Novartis International AG
CH-4002 Basel
Switzerland

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PRESS RELEASE

Novartis receives FDA approval for Itvisma®, the only gene replacement therapy for children two years and older, teens, and adults with spinal muscular atrophy (SMA)

Ad hoc announcement pursuant to Art. 53 LR

- *Itvisma (onasemnogene abeparvovec-brve) demonstrated improved motor function and stabilization in patients regardless of SMA treatment history in Phase III studies*
- *One-time dose of Itvisma replaces SMN1 gene, potentially reducing the need for chronic SMA treatment*
- *Gene replacement therapy now available to eligible people of all ages living with SMA*

Basel, November 24, 2025 – Novartis today announced that the US Food and Drug Administration (FDA) has approved Itvisma® (onasemnogene abeparvovec-brve) for the treatment of children two years and older, teens and adults living with spinal muscular atrophy (SMA) with a confirmed mutation in the survival motor neuron 1 (*SMN1*) gene, making it the first and only gene replacement therapy available for this broad population. 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 FDA’s approval of intrathecal onasemnogene abeparvovec is a game-changing advance, expanding the use of transformational gene replacement therapy for SMA across age groups,” said John W. Day, MD, PhD, Professor of Neurology and Pediatrics, Director, Division of Neuromuscular Medicine at Stanford University School of Medicine, and Co-Director of Stanford’s Neuro IGNITE Center. “This achievement is not only a significant step forward for SMA – it also signals new possibilities for the broader field of neurological disorders and genetic medicine.”

The approval of Itvisma is based on data from the registrational Phase III STEER study and supported by the open-label Phase IIIb STRENGTH study. Itvisma showed statistically significant improvements in motor function and stabilization of motor abilities typically not seen in the natural history of the disease, with effects sustained over 52 weeks of follow-up.[2,3] Additionally, Itvisma demonstrated a safety profile with adverse events that were consistent across both studies.[2,3] The most common adverse events in the STEER study were upper respiratory tract infection and pyrexia, and the most common adverse events in the STRENGTH study were common cold, pyrexia, and vomiting.[2,3] These data were presented at the 2025 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference.

“This new route of administration for a single dose of gene replacement therapy can mean so much more than what is measured by numbers on a functional motor scale – it could mean greater independence and freedom in activities of daily life,” said Kenneth Hobby, President, Cure SMA. “The SMA disease landscape has dramatically changed over the last six years, when the first gene therapy was approved. This is another welcome advancement, and it represents real progress in expanding access for many older patients and addressing the unmet needs that remain in our community.”

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]

Approximately 9,000 people in the US live with SMA, and though there have been advancements in treating the disease, unmet needs remain for older children, teens, and adults in preserving motor neurons and maintaining physical strength.[7,8]

Transforming care in SMA
“After redefining SMA care with the first gene replacement therapy for this challenging disease, we can now help address unmet needs across an even broader SMA population with the approval of Itvisma," said Victor Bultó, President, Novartis, US. “We are proud to support the SMA community by empowering patients of all ages through our innovative, one-time therapies, offering the potential to reduce the burden that comes with chronic treatment.”

Itvisma will be available in the US in December. Novartis Patient Support is available to help eligible patients get started on treatment. Patients and providers can call 1-855-441-4363 for personalized assistance, including help understanding insurance coverage and identifying potential financial assistance options.

About Itvisma® (onasemnogene abeparvovec-brve)
Itvisma is 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.

Novartis has an exclusive, worldwide license with Nationwide Children's Hospital to both the intravenous and intrathecal delivery of adeno-associated virus 9 (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 spinal muscular atrophy (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 nearly 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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Novartis Media Relations
E-mail: media.relations@novartis.com

Novartis Investor Relations
Central investor relations line: +41 61 324 7944
E-mail: investor.relations@novartis.com