30 May 2024
EMA/237295/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Adzynma

rADAMTS13

On 30 May 2024, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Adzynma[3], indicated for the treatment of ADAMTS13 deficiency in patients with congenital thrombotic thrombocytopenic purpura (cTTP).

The applicant for this medicinal product is Takeda Manufacturing Austria AG.

Adzynma will be available as 500 and 1500 IU powder and solvent for solution for injection. The active substance of Adzynma is recombinant ADAMTS13 (rADAMTS13), an antithrombotic enzyme (ATC code: B01AD13). The lack or a severe reduction of the Von Willebrand Factor (VWF) cleaving metalloprotease ADAMTS13 is associated with thrombotic thrombocytopenic purpura (TTP). rADAMTS13 is expected to reduce or eliminate the spontaneous formation of VWF-platelet microthrombi, responsible for platelet consumption and thrombocytopenia in patients with cTTP.

Compared to plasma-based therapies, the benefit of Adzynma is the reduction of acute and subacute thrombotic thrombocytopenic purpura (TTP) events (such as thrombocytopenia, microangiopathic haemolytic anaemia) and TTP manifestations (organ-specific signs and symptoms including neurological symptoms, renal dysfunction and abdominal pain), and the incidence of supplemental doses prompted by subacute TTP events. The most common side effects with Adzynma are upper respiratory tract infection, thrombocytosis, headache, dizziness, migraine, somnolence, diarrhoea, nausea, constipation, abdominal distension, asthenia, feeling hot and abnormal ADAMTS13 activity.

The full indication is:

> ADZYNMA is an enzyme replacement therapy (ERT) indicated for the treatment of ADAMTS13 deficiency in children and adult patients with congenital thrombotic thrombocytopenic purpura

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion.
[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.
[3] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

(cTTP). ADZYNMA can be used for all age groups.

Adzynma should be prescribed by physicians experienced in the treatment of haematological disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.