



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EPAR summaries

Introduction to review by patients and consumers

Presented by: Paul Blake
Medical Writer/Stakeholder and Communications Division

An agency of the European Union





What are EPAR summaries?

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Text size: A A A Site-wide search GO

Follow us: [Social media icons]

Home Find medicine Regulatory Special topics Document search News & events Partners & networks About us Quick links

Human medicines
European public assessment reports
Patient safety
Pending EC decisions
Withdrawn applications
Paediatrics
Rare disease designations
Medicines under evaluation
Medicines for use outside the EU
Referrals
Shortages catalogue
Veterinary medicines
Herbal medicines for human use

Defitelio
defibrotide

Email Print Help Share

About Authorisation details Product information Assessment history

This is a summary of the European public assessment report (EPAR) for Defitelio. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Defitelio.

For practical information about using Defitelio, patients should read the package leaflet or contact their doctor or pharmacist.

[Collapse all items in this list](#)

What is Defitelio and what is it used for?

Defitelio is a medicine containing the active substance defibrotide. It is used to treat severe veno-occlusive disease (VOD) in patients undergoing haematopoietic (blood) stem-cell transplantation. VOD is a condition in which the veins in the liver become blocked, leading to liver dysfunction. Defitelio is used in adults and in children from one month of age.

Because the number of patients with VOD is low, the disease is considered 'rare', and Defitelio was designated an 'orphan medicine' (a medicine used in rare diseases) on 29 July 2004.

How is Defitelio used?

Defitelio can only be obtained with a prescription and must be prescribed and given by a doctor experienced in the management of complications of haematopoietic stem cell transplantation. It is available as a concentrate to be made into a solution for infusion (drip) into a vein. Defitelio is given four times a day at a dose of 6.25 mg per kg body-weight. Treatment should last for at least for 3 weeks and continue until the patient's symptoms resolve.

AUTHORISED
This medicine is approved for use in the European Union

Defitelio RSS feed

News

Meeting highlights from the Committee for Medicinal Products for Human Use (CHMP) 22-25 July 2013 (26/07/2013)

Related information

Defitelio: Orphan designation

- Every centrally authorised medicine has its own page on the EMA website.
- The EPAR summary is the first thing you see when you look up a medicine: <http://www.ema.europa.eu/ema/>



What are EPAR summaries?

- Each centrally authorised medicine has an EPAR, including:
 - product information
 - Assessment Report by the Committee for Medicinal Products for Human Use (CHMP)
- Summary is a short public-friendly document based on these, explaining the medicine and how it came to be approved
- Required by EU law (but the form isn't specified by legislation)
- Not a replacement for the product information (which includes SmPC and patient leaflet)
- A living document, which is kept updated throughout the lifecycle of the medicine



Content of EPAR summaries

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



How EPAR summaries are prepared

- Documents
 - Product information
 - Adopted CHMP Assessment Reports
 - Internal style guide
 - Glossary of medical terms

1. NAME OF PRODUCT
Ruconest 2100 U/ml

2. QUALITATIVE AND QUANTITATIVE INFORMATION
One vial contains 2100 IU of conestat alfa for reconstitution, or a 100 mg vial contains 100 mg of conestat alfa for reconstitution.
Conestat alfa is the recombinant DNA derived protein.
1 Unit of conestat alfa is defined as 1 ml of pooled normal human plasma containing 1 IU of conestat alfa.
For a full list of excipients, see Annex 1.

3. PHARMACOLOGICAL PROPERTIES
Powder for solution
White to off-white powder

4. CLINICAL PARTICULARS
4.1 Therapeutic Indications
Ruconest is indicated for the treatment of congenital (HAE) due to C1 esterase inhibitor deficiency.
4.2 Posology and Method of Administration
Ruconest should be used for the treatment of acute attacks of HAE. Ruconest should be used for the treatment of chronic HAE.
Patients who have antibodies against rC1-INH should be treated with Ruconest.
Posology
- Adults up to 84 kg
One intravenous injection of 100 mg (1 vial) of Ruconest.
- Adults of 84 kg
One intravenous injection of 200 mg (2 vials) of Ruconest.
In the majority of cases, the treatment should be administered (see section 4.1).
Not more than two injections per day.
Dose calculation
Determine the patient's weight.



EUROPEAN MEDICINES AGENCY
SCIENCE · MEDICINES · HEALTH

24 June 2010
EMA/CHMP/450953/2010
Evaluation of Medicines for Human Use

CHMP assessment report

Ruconest

International Nonproprietary Name: conestat alfa

Procedure No. EMEA/H/C/001223

Assessment Report as adopted by the CHMP with all information of a commercially confidential nature deleted.

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
Telephone +44 (0)20 7418 8400 Facsimile +44 (0)20 7523 7455
E-mail info@ema.europa.eu Website www.ema.europa.eu

An Agency of the European Union 

European Medicines Agency, 2010. Reproduction is authorised provided the source is acknowledged.



The review process

- Medical writers
- EMA product team
- Patient and consumer organisations
- Rapporteur and CoRapporteur (assessors)
- Company



Why the review by patient and consumers?

- Patient/consumer perspective
- Patients and public concerns
- No source documents (check that it stands alone)
- Appropriate use of language
- Quality check



Things to look out for

- Complicated/oversimplified language
- Unexplained scientific terms
- Inappropriate explanations
- Unnecessary/missing information
- Confusing numbers
- Do you understand the main benefits?
- Do you understand basis for approval?



Comments from patients and consumers

- All comments are considered
- Write what you think/feel
- Comments can be in any form:
 - General or specific
 - Text changes (tracked)
 - Suggestions
 - Questions



Impact of review

- Around half of comments estimated to lead to text changes (not less than other reviews)
- Many implemented with modifications
- Some may not be implemented immediately but are used for changing templates and standard definitions



Responses to feedback from patients and consumers

What is the risk associated with Halaven?

The most common side effects with Halaven (seen in more than 1 patient in 10) are neutropenia (low levels of neutrophils, a type of white blood cell that fights infection), leucopenia (low white blood cell counts), anaemia (low red blood cell counts), reduced appetite, peripheral neuropathy (damage to the nerves in the extremities), headache, nausea (feeling sick), constipation, diarrhoea, vomiting, alopecia (hair loss), muscle and joint pain, fatigue (tiredness) and pyrexia (fever). For the full list of all side effects reported with Halaven, see the package leaflet.

Halaven should not be used in people who may be hypersensitive (allergic) to eribulin or any of the other ingredients. It must not be used in women who are breastfeeding.

Comment [PE6]: What would be the effect on the patient? Pain?, tingling, numbness? I think that cn be added to make it more clear to the patient what it means.

Comment [NM(7)]: More explanation added: causing numbness, tingling and prickling sensations



Responses to feedback from patients and consumers

What is the risk associated with Halaven?

The most common side effects with Halaven (seen in more than 1 patient in 10) are neutropenia (low levels of neutrophils, a type of white blood cell that fights infection), leucopenia (low white blood cell counts), anaemia (low red blood cell counts), reduced appetite, peripheral neuropathy (damage to the nerves in the extremities causing numbness, tingling and prickling sensations), headache, nausea (feeling sick), constipation, diarrhoea, vomiting, alopecia (hair loss), muscle and joint pain, fatigue (tiredness) and pyrexia (fever). For the full list of all side effects reported with Halaven, see the package leaflet.

Halaven should not be used in people who may be hypersensitive (allergic) to eribulin or any of the other ingredients. It must not be used in women who are breastfeeding.



Responses to feedback from patients and consumers

■ What benefits of Harvoni have been shown in studies?¶

Harvoni was investigated in three main studies involving a total of 1,952 patients infected with hepatitis C of genotype 1. In all three studies, the main measure of effectiveness was the number of patients whose blood tests did not show any sign of hepatitis C virus 12 weeks after the end of treatment.¶

In these studies, patients were given Harvoni, with or without ribavirin. After 8 or 12 weeks, around 94% to up to 98% of patients given Harvoni alone tested negative for the virus. The addition of ribavirin or extending treatment for more than 8 weeks was not needed for most patients. In one study, 97% of patients with liver damage achieved a (negative test result) after 24 weeks of treatment.¶

Results of the studies also showed that patients whose virus infection was resistant to other antiviral medicines, ~~achieve better results could be better controlled~~ by extending treatment to 24 weeks.¶

Preliminary results from additional studies showed that Harvoni is also effective in patients with genotype 3 and 4, and that Harvoni in combination with ribavirin could be of benefit for patients with decompensated cirrhosis (scarring of the liver with reduced liver function) or for those who had received a liver transplant.¶

Comment [SS5]: "negative test result" may be misinterpreted. "tested negative for the virus" or something in that direction would be better.¶

Comment [RGQ6]: Taken. This section is now simplified and reworded.¶



Responses to feedback from patients and consumers

• **What benefits of Harvoni have been shown in studies?**

Harvoni was investigated in three main studies involving a total of around 2,000 patients infected with hepatitis C of genotype 1 who did not have failure of liver function. In all three studies, the main measure of effectiveness was the number of patients whose blood tests did not show any sign of hepatitis C virus 12 weeks after the end of treatment.

In these studies, patients were given Harvoni, with or without ribavirin, for 8, 12 or 24 weeks, depending on the characteristics of the patients. Around 94% to up to 99% of patients given Harvoni alone tested negative for the virus 12 weeks after the end of treatment. The addition of ribavirin was not needed for most patients.

Results of the studies also showed that patients who have compensated cirrhosis (scarring of the liver but who maintained liver function) had a higher likelihood of clearing the virus when treatment was extended to 24 weeks. Patients whose infection was resistant to other antiviral medicines could also benefit from extending treatment to 24 weeks.

Supportive data showed that Harvoni in combination with ribavirin would be of benefit for some patients with genotype 3 virus, as well as for patients with genotype 1 or 4 and decompensated cirrhosis (scarring of the liver with reduced liver function) and/or for those who had received a liver transplant.



Responses to feedback from patients and consumers

• **What is Mekinist and what is it used for?**

Mekinist is a cancer medicine that contains the active substance trametinib. It is used to treat adults with melanoma (a type of skin cancer) that has spread to other parts of the body or cannot be surgically removed. Mekinist is only for patients whose melanoma cells have been tested and shown to have a specific mutation (change) called 'BRAF-V600' in their genes.

• **How is Mekinist used?**

Treatment with Mekinist must be started and supervised by a doctor experienced in the use of cancer medicines. The medicine can only be obtained with a prescription.

Mekinist is available as tablets (0.5 mg, 1 mg and 2 mg). It is given at a recommended dose of 2 mg once a day, at a similar time every day. It should be taken without food, at least 1 hour before or 2 hours after a meal. Treatment may need to be interrupted or stopped, or the dose reduced, if the patient experiences certain side effects. For further information, see the summary of product characteristics (also part of the EPAR).

• **How does Mekinist work?**

The active substance in Mekinist, trametinib, works by blocking proteins known as MEK, which are involved in stimulating cell division. In melanomas with the BRAF-V600 mutation, an abnormal form of

Comment [R51]: This information here tells nothing. I realize that in the 3rd part is about how it works. So my suggestion is to:
1. Throw away this information from here and make: Mekinist is a cancer medicine used to treat adults with melanoma..... and left next parts as they are
OR
2. Leave this information, but the part "How does Mekinist work?" should be the next one -- it would have explained why we use trametinib at the very beginning. So the part "How is Mekinist used" should be the 3rd.

Comment [AI2]: This is the wording we have agreed and used for most EPAR summaries. We may not be able to change how but the comment will be considered when revising the EPAR summary template.

Comment [PB3R2]: template revised after internal discussion



Responses to feedback from patients and consumers

What is Mekinist and what is it used for?

Mekinist is a cancer medicine used to treat adults with melanoma (a type of skin cancer) that has spread to other parts of the body or cannot be surgically removed. Mekinist is only for patients whose melanoma cells have been tested and shown to have a specific mutation (change) in their genes called 'BRAF V600'.

Mekinist contains the active substance trametinib.



Responses to feedback from patients and consumers

What is Jevtana used for?

Jevtana is used to treat men with hormone refractory metastatic prostate cancer. This is cancer that affects the prostate gland, the gland below the bladder in men that produces the liquid in semen. Jevtana is used when the cancer has spread to other parts of the body (metastatic) and does not respond to hormonal treatment (hormone refractory). It is used in combination with prednisone or prednisolone (anti-inflammatory medicines) in patients who have previously been treated with docetaxel (another anticancer medicine).

The medicine can only be obtained with a prescription.

Comment [PE1]: And do not react to docetaxel anymore

Comment [DG2R1]: It could be for other reasons such as that the side effects were too severe, so this has not been implemented to avoid too much detail.



Conclusion

- Patients & consumers review an essential part of the process
- Aims include checking clarity, use of language
- Reviewers from patient & consumer organisations provide unique perspectives that improve the final document