New data at AAN reinforce Novartis commitment to transforming the lives of people of all ages who live with neurological conditions

- **Novartis leads the way at AAN with 53 abstracts, showcasing its broad portfolio of transformative medicines and innovative solutions for people living with spinal muscular atrophy (SMA), multiple sclerosis (MS) and migraine.**

- **Interim data from multiple ongoing clinical studies of Zolgensma® (onasemnogene abeparvovec-xioi; AVXS-101) show positive results in the treatment of SMA.**

- **EXPAND analyses show people with secondary progressive MS (SPMS) taking Mayzent® (siponimod) – the only therapy proven to delay disability progression in a study of typical SPMS patients – experienced significant benefits on cognitive function.**

- **With the greatest real world exposure and longest clinical trial experience of any anti-CGRP therapy, Aimovig® (erenumab) continues to show positive long-term safety and efficacy across the spectrum of migraine with new clinical trial data.**

**Basel, April 30, 2019** – Novartis announced today it will present 53 abstracts from across its unique neurological portfolio and pipeline at the upcoming 71st American Academy of Neurology (AAN) meeting May 4 - 10 in Philadelphia, Pennsylvania, USA. The breadth and depth of the data highlights the company’s commitment to decoding the science behind neurological diseases such as spinal muscular atrophy (SMA), multiple sclerosis (MS) and migraine.

“Our presence at AAN this year is a testament to how we are reimagining medicine across a broad spectrum of neurological diseases, for many patients of all ages,” said Danny Bar-Zohar, Global Head, Neuroscience Development for Novartis Pharmaceuticals. “Nothing is more rewarding than combining cutting-edge science, advanced algorithms and technology with a relentless ambition to bring life-changing solutions to people who need them.”

**Novartis highlights at AAN include:**

**Spinal muscular atrophy:**
- Four new scientific abstracts spanning the clinical development program for the investigational gene therapy Zolgensma® (onasemnogene abeparvovec-xioi; AVXS-101) show positive results in the treatment of SMA.
- Interim data presented for the first time from the Phase I STRONG trial demonstrated encouraging results via the intrathecal delivery in patients with SMA Type 2.
- New interim data from the Phase III STR1VE study demonstrated survival and motor function improvements that continue to parallel the Phase I START study.
• Preliminary data from the Phase III SPR1NT trial demonstrated significant motor function improvements in pre-symptomatic SMA patients.
• Data from the ongoing long-term follow-up study for the Phase I START trial show no waning of motor milestone achievements in patients with SMA Type 1.
• Three health economics studies demonstrated improved survival and milestone achievement with Zolgensma.
• New data supporting the potential use of serum neurofilament light chain as biomarker for SMA Type 1 disease activity and therapy response under branaplam (LMI070), an oral, once-weekly RNA splicing modulator.

Zolgensma was granted Priority Review for the treatment of SMA Type 1 by the FDA and regulatory action is anticipated in May 2019.

Multiple Sclerosis12,13, 23-38:
• New analyses from the Phase III EXPAND study show Mayzent® (siponimod) had a sustained benefit on cognition in secondary progressive MS (SPMS) patients. Impaired cognitive function is a key aspect of disability associated with MS. It substantially affects the social and professional lives of people with MS and their families. Mayzent was approved by US Food and Drug Administration (FDA) in March 2019 for the treatment of adults across the spectrum of relapsing multiple sclerosis, including SPMS with active disease*. It is the only FDA-approved treatment for active SPMS, based on a positive pivotal study in a typical SPMS population.
• Presented for the first time, the full results from the Phase III ASSESS study, a direct head-to-head trial comparing Gilenya® (fingolimod) vs. glatiramer acetate patients with RRMS. Top line results announced in October 2018 showed treatment with Gilenya 0.5mg demonstrated superior efficacy in significant reduction of annualized relapse rate, with nearly 41% fewer relapses versus glatiramer acetate, as well beneficial effects on other key measures of disease activity.
• MSProDiscuss™, the first-of-its-kind algorithm-based tool developed side-by-side with renowned MS researchers, healthcare professionals and patients, to facilitate doctor-patient discussions around MS progression.
• Scientific explorations of ofatumumab, the first fully human anti-CD20 monoclonal antibody with a monthly self-administered subcutaneous dosing regimen tailored for RMS, allowing for the potential preservation of immune function.
• Furthermore, new analyses supporting neurofilaments as an increasingly used biomarker in MS clinical trials will be highlighted. Utilizing blood neurofilaments as an easy-to-use biomarker for MS could revolutionize the way MS treatments are assessed in clinical trials and ultimately, in clinical practice. Novartis is leading the field with 18 data analyses presented so far.

*Mayzent was approved by the US FDA in March 2019 for the treatment of adults with relapsing forms of multiple sclerosis, including SPMS with active disease, relapsing remitting multiple sclerosis (RRMS) and clinically isolated syndrome (CIS). Clinically isolated syndrome (CIS) is defined as a first episode of neurologic symptoms that lasts at least 24 hours and is caused by inflammation or demyelination in the central nervous system.

Migraine14-22,
• Nine new scientific abstracts on Aimovig® (erenumab), reinforcing the extensive long term safety and efficacy profile of the drug across the spectrum of migraine, including chronic, episodic and difficult to treat migraine patients.
• A new set of data shows that a majority of chronic migraine patients on Aimovig converted to episodic migraine. In a separate study, almost two thirds of patients with episodic migraine reported a sustained reduction of migraine days at one year.
Aimovig has been studied extensively in a broad clinical program involving more than 3,000 patients. Since launch in May 2018, Aimovig has been used to treat an estimated 220,000 patients worldwide, making it the most prescribed anti-CGRP therapy worldwide.

**Novartis in Neuroscience**

Novartis has a strong ongoing commitment to neuroscience and to bringing innovative treatments to patients suffering from neurological conditions where there is a high unmet need. We are committed to supporting patients and physicians in multiple disease areas, including MS, migraine, Alzheimer's disease and Parkinson's disease and have a promising pipeline in MS, Alzheimer's disease and spinal muscular atrophy.

**About AveXis, a Novartis Company**

AveXis, a Novartis company, is dedicated to developing and commercializing novel treatments for patients suffering from rare and life-threatening neurological genetic diseases. Our initial product candidate, Zolgensma (onasemnogene abeparvovec-xioi; AVXS-101), is its proprietary gene therapy currently in development for the treatment of spinal muscular atrophy, or SMA. In addition to developing Zolgensma to treat SMA, AveXis also plans to develop other novel treatments for rare neurological diseases, including Rett syndrome and a genetic form of amyotrophic lateral sclerosis caused by mutations in the superoxide dismutase 1 (SOD1) gene. For additional information, please visit [www.avexis.com](http://www.avexis.com).

**About Amgen and Novartis Neuroscience Collaboration**

In August 2015, Amgen entered into a global collaboration with Novartis to develop and commercialize pioneering treatments in the field of migraine and Alzheimer's disease. The collaboration focuses on investigational Amgen drugs in the migraine field, including Aimovig (approved by the FDA in May 2018 for the preventive treatment of migraine in adults). In April 2017, the collaboration was expanded to include co-commercialization of Aimovig in the U.S. For the migraine programs, Amgen retains exclusive commercialization rights in the U.S. (other than for Aimovig as described above) and Japan, and Novartis has exclusive commercialization rights in Europe, Canada and rest of world. Also, the companies are collaborating in the development and commercialization of a beta-secretase 1 (BACE) inhibitor program in Alzheimer's disease. The oral therapy CNP520 (currently in Phase III for Alzheimer's disease) is the lead molecule and further compounds from both companies' preclinical BACE inhibitor programs may be considered as follow-on molecules. At the center of the Amgen and Novartis neuroscience collaboration is the shared mission to fight migraine and the stereotypes and misperceptions surrounding this debilitating disease.

**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," "expect," "anticipate," "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 or the collaboration with Amgen. 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. Neither can there be any guarantee that the collaboration with Amgen will achieve any or all of its intended goals, or within any particular time frame. Nor can there be any guarantee that such products or the collaboration with Amgen will be commercially successful in the future. In particular, our expectations regarding such products and the
collaboration with Amgen 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 outcome of litigation and legal disputes, including the legal dispute with Amgen regarding our collaboration agreements in the field of migraine; the particular prescribing preferences of physicians and patients; general political and economic conditions; safety, quality 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.

*The brand name Zolgensma has been provisionally approved by the FDA for the investigational product AVXS-101 (onasemnogene abeparvovec-xioi), but the product itself has not received marketing authorization or BLA approval from any regulatory authorities.

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 more than 750 million people globally and we are finding innovative ways to expand access to our latest treatments. About 105,000 people of more than 140 nationalities work at Novartis around the world. Find out more at www.novartis.com.

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