25 February 2021
EMA/CHMP/19316/2021
Committee for Medicinal Products for Human Use (CHMP)

**Summary of opinion[1] (initial authorisation)**

# Evrysdi

risdiplam

On 25 February 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Evrysdi[2], intended for the treatment of spinal muscular atrophy (SMA). Evrysdi was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is Roche Registration GmbH.

Evrysdi will be available as 0.75 mg/ml powder for oral solution. The active substance of Evrysdi, risdiplam, an *SMN2* mRNA splicing modifier, is an orally administered small molecule with systemic distribution (ATC code: M09AX10).

The benefits with Evrysdi are its ability to alter the clinical course of the disease, allowing patients to achieve developmental milestones which would have been otherwise impossible to achieve. The most common side effects are diarrhoea, rash and headache.

The full indication is:

Evrysdi is indicated for the treatment of 5q spinal muscular atrophy (SMA) in patients 2 months of age and older, with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four *SMN2* copies.

Treatment with Evrysdi should be initiated by a physician with experience in the management of SMA.

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.

[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] This product was designated as orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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