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MEDIA & INVESTOR RELEASE

Novartis expands Zolgensma manufacturing capacity with approval of multi-product North Carolina facility

- *Clearance of critical milestone expands capability for production of gene therapies, starting with Zolgensma®*

Basel, April 5, 2022 – Novartis today announced the U.S. Food and Drug Administration (FDA) has granted commercial licensure approval for its Durham, N.C. site, a multi-product gene therapy manufacturing facility. This approval allows the state-of-the-art, 170,000 square-foot facility to make, test and release commercial Zolgensma, as well as produce gene therapy product for current and future clinical trials.

The clearance of this milestone brings online the second commercially-licensed manufacturing facility for Novartis Gene Therapies, joining the Libertyville, Ill. site, which was approved for Zolgensma® (onasemnogene abeparvovec) manufacturing and distribution in 2019. The Durham site will build on Novartis Gene Therapies' manufacturing capacity to form a two-site network, adding extensive segregated production suites for multi-product manufacturing.

"Bringing the North Carolina facility fully online reinforces the supply of Zolgensma and signifies the Novartis commitment to this critical advanced therapy platform," said Christine Fox, President of Novartis Gene Therapies. "Not only will this facility support the Novartis pipeline through the manufacture of both clinical trial and commercial products, it ultimately allows us to help more patients and families living with rare, genetic diseases."

The North Carolina facility is located in Research Triangle Park. Through close collaboration with the government and life sciences community, Novartis is contributing to this hub of innovation.

North Carolina Governor Roy Cooper said, "The investment by Novartis Gene Therapies in Durham illustrates North Carolina's leadership in the life sciences industry, our talented workforce and extensive resources to support major companies. This commercial licensure approval places our state at the forefront of cutting-edge gene therapies and we're looking forward to continued collaboration with Novartis."

The North Carolina site's primary initial responsibility will be manufacturing Zolgensma®, an essential one-time treatment for spinal muscular atrophy (SMA). The facility will begin producing and shipping the gene therapy across the U.S. immediately.

If left untreated in its most severe forms, SMA leads to death or the need for permanent ventilation by the age of two in more than 90 percent of cases.[1,2] Approved in more than 40 countries, to date, more than 1,800 patients have been treated with Zolgensma globally, including in clinical trials, managed access programs and in the commercial setting.[3]

“Powered by our multidisciplinary teams and our pioneering technology, this milestone represents our ability to effectively manufacture and supply complex gene therapies at quality and scale,” added Steffen Lang, Global Head of Novartis Technical Operations.

About Zolgensma
Zolgensma® (onasemnogene abeparvovec) is the only approved gene therapy for the treatment of spinal muscular atrophy (SMA) and the only SMA treatment designed to directly address the genetic root cause of the disease by replacing the function of the missing or non-working *SMN1* gene to halt disease progression through sustained SMN protein expression with a single, one-time IV infusion. Zolgensma is now approved in more than 40 countries and more than 1,800 patients have been treated with Zolgensma globally across clinical trials, managed access programs, and in the commercial setting.[3] Novartis Gene Therapies is unwavering in its commitment to reimagine the possibilities for children living with SMA and continues to evaluate Zolgensma across a robust clinical development program, as well as the investigational intrathecal administration of OAV101 in patients with later-onset forms of SMA.

Novartis Gene Therapies has an exclusive, worldwide license with Nationwide Children's Hospital to both the intravenous and intrathecal delivery of AAV9 gene 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 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; and a non-exclusive, worldwide license agreement with AskBio for the use of its self-complementary DNA technology for the treatment of SMA.

About Spinal Muscular Atrophy
Spinal muscular atrophy (SMA) is a rare, genetic neuromuscular disease and a leading genetic cause of infant death.[1,2] Caused by the lack of a functional *SMN1* gene, the most severe forms of SMA results in the rapid and irreversible loss of motor neurons, affecting muscle functions including breathing, swallowing and basic movement.[4] Severity varies across a spectrum of types corresponding to the number of copies of the back-up *SMN2* gene.[5] The majority (>70 percent) of patients with two copies of *SMN2* develop Type 1, the most common form accounting for 60 percent of cases.[6,7] Type 1 is severe and, left untreated, leads to death or the need for permanent ventilation by the age of two in more than 90 percent of cases.[1,2] Most patients (>80 percent) with three copies of *SMN2* develop Type 2, accounting for 30 percent of cases.[6] Left untreated, patients with Type 2 are unable to walk and will require a wheelchair, and more than 30 percent will die by age 25.[8] Loss of motor neurons cannot be reversed, so it is imperative to diagnose SMA and begin treatment, including proactive supportive care, as early as possible to halt irreversible motor neuron loss and disease progression.[9,10]

About Novartis Gene Therapies
Novartis Gene Therapies is reimagining medicine to transform the lives of people living with rare genetic diseases. Utilizing cutting-edge technology, we are working to turn promising gene therapies into proven treatments, starting with our first approved treatment for spinal muscular atrophy (SMA). We are powered by an extensive manufacturing footprint, in capacity and expertise, enabling us to bring gene therapy to patients around the world at quality and scale. Novartis Gene Therapies OAV101 clinical development program represents a growing body of research in a range of patients with SMA, across ages, SMA types and incident and prevalent populations, investigating both intravenous and intrathecal formulations.

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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,” “anticipate,” “seek,” “look forward,” “believe,” “committed,” “investigational,” “pipeline,” “launch,” or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for the investigational or approved products described in this press release, or regarding potential future revenues from such products. 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 the investigational or approved products described in this press release 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 such products will be commercially successful in the future. In particular, our expectations regarding such products 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 such as COVID-19; 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 reimagining medicine to improve and extend people’s lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world’s top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 108,000 people of more than 140 nationalities work at Novartis around the world. Find out more at **https://www.novartis.com.**

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