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Answer given by Ms Kyriakides
on behalf of the European Commission
(4.5.2021)

The conditional marketing authorisation granted to Zolgensma has the following therapeutic indications:

- treatment of patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the survival motor neural 1 (SMN1) gene and a clinical diagnosis of SMA Type 1, or
- treatment of patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to three copies of the survival motor neural 2 (SMN2) gene.

While the authorised indications do not include an age restriction, the summary of product characteristics explains that there is limited experience in patients of two years of age and older or with a body weight above 13.5 kg and that the safety and efficacy of onasemnogene abeparvovec in these patients have not been established.

The authorised indications are valid in all Member States. However, it is a national competence to decide the conditions under which a medicinal product that has been granted a marketing authorisation can be reimbursed by the public health budget.

The Commission is not informed of the reimbursements decisions that are taken by the Member States and it does not have the competence to intervene.