

MEDIA RELEASE • COMMUNIQUE AUX MEDIAS • MEDIENMITTEILUNG**Gene therapy “Made in Europe”: Novartis to make Kundl a center of excellence in nucleic acid production**

- *Opening of a high-tech production facility for plasmids – the key raw material in cell and gene therapies, with potential for significant expansion*
- *An essential part of the rapidly increasing global demand for plasmids for development programs, as well as for the in-market product from Novartis Gene Therapies, will be covered from Kundl as of 2021*
- *A total of USD 20.4 million (equivalent to EUR 17.2 million) has been earmarked for investment by mid-2021 in nucleic acid production lines at the location, which will employ around 45 highly skilled members of staff*

Kundl, Austria, November 30, 2020 — Novartis is focusing increasingly on Europe in the manufacture of its innovative gene therapies. The company plans to make the Kundl location a center of excellence in the production of nucleic acids for its commercial therapies and also for its diverse development projects. A new facility was opened today and will be used with immediate effect for Austria-based manufacture of plasmids for study medication in gene-therapy pipeline programs. Plasmids are ring-shaped DNA molecules that are used in cell and gene therapies, making them a vital component of all innovative cell and gene therapies developed by Novartis. These include the gene therapy platform of Novartis Gene Therapies that the company is using to herald a new era in the treatment of monogenic neuromuscular disorders.

“Demand for nucleic acid products has increased significantly as a result of intensified research in cell and gene therapies. The increase in proprietary production in this high-tech area is an important strategic step in aligning our production network with future medical demands,” explained Steffen Lang, Global Head of Novartis Technical Operations and member of the Novartis Executive Committee. “The new facility in Kundl is an ideal addition to our global production network for cell and gene therapies. We plan on continuing to increase capacities for the manufacture of nucleic acid products at the location.”

Mario Riesner, Managing Director of Sandoz GmbH, added: “We are delighted to be able to support patients all over the world who suffer from severe monogenic diseases. These new facilities will enable us to make a significant contribution to the supply of innovative gene therapeutics and open a new chapter in our long-established Kundl location. We have been involved in pioneering work here since 1946 – starting with antibiotics, then the first biosimilar and on to today’s central production facility for nucleic acids.”

Investment in technological development

Novartis Gene Therapies is the world’s leading gene therapy company and, with its innovative gene therapy platform, is redefining the options available to patients affected by life-threatening

genetic diseases and their families. Novartis has already invested USD 15.7 million this year in the new facility for the production of plasmid DNA. A further USD 4.7 million will result in an additional facility for the manufacture of mRNA by the middle of next year, by which time a total of 45 employees will be involved in nucleic acid production. Staff at this center of excellence will be qualified for highly specialized functions in the production of nucleic acids. The center also has its own development department that focuses exclusively on the ongoing technological development of gene therapies.

Plasmids: a cell construction manual for treating rare genetic defects at the root cause

Gene therapies are manufactured in a long and complex process requiring a series of biological stages. One highly important element in these innovative therapies is also the smallest: plasmids. These ring-shaped DNA molecules carry the instructions to enable the cells in patients' bodies to manufacture the gene therapies from Novartis. The production of plasmid DNA takes approximately seven days. The various cell and gene therapies from Novartis require three to four of these plasmids. The DNA molecules produced in Kundl are brought together with special cell lines and used to produce adeno-associated viruses (AAVs). Once the viral envelopes have taken up the DNA plasmids, they are referred to as AAV vectors, which serve as a vehicle for transporting the therapeutically active genetic material to patients' cells. It takes a total of 30 days, for example, to produce a single-use gene therapy for spinal muscular atrophy that is tailored according to a patient's individual weight.

About Novartis

Novartis is reimagining medicine in order 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 medicines 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 145 nationalities work at Novartis around the world. Find out more at www.novartis.com.

About Novartis Gene Therapies

Novartis Gene Therapies (formerly AveXis) is rethinking medicine with a view to improving and extending the lives of people with rare genetic diseases. We use cutting-edge technology to develop promising gene therapies into tried-and-tested treatments, starting with our transformative gene therapy for spinal muscular atrophy (SMA). This therapy has now been authorized in the USA, Japan, the EU, Brazil and Israel, with further approval processes under way in almost three dozen countries. Decisions from the regulatory authorities in Switzerland, Canada, Australia, Argentina, Taiwan and South Korea are expected by the end of 2020 or the beginning of 2021. Our solid AAV-based pipeline is driving treatment options for Rett syndrome, a genetic form of amyotrophic lateral sclerosis (ALS) that is caused by mutations in the superoxide dismutase 1 (SOD1) gene, as well as Friedreich's ataxia. With more than 90,000 square meters, we have the world's largest production space for gene therapies, enabling us to provide gene therapies in the requisite quality and volumes for patients all over the world.

About Novartis Austria

We are reimagining medicine – at the cutting edge of society, in the heart of Austria. The Austrian Novartis Group is one of the country's leading pharmaceutical companies and is divided into the business areas of innovative medicines (Pharmaceuticals, Oncology) and generics (Sandoz). Day in, day out, our employees are committed to achieving our mission of improving and extending people's lives. Our medicines enable us to reach 5 million patients worldwide. The Novartis Kundl/Schaftenau locations in the Tirol region form part of the company's global network of research and development centers. Find out more at www.novartis.at

###

Contact person for media inquiries:

Simone Farina

Head Pharma Communications
Novartis Pharma GmbH
+43 1 86657-0
novartis.austria@novartis.com

Angelika Gaufer

Associate Director, Communications, Region EMEA
Novartis Gene Therapies
+43 1 86657-0
novartis.austria@novartis.com

Novartis Pharma GmbH
Jakov-Lind-Straße 5, Top 3.05
1020 Vienna
Austria
www.novartis.at