



Press Release

Ontario grants broader access to SPINRAZA™ (nusinersen) for patients living with spinal muscular atrophy (SMA)

- Access to the first and only approved treatment for pre-symptomatic, Type I, II, and III SMA patients under the age of 18
- Ontario joins Saskatchewan and Quebec to give patients with SMA broader access to SPINRAZA™

Mississauga, Ontario, Canada, June 13, 2019 – Biogen Canada is pleased to announce that patients in Ontario living with spinal muscular atrophy (SMA) now have broader publicly funded access to SPINRAZA™ (nusinersen), the first and only treatment approved in Canada for SMA.

This announcement follows the decision in November 2018 to provide publicly funded access for those living with Type I SMA. Ontario has expanded coverage for SPINRAZA™ to include the following patients:

- Patients who are pre-symptomatic with two or three copies of the survival motor neuron 2 (SMN2) gene;
- Patients with disease duration of less than six months, two copies of the SMN2 gene, and symptom onset after the first week after birth and on or before seven months of age;
- Patients under the age of 18 with symptom onset after six months of age, who have never achieved the ability to walk independently;
- Other Type II and III patients regardless of ever achieving the ability to walk independently are encouraged to apply for access through their clinicians to be considered on a case by case basis;
- Treatment for adult patients can be approved exceptionally on a case by case basis.

“We welcome the decision by the Ontario government to grant broader access to SPINRAZA™,” said Marina Vasiliou, Vice President and Managing Director of Biogen Canada. “We believe that all SMA patients, including adults, should have broad access and we will continue to work with all jurisdictions until this is achieved in Canada.”

The Ontario government decision follows the decisions of Quebec, Saskatchewan, Non-Insured Health Benefits (NIHB) and more than 44 countries around the world who grant broad reimbursement to SPINRAZA™.

The Health Canada approval of SPINRAZA™ in June 2017 for all pre-symptomatic, type I, II and III SMA patients regardless of age is based on Biogen’s extensive clinical development program for this treatment. Biogen continues to partner with the medical community in Canada and worldwide for the ongoing evaluation of the long-term effectiveness of this treatment in SMA.

Reaction from the Canadian SMA community

Toronto pediatric neurologist Dr. Jiri Vajsar welcomes the news for young SMA patients: “This is very good news. We feel that broad access to this disease-modifying therapy for SMA is critical and will give more patients and families dealing with the disease hope to slow the natural progression of the disease and improve or at least maintain their motor control, strength, function and independence. This is ultimately what we aspire to achieve for all SMA patients across the spectrum of the disease.”

London pediatric neurologist, Dr. Craig Campbell is optimistic about the Ontario government decision: “The decision to expand nusinersen access in Ontario is an important, thoughtful and positive decision for the SMA community. It will allow a wide range of people with SMA to realize the substantial benefits of this medication. As an SMA community this allows us to make sure nusinersen gets to those who need it now, and collectively document the long-term real world data to further demonstrate the positive impact of nusinersen.”

Toronto neurologist Dr. Aaron Izenberg recognizes the significance of what this means for adults living with SMA: “SPINRAZA™ is currently the only approved disease-modifying treatment for SMA. I am pleased with the Ontario government’s decision to include access for adults on a case by case basis.”

Susi Vander Wyk, Executive Director, Cure SMA Canada: “We have been hoping this day would come and are thrilled that the Ontario government has recognized the need for SPINRAZA™ to be accessible more broadly. We welcome this step forward and urge all other jurisdictions to ensure this needed treatment is readily accessible to all patients across Canada who can benefit.”

Barbara Stead-Coyle, CEO, Muscular Dystrophy Canada: “SMA is a devastating and debilitating disease for the individual and the families. We are pleased with the Ontario government’s decision and will continue our work to advocate for expanded access for all patients living with SMA, as the evidence supporting SPINRAZA™ shows that treatment can have a meaningful impact across all forms of the disease.”

About Spinal Muscular Atrophy (SMA)

SMA is a rare, debilitating neurodegenerative condition that is characterized by loss of motor neurons in the spinal cord and lower brain stem, resulting in severe and progressive muscular atrophy and weakness.

SMA is the leading genetic cause of death among infants. It is estimated that 1 in 10,000 live births are affected by SMA. Untreated, children with the most severe form of SMA rarely live to see their second birthday.

Due to a deletion of, or mutation in, the SMN1 gene, people with SMA do not produce enough survival motor neuron (SMN) protein, which is critical for the maintenance of motor neurons. The severity of SMA correlates with the amount of SMN protein an individual has. Ultimately, individuals with SMA can become paralyzed and have difficulty performing the basic functions of life, like breathing and swallowing.

People with Type I (infantile-onset) SMA, the form that requires the most intensive and supportive care, produce very little SMN protein and do not achieve the ability to sit without support or typically live beyond two years

without respiratory support. People with Type II and Type III SMA produce greater amounts of SMN protein and have less severe, but still life-altering forms of SMA.

About SPINRAZA™ (nusinersen)

SPINRAZA™ (nusinersen) is the first and only approved treatment in Canada for spinal muscular atrophy (SMA). It is the only SMA therapy with a well-established safety and durability profile and is supported by the largest clinical data set and real-world evidence, with more than 7,500 patients treated worldwide. Approved in more than 44 countries, it has been shown to improve survival for those with the most severe form of SMA (infantile-onset) and has enabled children, teens and adults with SMA to maintain or improve motor function, transforming the course of the disease.

About Biogen

At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies. One of the world's first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann, Heinz Schaller, Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp, and today has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, and is focused on advancing neuroscience research programs in MS and neuroimmunology, Alzheimer's disease and dementia, movement disorders, neuromuscular disorders, acute neurology, neurocognitive disorders, pain, and ophthalmology. Biogen also commercializes biosimilars of advanced biologics.

We routinely post information that may be important to investors on our website at www.biogen.ca. To learn more, please visit www.biogen.ca and follow us on social media – [Twitter](#), [LinkedIn](#), [Facebook](#), [YouTube](#).

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