Novartis applauds Quebec, the first province to list Zolgensma® for the treatment of pediatric patients with spinal muscular atrophy (SMA)

Oct 19, 2021

- *Public reimbursement is effective October 20th and will apply for children with SMA following the recommendation from the Institut national d'excellence en santé et services sociaux*
- *Zolgensma is the first gene therapy to receive formal public reimbursement in Canada*

Dorval, Quebec, October 18, 2021 — Novartis Pharmaceuticals Canada Inc. applauds the government of Quebec's decision to provide public reimbursement for Zolgensma® (onasemnogene abeparvovec) for the treatment of children with SMA. The announcement, which takes effect on October 20th, was made today by Health Minister Christian Dubé and follows the recommendation from the Institut national d'excellence en santé et services sociaux (INESSS) made earlier this year, which includes a pathway for children up to 6 months of age and case-by-case access for children beyond 6 months of age. The specific criteria for reimbursement will be added to the Liste des médicaments, available soon on the Régie de l'assurance maladie du Québec (RAMQ) website.

"This is a momentous day and one we know the Quebec SMA community has been waiting for. I want to thank the Quebec government for their leadership, both in moving quickly to cover Zolgensma, as well as adopting the INESSS recommendations, which acknowledge the societal impact of unequal access due to delayed diagnoses for Quebec children with SMA, " said Andrea Marazzi, Country Pharma Organization Head, Novartis Pharmaceuticals Canada. "We believe all Canadian children with SMA who may benefit from this treatment should have the same opportunity and will continue to collaborate with the provinces, territories and federal plans to provide timely and equitable public reimbursement."

"Today's news from Minister Dubé brings hope to the families in my care who have been seeking access to Zolgensma for their children," said Dr. Nicolas Chrestian, Chief of Pediatric Neurology, specialized in neuromuscular disorders at Centre Hospitalier Mère Enfant Soleil, Université Laval in Québec City. "Prompt access to treatments like Zolgensma that can halt progression of SMA are critical for children to reach their developmental potential and having costs covered by the province helps remove some of the impact that SMA can have on families."

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 SMN1 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.

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.

Novartis Media Relations
Daphne Weatherby
+1 514 633 7873
E-mail: camlph.communications@novartis.com

References

1. Novartis Pharmaceuticals Canada Inc. Zolgensma® (onasemnogene abeparvovec) Product Monograph. June 9, 2021.

2. National Organization for Rare Disorders (NORD). Spinal Muscular Atrophy. Available at https://rarediseases.org/rare-diseases/spinal-muscular-atrophy/. Last accessed October 1, 2021.
3. Anderton RS and Mastaglia RL. Advances and challenges in developing a therapy for spinal muscular atrophy. Expert Rev Neurother. 2015;15(8)895-908.
4. Finkel RS, et al. Observational study of spinal muscular atrophy type I and implications for clinical trials. Neurology. 2014;83(9):810-817.
5. Govoni A et al. Time Is Motor Neuron: Therapeutic Window and Its Correlation with Pathogenetic Mechanisms in Spinal Muscular Atrophy. Mol Neurobiol. 2018 Aug;55(8):6307-6318

List of links present in page

1. https://prod1.novartis.ca/ca-fr/ca-fr/en/news/media-releases/novartis-applauds-quebec-first-province-list-zolgensma-treatment-pediatric-patients-spinal-muscular-atrophy-sma
2. https://prod1.novartis.ca/ca-fr/ca-fr/en/home
3. mailto:camlph.communications@novartis.com
4. https://rarediseases.org/rare-diseases/spinal-muscular-atrophy/