Novartis and the pan-Canadian Pharmaceutical Alliance (pCPA) complete negotiations for Zolgensma® for the treatment of pediatric patients with spinal muscular atrophy (SMA)

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Dorval, Quebec, October 13, 2021 — Novartis Pharmaceuticals Canada Inc. today announced that it has completed negotiations with the pan-Canadian Pharmaceutical Alliance (pCPA) for a Letter of Intent (LOI) for Zolgensma® (onasemnogene abeparvovec), an adeno- associated virus (AAV) vector-based gene therapy indicated for the treatment of pediatric patients with 5q spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene and 3 or fewer copies of SMN2 gene; or infantile-onset SMA[1].

“We know the SMA community has been waiting for this day and we’re thrilled that children with SMA are one step closer to having provincial access to Zolgensma,” said Andrea Marazzi, Country Pharma Organization Head, Novartis Pharmaceuticals Canada. “We look forward to working with provincial jurisdictions to make Zolgensma available as quickly as possible through public formularies to ensure all children who may benefit from this one-time therapy can access it.”

“This is encouraging news for the SMA community across Canada,” said Susi Vander Wyk, Executive Director, Cure SMA Canada. “However, motor neuron damage from SMA won’t stop while the provincial reimbursement process continues. We hope that provinces move swiftly to add Zolgensma to their drug plans. Access to Zolgensma will offer children with SMA the possibility of disease stabilization, drastically changing the outcome of this disease.”

About Zolgensma

Zolgensma is a gene therapy designed to address the genetic root cause of the disease by replacing the missing or defective SMN1 gene[1]. It is administered during a single intravenous (IV) infusion, delivering a new working copy of the SMN[1] gene into a patient’s cells, halting disease progression and restoring production of SMN protein[1].

The efficacy and safety data supporting the approval of Zolgensma in treating pediatric patients with SMA are derived from completed and ongoing open-label, single-arm, clinical trials in patients with infantile-onset SMA and 2 copies of SMN2 gene; and presymptomatic genetically diagnosed SMA and 2 or 3 copies of SMN2 gene[1].

About Spinal Muscular Atrophy

In Canada each year, approximately one in 10,000 babies are born with SMA, a rare, genetic neuromuscular disease caused by a defective or missing SMN1 gene[2]. Without a functional SMN1 gene, infants with SMA lose the motor neurons responsible for muscle functions such as breathing, swallowing, speaking and walking[3]. Left untreated, muscles become progressively weaker[2,3]. In the most severe form, this eventually leads to paralysis and ultimately permanent ventilation or death by age 2 in more than 90% of cases[4]. This is

why 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[5].

About Novartis in Gene Therapy and Rare Disease

Novartis is at the forefront of cell and gene therapies designed to halt diseases in their tracks or reverse their progress rather than simply manage symptoms. The company is collaborating on the cell and gene therapy frontier to bring this major leap in personalized medicine to patients with a variety of diseases, including genetic disorders and certain deadly cancers.

Cell and gene therapies are grounded in careful research that builds on decades of scientific progress. Following key approvals of cell and gene therapies by health authorities, new treatments are being tested in clinical trials around the world.

About Novartis in Canada

Novartis Pharmaceuticals Canada Inc., a leader in the healthcare field, is committed to the discovery, development and marketing of innovative products to improve the well-being of all Canadians. In 2020, the company invested $45 million in research and development in Canada. Located in Dorval, Quebec, Novartis Pharmaceuticals Canada Inc. employs approximately 1,000 people in Canada and is an affiliate of Novartis AG, which provides innovative healthcare solutions that address the evolving needs of patients and societies. The company prides itself on its commitment to diversity and to nurturing an inclusive and inspiring environment. Novartis is recognized as a Great Place to Work®, ranked among the Top 50 Best Workplaces™ in the country and is proudly named on the 2021 Best Workplaces™ for Women in Canada and Best Workplace™ for Mental Wellness lists. For further information, please consult https://www.novartis.com/ca-en/en.

Zolgensma is a registered trademark.

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; has 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.

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