Drugs

PEER-REVIEWED FEATURE

Valoctocogene Roxaparvovec: Adis Evaluation

Key Points

- Gene therapy being developed by BioMarin Pharmaceutical Inc. for the treatment of haemophilia A
- Received its first approval on 24 August 2022 in the EU
- Approved for the treatment of severe haemophilia A (congenital factor VIII deficiency) in adult patients without a history of factor VIII inhibitors and without detectable antibodies to AAV5

Summary

Valoctocogene roxaparvovec (ROCTAVIAN™) is a gene therapy being developed by BioMarin Pharmaceutical Inc. for the treatment of haemophilia A.

In August 2022, valoctocogene roxaparvovec was granted conditional marketing authorization in the EU for the treatment of severe haemophilia A (congenital factor VIII deficiency) in adults without a history of factor VIII inhibitors and without detectable antibodies to adeno-associated virus serotype 5 (AAV5).

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