of fat from under the skin and its build-up in places such as the liver and muscles, as well as high levels of fat in the blood. It also results in insulin resistance (when the body is unable to recognise insulin, a hormone that helps regulate blood sugar levels).

The active substance in Myalepta, metreleptin, is similar to leptin. It replaces leptin and increases fat breakdown in the blood, muscles and liver, thereby correcting some abnormalities in patients with lipodystrophy, including insulin resistance. However, the medicine does not restore fat tissue under the skin.

What benefits of Myalepta have been shown in studies?

Myalepta has been shown to be effective at lowering blood fat levels in 2 main studies involving a total of 107 adults and children with generalised or partial lipodystrophy. In the studies Myalepta was not compared with any other treatment. After 12 months of treatment, blood levels of fats (triglycerides) decreased from around 15 mmol/l to around 5 mmol/l in patients with generalised disease, and from around 16 mmol/l to around 6 mmol/l in patients with partial disease.

Insulin resistance was also improved: blood levels of a substance called glycosylated haemoglobin (HbA1c), which gives an indication of how well the blood glucose is controlled, fell from 8.6% to 6.4% in patients with generalised lipodystrophy, and from 8.8% to 8.0% in patients with partial lipodystrophy.

What are the risks associated with Myalepta?

The most common side effects with Myalepta (which may affect more than 1 in 10 people) are hypoglycaemia (low blood glucose) and weight loss. For the full list of side effects and restrictions with Myalepta, see the package leaflet.

Why is Myalepta authorised in the EU?

Myalepta has been shown to correct some abnormalities caused by leptin deficiency in patients with lipodystrophy, a rare condition for which few treatments are available. Side effects seen with Myalepta are of the kind expected from this type of treatment. The European Medicines Agency therefore decided that Myalepta's benefits are greater than its risks and it can be authorised for use in the EU.

Myalepta has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Myalepta due to the rarity of the condition. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Myalepta?

Since Myalepta has been authorised under exceptional circumstances, the company that markets Myalepta will set-up a registry of patients treated the medicine, and conduct studies to further investigate the benefits and risks of treatment including the possibility for Myalepta to trigger the production of antibodies.

What measures are being taken to ensure the safe and effective use of Myalepta?

The company that markets Myalepta will provide educational materials to patients and doctors with detailed information on how to use the medicine and what to do in case of side effects, including allergic reactions, hypoglycaemia and serious infections.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Myalepta have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Myalepta are continuously monitored. Side effects reported with Myalepta are carefully evaluated and any necessary action taken to protect patients.

Other information about Myalepta

Myalepta received a marketing authorisation valid throughout the EU on 30 July 2018.

Further information on Myalepta can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports.

This overview was last updated in 07-2018.