

PRESS RELEASE

Novartis receives positive CHMP opinion for Itvisma[®] for spinal muscular atrophy (SMA)

- *If approved, Itvisma (intrathecal onasemnogene abeparvovec) will be first and only gene replacement therapy for children two years and older, teens and adults with SMA in the European Union*
- *Itvisma demonstrated clinically meaningful and statistically significant improvement in motor function in Phase III STEER study*
- *One-time dose of Itvisma replaces SMN1 gene, with potential to reduce need for chronic SMA treatment*

Basel, April 24, 2026 – Novartis today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending marketing authorization for Itvisma[®] (intrathecal onasemnogene abeparvovec). The opinion supports its use for the treatment of children two years and older, teens, and adults living with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the survival motor neuron 1 (*SMN1*) gene.

Itvisma is uniquely designed to address the genetic root cause of SMA with a one-time fixed dose that does not need to be adjusted for age or body weight.¹ By replacing the *SMN1* gene, Itvisma can improve motor function, offering the potential to reduce the need for chronically administered treatment associated with other available therapies for this population.¹

The CHMP opinion is based on data from the registrational STEER study, and supportive Phase IIIb STRENGTH and Phase I/II STRONG studies.^{1,2,3} In STEER, Itvisma demonstrated a statistically significant 2.39-point improvement in the Hammersmith Functional Motor Scale (HFMSE) with effects sustained over 52 weeks of follow-up.^{1,2,3} The STEER and STRENGTH studies also showed clinically meaningful benefit for treatment-naïve and pre-treated patients.^{1,2}

Nicole Gusset, CEO, SMA Europe, said: “Living with SMA affects every stage of life, including education, employment, and independence. Older children, teenagers, and adults face fewer opportunities to benefit from innovative therapies. A positive CHMP opinion for Itvisma is an important step toward addressing this gap.”

Professor Tim Hagenacker, Department of Neurology, University Hospital Essen, Essen, Germany, said: “Itvisma met the primary endpoint showing motor function improvement versus placebo. Even a 1-point difference in the HFMSE can translate into tangible functional gains for individuals with SMA, such as the ability to grasp a pen. Preserving existing capabilities is critical, as maintaining independence and autonomy is a central goal of care for patients living with a progressive neuromuscular disease.”

“Today’s positive CHMP opinion is a significant step towards potentially reducing the long-term burden of chronic treatment administration for patients living with SMA in Europe,” said Patrick Horber, MD, President, International, Novartis. “Building on the established role of Zolgensma for babies and young children with SMA, this opinion for Itvisma reflects our ambition to expand treatment options for a broader patient population in SMA. It also underscores Novartis’ commitment to pushing scientific boundaries to address unmet needs and improve long-term outcomes across the SMA and rare disease community.”

Key efficacy results from the registrational STEER study

Endpoint	Itvisma (n=75)	Sham (n=51)	P value
HF MSE point improvement	2.39	0.51	0.0074

The most frequently reported adverse reactions were similar across the STEER, STRENGTH and STRONG studies.^{1,2,3} Results from the STEER and STRENGTH studies were published in *Nature Medicine*.

Following the CHMP's recommendation for approval, the European Commission (EC) is expected to issue a final decision within approximately two months.

About SMA

Spinal muscular atrophy (SMA) is a rare, genetic neuromuscular disease caused by a mutated or missing *SMN1* gene.^{4,5} The *SMN1* gene is responsible for producing most of the SMN protein a body needs for muscle function, including breathing, swallowing and basic movement.⁵ Without it, motor neurons are irreversibly lost, leading to progressive, debilitating muscle weakness.⁵ A second gene, the *SMN2* gene, produces a small fraction (~10%) of functional SMN protein compared with the *SMN1* gene.⁶ Individuals with more copies of the *SMN2* gene generally have a less severe form of SMA than those with fewer copies.⁶ SMA has an estimated global prevalence of around 1 to 2 per 100,000 people, with an incidence of roughly 1 in 10,000 live births.⁷

About Itvisma[®] (onasemnogene abeparvovec)

Itvisma, an adeno-associated virus 9 (AAV9)-based gene therapy, is uniquely designed to address the genetic root cause of SMA by providing a functional copy of the human *SMN1* gene to improve motor function through sustained SMN protein expression with a single, one-time intrathecal injection. The brand name Itvisma has been conditionally accepted by EMA for the investigational product OAV101B (onasemnogene abeparvovec), but the product itself has not yet received marketing authorization from the EC. In the US, OAV101B has been approved under the brand name Itvisma.

Novartis has an exclusive, worldwide license with Nationwide Children's Hospital to both the intravenous and intrathecal delivery of AAV9 gene replacement therapy for the treatment of all types of SMA; an exclusive, worldwide license from REGENXBIO for any recombinant AAV vector in its intellectual property portfolio for the in vivo gene replacement therapy treatment of SMA in humans; an exclusive, worldwide licensing agreement with Généthon for in vivo delivery of AAV9 vector into the central nervous system for the treatment of SMA.

Novartis in neuroscience

Neurological diseases are deeply personal, affecting people of any age, from newborns to seniors, often striking in the prime of life. At Novartis, we're doubling down on our commitment to neurology, expanding our legacy of innovation in SMA and multiple sclerosis (MS) to work in neuroimmunology, neurodegeneration, and neuromuscular diseases. Our goal is to protect people's health across their lifespan, developing more treatment options that lead to better outcomes.

Disclaimer

This press release contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as "potential," "can," "will," "plan," "may," "could," "would," "expect," "believe," "committed," "commitment," "pipeline," "launch," "potentially," "step forward," "goal," or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for Itvisma, or regarding potential future revenues from Itvisma. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that Itvisma will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that Itvisma will be commercially successful in the future. In particular, our expectations regarding Itvisma could be affected by,



among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases; safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG's current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

About Novartis

Novartis is an innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease. Our medicines reach more than 300 million people worldwide.

Reimagine medicine with us: Visit us at <https://www.novartis.com> and connect with us on [LinkedIn](#), [Facebook](#), [X/Twitter](#) and [Instagram](#).

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