Novartis receives EMA CHMP positive opinion for Itvisma, gene therapy for children two years and older, teens, and adults with spinal muscular atrophy (SMA)

24 April 2026

SUMMARY: On 23 April 2026, the European Medicines Agency (EMA) Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion for Itvisma (intrathecal formulation of onasemnogene abeparvovec, developed by Novartis) for the treatment of adults and children aged 2 years and older living with spinal muscular atrophy (SMA).

As **Thomas Doktor, the Co-Chair of the Treatment Committee of SMA Europe** explains, **Itvisma is a gene therapy based on the same active ingredient as Zolgensma but is administered via a single intrathecal injection into the cerebrospinal fluid surrounding the spinal cord, rather than intravenously.**

This direct administration enables delivery to motor neurons at a lower vector dose. Its formulation is more concentrated and delivered in a smaller volume, and it is administered independently of patient weight. This has supported the study of this treatment in individuals older than two years of age and, subsequently, the adoption of a positive CHMP opinion for its use in older children, adolescents, and adults.

> **Yasemin Erbas, President of SMA Europe, states:**
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> 'This is a meaningful development for people living with SMA and their families. We have waited a long time for this treatment option, made possible through years of work across the patient, research, and healthcare communities.'

Next Steps

The CHMP's positive opinion for Itvisma will now be reviewed by the European Commission with a final decision expected in upcoming months.

> **Nicole Gusset, CEO of SMA Europe emphasises:**
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> 'With the European Commission decision expected soon, our focus now is on practical readiness and transparent communication. SMA Europe will work with clinicians and other stakeholders to help the community understand what this potential option could mean in practice, including eligibility considerations, delivery in specialist centers, and follow-up, so that health systems are prepared and patients and families receive clear, timely information.'

> **Yasemin Erbas, President of SMA Europe affirms:**
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> 'This is only the beginning: access will depend on national reimbursement decisions, which may take time, and member countries outside the EMA regulatory framework may face further uncertainty. SMA Europe remains committed to advocating for timely and equitable access to treatments across all regions, ensuring that no one is left behind.'

SMA Europe is working closely with Novartis and will share updates as soon as more information becomes available, as patient safety and health remain the top priority.

In addition, SMA Europe is considering the organisation of an informational webinar and the development of a Q&A document, depending on future developments and identified needs.

In the meantime, please refer to the summary of opinion below. You may also send us any questions or queries at communications@sma-europe.eu.

Additional information:
CHMP summary of opinion: Itvisma, INN-onasemnogene abeparvovec

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