EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EPAR summaries

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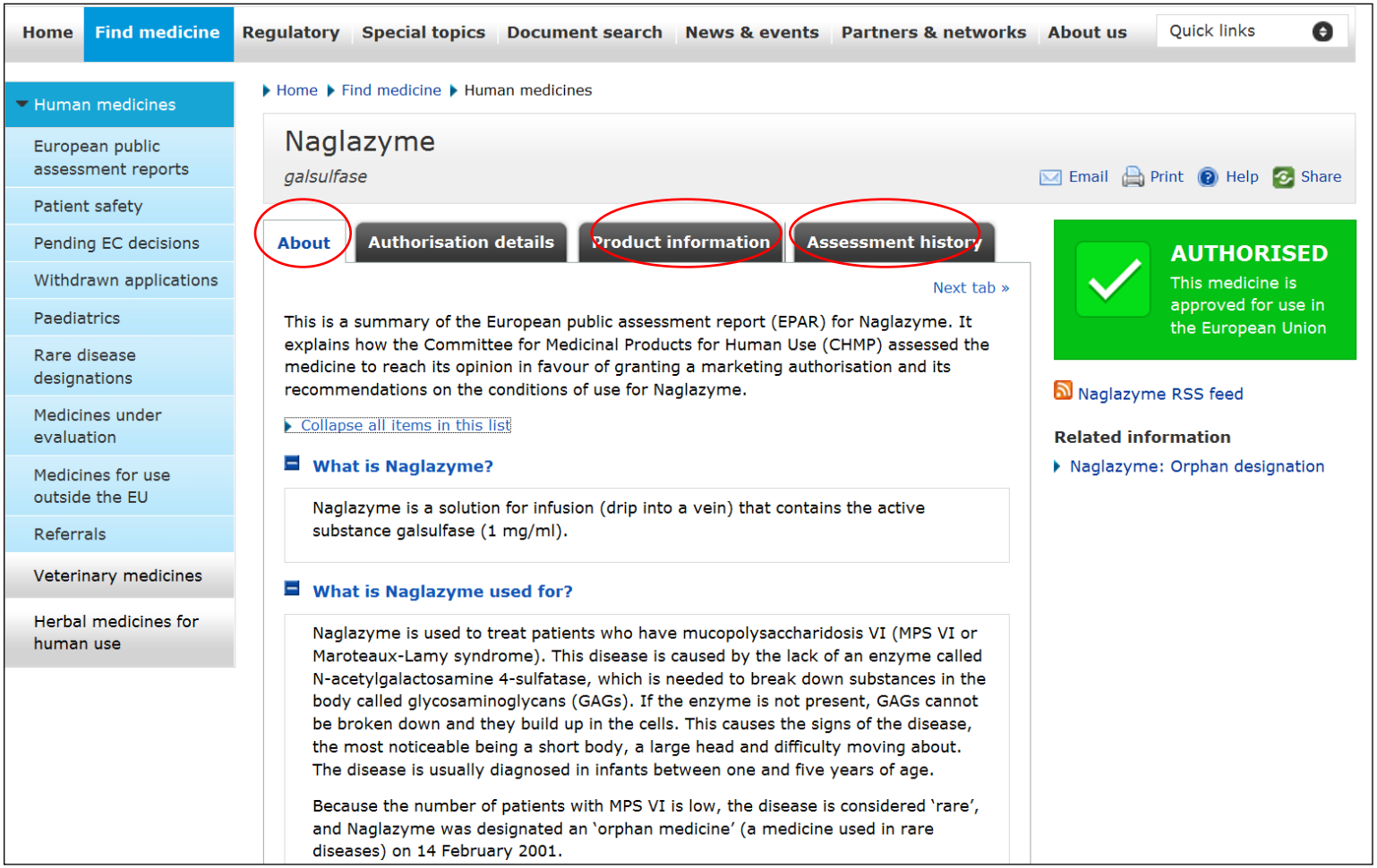
Medical Information sector

An agency of the European Union

Overview

- Legal requirement: public-friendly summary of full EPAR
- Purpose: summarise how CHMP assessed medicine and conditions of use
- Prepared after CHMP opinion, published together with full EPAR
- In all official EU languages
- Kept updated
- Html and pdf versions

Overview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This is a summary of the European public assessment report (EPAR) for Naglazyme. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Naglazyme.
Collapse all items in this list
What is Naglazyme?
Naglazyme is a solution for infusion (drip into a vein) that contains the active substance galsulfase (1 mg/ml).
What is Naglazyme used for?
Naglazyme is used to treat patients who have mucopolysaccharidosis VI (MPS VI or Maroteaux-Lamy syndrome). This disease is caused by the lack of an enzyme called N-acetylgalactosamine 4-sulfatase, which is needed to break down substances in the body called glycosaminoglycans (GAGs). If the enzyme is not present, GAGs cannot be broken down and they build up in the cells. This causes the signs of the disease, the most noticeable being a short body, a large head and difficulty moving about. The disease is usually diagnosed in infants between one and five years of age.
Because the number of patients with MPS VI is low, the disease is considered 'rare', and Naglazyme was designated an 'orphan medicine' (a medicine used in rare diseases) on 14 February 2001.
AUTHORISED
This medicine is approved for use in the European Union
Naglazyme RSS feed
Related information
Naglazyme: Orphan designation

Content

- Based on final assessment report and product information
- Public-friendly language (internal glossary, writing guidance)
- Template based on extensive consultation
- ~ 2 pages long, Q&A format

Content

- What is the medicine and what is it used for?
- How is it used?
- How does it work?
- How has it been studied and what benefits were seen?
- What are the risks?
- Why has it been approved?
- What measures are being taken to ensure its safe and effective use?

Benefit and risk

- Benefit described with studies (primary endpoint results):

What benefit has Jakavi shown during the studies?

Jakavi was more effective than placebo and the best available treatment at reducing the size of the spleen. In the first study, the target reduction in spleen size was achieved in 42% of patients treated with Jakavi (65 out of 155) compared with less than 1% of patients given placebo (1 out of 153). In the second study, the target reduction in spleen size was achieved in 29% of patients treated with Jakavi (41 out of 144) compared with 0% of patients receiving the best available treatment (0 out of 72).

Benefit and risk

- Risk described with side effects + contraindications:

What is the risk associated with Betmiga?

The most common side effects with Betmiga are tachycardia (rapid heartbeat) seen in just over 1 person in 100, and urinary tract infection (infection of the structures that carry urine) seen in just under 3 people in 100.

Serious but uncommon side effects include atrial fibrillation (cardiac rhythm disorder). For the full list of all side effects reported with Betmiga, see the package leaflet.

Betmiga must not be used in people who are hypersensitive (allergic) to mirabegron or any of the other ingredients.

Benefit and risk

- Benefit-risk balance described with reasons for approval:

Why has Jakavi been approved?

The CHMP considered that the reduction in spleen size and symptoms related to myelofibrosis seen in patients taking Jakavi is clinically important. The Committee noted that the quality of life of patients treated with Jakavi was improved but that the medicine's effects had still to be evaluated in terms of extending the life of patients or delaying the progress of the disease or the onset of leukaemia. With regard to safety, the Committee considered that the risk of infections is acceptable but should be monitored further, while other known risks, such as bleeding or a reduction in blood cell counts, can be appropriately managed. The CHMP therefore decided that Jakavi's benefits are greater than its risks and recommended that it be given marketing authorisation.

Benefit and risk

What measures are being taken to ensure the safe and effective use of <X>?

The company that markets NexoBrid must ensure that healthcare professionals in specialised burns centres who are expected to use NexoBrid receive appropriate training and an educational pack including a step-by-step guide to NexoBrid treatment, covering important safety considerations before and after using NexoBrid. The company will also carry out a long-term study in adults and children comparing NexoBrid with standard debridement treatment, to investigate the long-term benefit for patients including cosmetic considerations.

Benefit and risk (new proposal)

What measures are being taken to ensure the safe and effective use of <X>?

A risk management plan has been developed to ensure that <X>is used as safely as possible. Based on this plan, safety information, including the appropriate precautions to be followed by healthcare professionals and patients, has been included in the summary of product characteristics and the package leaflet for <X>.

In addition, the company that markets NexoBrid must ensure that healthcare professionals in specialised burns centres who are expected to use NexoBrid receive appropriate training and an educational pack including a step-by-step guide to NexoBrid treatment, covering important safety considerations before and after using NexoBrid. The company will also carry out a long-term study in adults and children comparing NexoBrid with standard debridement treatment, to investigate the long-term benefit for patients including cosmetic considerations.

Updates

- Summary is updated with any change to EPAR that impacts on published text
- New/extended indication: ⇨ studies section updated to explain benefit
- New safety-related information: ⇨ risks section updated for side effects (most common or serious), contraindications, boxed warnings.
- Change to indication, form/strength, posology, additional risk management measures: ⇨ relevant sections updated
- Re-appraisal of benefit-risk balance (e.g. safety referral resulting in restriction of indication): ⇨ Why has <X> been approved section updated

And finally…

- Attempt to summarise full EPAR in concise document
- High-level overview of benefits, risks and reason for approval
- Living document
- Increased awareness of RMP

- Involvement of patient and consumer organisations:
  - Review of new EPAR summaries (also safety communications)
  - Practical guidance available in training manual:

    http://www.ema.europa.eu/docs/en_GB/document_library/Other/2010/05/WC500090127.pdf

Thank you!