



26 March 2020
EMA/106773/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (initial authorisation)

Zolgensma

onasemnogene abeparvovec

On 26 March 2020, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Zolgensma², intended for the treatment of babies and young children who have a rare, serious inherited condition called spinal muscular atrophy (SMA).

As Zolgensma is an advanced therapy medicinal product, the CHMP positive opinion is based on an assessment by the Committee for Advanced Therapies. The applicant for this medicinal product is AveXis EU Limited.

Zolgensma will be available as a solution for infusion (2×10^{13} vector genomes/ml). Zolgensma is a type of medicine called a 'gene therapy'. It contains the active ingredient onasemnogene abeparvovec which contains human genetic material.

Patients with SMA lack a functioning gene needed to make a protein essential for nerves that control muscles (Survival Motor Neuron or SMN). Zolgensma supplies a fully functioning copy of the human SMN gene enabling the body to produce enough SMN protein. The gene is delivered into the cells where it is needed using a modified virus that does not cause disease in humans. The most common side effects are increases in liver enzymes (transaminases) seen in blood tests.

The full indication is:

Zolgensma is indicated for the treatment of:

- patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or
- patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene.

Zolgensma is for single treatment only and should be administered in suitable clinical centres under the supervision of a physician experienced in the management of patients with SMA.

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



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.