AveXis expands world-leading gene therapy manufacturing capacity with purchase of advanced biologics therapy manufacturing campus in Longmont, Colorado

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- - Facility to become the largest of four state-of-the-art sites involved in manufacturing of AveXis gene therapies for pipeline of rare genetic diseases including spinal muscular atrophy
- - AveXis plans to offer positions to all approximately 150 employees previously employed at the site, and to announce further expansion of new jobs in the near term
- - Investment in the Longmont campus adds to existing \$115 million investment in Durham facility, leading to creation of more than 1,000 US-based, high-tech, biologics manufacturing jobs by the end of 2019

BASEL, Switzerland, April 1, 2019 /PRNewswire/ -- AveXis, a Novartis company, today announced it signed an agreement to purchase an advanced biologics therapy manufacturing campus in Longmont, Colorado, further expanding AveXis' production capacity as it prepares to launch Zolgensma[®] (onasemnogene abeparvovec-xioi¹) an investigational gene therapy awaiting global regulatory approvals for the treatment of spinal muscular atrophy (SMA) Type 1 and for future gene therapy treatments in development.

AveXis currently has a fully-operational state-of-the-art manufacturing facility in Illinois, is building a facility in North Carolina scheduled to be operational in 2020 and is expanding its product development capacity at its San Diego facility. The addition of the six-building Longmont campus consists of nearly 700,000 square-feet of space for biologic drug manufacturing, offices, laboratories, warehousing and utilities. Initial start-up activities in Longmont will include preparing the facility for scaling, manufacturing and testing of gene therapies and hiring staff.

"Our Longmont, Colorado, campus, along with our existing manufacturing sites in Illinois, California and North Carolina, will play a crucial role in helping us achieve the future manufacturing capacity required to meet the global patient need for novel gene therapies," said Andrew Knudten, Senior Vice President, Global Strategic Operations. "We have built a team with exceptional depth of experience, unified by a common mission: to positively impact the lives of patients and families devastated by rare and life-threatening neurological genetic diseases. We are eager to add the talented team in Longmont to AveXis, and we hope that they will choose to join us as we build world-leading manufacturing capabilities in gene therapy."

"AveXis' success requires not just medical breakthroughs, but innovations in R&D and manufacturing. With the opening of our fourth location in the US, we will create more than 1,000 high-tech biologics manufacturing jobs by the end of 2019," said Dave Lennon, President. "AveXis has now established leading technical manufacturing capabilities with the capacity to deliver our robust pipeline, as well as the flexibility to enter into multiple external partnerships as the development and manufacturing partner of choice in gene therapy."

About Zolgensma®

development as a one-time infusion for SMA Type 1. Zolgensma is designed to address the monogenic root cause of SMA and prevent further muscle degeneration. Zolgensma represents the first in a proprietary platform to treat rare, monogenic diseases using gene therapy. In December, the FDA accepted the company's Biologics License Application for use of Zolgensma with SMA Type 1 patients. The drug previously received Breakthrough Therapy designation and has been granted Priority Review by the FDA, with regulatory action anticipated in May 2019. In addition, the drug is anticipated to receive approval in Japan and the European Union later this year.

About SMA

SMA is a severe neuromuscular disease characterized by the loss of motor neurons leading to progressive muscle weakness and paralysis. SMA is caused by a genetic defect in the SMN1 gene that codes SMN, a protein necessary for survival of motor neurons. The incidence of SMA is approximately one in 10,000 live births and is the leading genetic cause of infant mortality. The most severe form of SMA is Type 1, a lethal genetic disorder characterized by rapid motor neuron loss and associated muscle deterioration, which results in mortality or the need for permanent ventilation support by 24 months of age for more than 90 percent of patients.

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 become," "plans," "to announce," "in the near term," "leading to," "awaiting," "building," "scheduled," "expanding," "eager," "hope," "anticipated," "potential," "can," "will," "expect," "anticipate," "look forward," "believe," "committed," "investigational," "pipeline," "launch," or similar terms, or by express or implied discussions regarding the acquisition of the manufacturing campus in Longmont, Colorado; regarding potential strategic benefits or opportunities from the acquisition; regarding potential marketing approvals, new indications or labeling for the investigational products described in this press release, or regarding potential future revenues from such products. You should not place undue reliance on these statements. Such forwardlooking 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 Novartis will be able to realize any of the potential strategic benefits or opportunities as a result of the acquisition of the manufacturing campus in Longmont, Colorado within any particular time frame, or at all. Neither can there be any guarantee that the investigational products described in this press release will be submitted or approved for sale or for any additional indications or labelling 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 and the acquisition of the manufacturing campus in Longmont, Colorado 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; the potential that the strategic benefits or opportunities expected from the acquisition may not be realized or may take longer to realize than expected; 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 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.

About AveXis

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, 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.

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 guest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach more than 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 130,000 people of nearly 150 nationalities work at Novartis around the world. Find out more at www.novartis.com.

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References

The brand name Zolgensma® (onasemnogene abeparvovec-xioi) has been provisionally approved by the 1. FDA for the investigational product AVXS-101, but the product itself has not received marketing authorization or BLA approval from any regulatory authorities.

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List of links present in page

- 1. https://qa1.novartis.us/us-en/us-en/news/media-releases/avexis-expands-world-leading-gene-therapy-manufacturing-capacity-purchase-advanced-biologics-therapy-manufacturing-campus-longmont-colorado
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