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MEDIA & INVESTOR RELEASE

Novartis Provides Update on AVXS-101 Intrathecal Clinical Development Program

- *Novartis Gene Therapies to initiate new pivotal confirmatory study to evaluate use of AVXS-101 intrathecal (IT) formulation in older patients with SMA to further support registration*

Basel, September 23, 2020 – Novartis Gene Therapies recently received feedback from the US Food and Drug Administration (FDA) following their review of data from the STRONG study of the intrathecal (IT) formulation of AVXS-101 in older patients with spinal muscular atrophy (SMA). The FDA has acknowledged the potential of AVXS-101 IT in this patient population and recommends a pivotal confirmatory study to supplement the existing STRONG data and further support the regulatory submission for AVXS-101 IT.

This guidance provides clarity on the path to registration for AVXS-101 IT. Trial design and other details are being evaluated and a comprehensive update on the overall Novartis SMA clinical development program will be provided at a future time following further discussions with health authorities. This request for a study is unrelated to the partial clinical hold on AVXS-101 IT, and the new study will not be initiated in the US until the hold has been lifted by the FDA. Novartis Gene Therapies remains confident in the overall benefit-risk profile for patients on treatment. This does not impact marketed Zolgensma® (onasemnogene abeparvovec) and the company continues to advance its regulatory filings and intravenous clinical studies.

Novartis Gene Therapies reaffirms its commitment to the SMA community and to pursuing solutions for patients with all types of SMA, including older children and adults. All patients deserve a gene therapy designed to address the genetic root cause of their disease with a single dose.

Zolgensma is approved in the US, Japan and, most recently, Brazil, for patients with SMA under the age of two. Zolgensma also continues to have a strong launch in Europe where it is approved for babies and young children with a clinical diagnosis of SMA Type 1 or SMA with up to three copies of the *SMN2* gene, with dosing guidance provided up to 21 kg. More than 600 patients have benefited from Zolgensma, including through clinical trials, commercially and through the managed access program. This number is expected to continue to grow as this transformative gene therapy is approved in additional markets and as the company pursues additional studies to fully explore the impact of Zolgensma across a broad population of patients with SMA.

The company's commitment to SMA extends beyond gene therapy to branaplam (LMI070), an oral, once-weekly RNA splicing modulator also currently under development, to expand the treatment options for SMA patients.

About Zolgensma® (onasemnogene abeparvovec)
Zolgensma® is designed to address the genetic root cause of SMA by providing a functional copy of the human SMN gene to halt disease progression through sustained SMN protein expression with a single, one-time IV infusion. Zolgensma was approved by the US Food and Drug Administration in May 2019 and represents the first approved therapeutic in the company's proprietary platform to treat rare, monogenic diseases using gene therapy. In addition to the US, Zolgensma is approved in Japan, Europe and Brazil. More than 600 patients have been treated with Zolgensma, including clinical trials, commercially and through the managed access program. Novartis Gene Therapies is pursuing registration in close to three dozen countries with regulatory decisions anticipated in Switzerland, Canada, Israel, Australia, and South Korea in late-2020 or early 2021.

About Spinal Muscular Atrophy
SMA is the leading genetic cause of infant death. If left untreated, SMA Type 1 leads to death or the need for permanent ventilation by the age of two in more than 90% of cases.
SMA is a rare, genetic neuromuscular disease caused by a lack of a functional *SMN1* gene, resulting in the rapid and irreversible loss of motor neurons, affecting muscle functions, including breathing, swallowing and basic movement. 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. This is especially critical in SMA Type 1, where motor neuron degeneration starts before birth and escalates quickly. Loss of motor neurons cannot be reversed, so SMA patients with symptoms at the time of treatment will likely require some supportive respiratory, nutritional and/or musculoskeletal care to maximize functional abilities. More than 30% of patients with SMA Type 2 will die by age 25.

About Novartis Gene Therapies
Novartis Gene Therapies (formerly AveXis) is reimagining medicine to transform the lives of people living with rare genetic diseases. Utilizing cutting-edge technology, we are turning promising gene therapies into proven treatments, beginning with our transformative gene therapy for spinal muscular atrophy (SMA). This therapy is now approved in the US, Japan, Europe and Brazil, and additional registrations are being pursued in close to three dozen countries, with regulatory decisions anticipated in Switzerland, Canada, Israel, Australia, Argentina and South Korea in late 2020 or early 2021. Our robust AAV-based pipeline is advancing treatments for Rett syndrome; a genetic form of amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 *(SOD1)* gene; and Friedreich's ataxia. We are powered by the world's largest gene therapy manufacturing footprint of more than one million square feet, enabling us to bring these therapies to patients around the world at quality and scale.

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 "to initiate," "to evaluate," "to further support," "potential," "recommends," "to supplement," "being evaluated," "will," "to advance," "could," "anticipated," "remains," "continues," "to advance," "reaffirms," "commitment," "to pursuing," "to address," "explore," "investigational," "launch," "under development," or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for AVXS-101 IT, Zolgensma and branaplam, or regarding potential future revenues from AVXS-101 IT, Zolgensma and branaplam. 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 AVXS-101 IT or branaplam will be submitted or approved for sale in any market, or at any particular time. Neither can there be any guarantee that Zolgensma 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 AVXS-101 IT, Zolgensma or branaplam 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 109,000 people of more than 140 nationalities work at Novartis around the world. Find out more at https://www.novartis.com.

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