swissmedic

Public Summary SwissPAR dated 13 April 2021

# Defitelio® (active substance: defibrotide)

First authorisation in Switzerland: 21 September 2020

Medicinal product (concentrate to make a solution for infusion) for the treatment of severe veno-occlusive disease of the liver

## About the medicine

The medicinal product Defitelio, with the active substance defibrotide, is a concentrate used to make a solution for infusion.

It was authorised for the treatment of a severe veno-occlusive disease of the liver that is also known as sinusoidal obstruction syndrome (impaired circulation in the small veins of the liver). It may be used in patients who are at least one month old.

This veno-occlusive disease affects the liver and is a life-threatening complication of treatments given to prepare the body of patients about to undergo blood stem cell transplantation. This complication can cause the failure of various vital organs and has a fatal outcome in 20 to 30 per cent of patients. The risk is greater in children.

Since this is a rare disease, the medicine has been authorised as an orphan drug. The term "orphan drug" refers to important medicines for rare diseases that meet specific requirements. Medicinal products of this kind benefit from simplified authorisation conditions in Switzerland.

## Information on authorisation

In deciding whether to authorise the medicinal product Defitelio with the active substance defibrotide, Swissmedic took into account the assessments of the Canadian health authority (Health Canada), the European Medicines Agency (EMA) and the US health authority (FDA). The assessment of the clinical data was based on the assessment reports issued by the EMA and the FDA and the corresponding product information texts.

Swissmedic authorised Defitelio in Switzerland on 21 September 2020.

For further information on this authorisation application, Swissmedic would refer readers to the authorisation of the foreign comparator medicinal product.

Since the assessment of the clinical data was based on the assessment reports of the foreign partner authorities, the preconditions for a SwissPAR (Swiss Public Assessment Report) and a resulting Public Summary SwissPAR are not fully met. Swissmedic refers to the authorisation of the foreign comparator product.
www.hc-sc.gc.ca; www.ema.europa.eu; www.fda.gov

## Further information on the medicinal product

Information for healthcare professionals:
Information for healthcare professionals Defitelio®

Healthcare professionals (doctors, pharmacists and others) can answer any further questions.

This information is correct as at the date above. New information concerning the authorised medicinal product in question will not be incorporated into the Public Summary SwissPAR.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.