Novartis Gene Therapies Recommits to Global Managed Access Program for 2021 [1]

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Dear SMA community,

One year ago we launched a global Managed Access Program (MAP) [2], the first for a one-time gene therapy, to make Zolgensma® (onasemnogene abeparvovec) available to eligible patients with spinal muscular atrophy (SMA) who are under the age of two in countries where Zolgensma has not received regulatory approval. Today we are pleased to share that we allocated 100 doses free-of-charge in 2020, and have renewed the program with up to 100 doses planned in 2021.

Through the global MAP in 2020, we are honored to have provided free access to this life-changing therapy to so many SMA families across nearly every continent – Africa, Asia, Australia, Europe, North America and South America – and spanning 24 countries.*

“The global Managed Access Program was established to help address some of the global need for treatment of babies with SMA. We are pleased to report that we have been able to deliver Zolgensma to 24 new countries to date in a way that allowed us to manage our supply constraints and other regulatory and logistical hurdles, while navigating a global pandemic. In fact, more than 80 percent of doses were delivered to babies in all corners of the world after the pandemic started, including the first-ever SMA gene therapy administration in countries such as Chile, Mexico, Tunisia, Belarus, India, Malaysia, United Arab Emirates and Vietnam,” said David Lennon, President of Novartis Gene Therapies. “The introduction of the program was not without challenges, but through the partnership and critical input from the patient advocacy community, we refined the MAP to better deliver against its foundational principles of fairness, clinical need and global accessibility. We are pleased to reaffirm our commitment to continuing the program in 2021 so that children around the world can benefit from SMA treatment who otherwise would not have access to Zolgensma.”

To date, our collective efforts in the US and globally have enabled approximately 170 babies and children around the world to receive Zolgensma free-of-charge. This is a significant percentage of the more than 700 patients in total who have received Zolgensma to date.

Zolgensma is now approved in 37 countries, with additional approvals expected in Switzerland, Australia, Argentina and South Korea in the first half of 2021. We remain committed to broadening access to Zolgensma, including via innovative programs that enable and incentivize early reimbursement, such as our Day One Access initiative in Europe, as well as by working closely with advocacy partners to accelerate newborn screening.

The global MAP has been informed, enabled and improved through collaboration with the

global SMA community, and the success of the program in its inaugural year is the result of your interest, insights and partnership. We thank you for your support and look forward to furthering our commitment to ensure that as many children as possible around the world can benefit from our innovative gene therapy.

Sincerely,
The Novartis Gene Therapies Team

Links
[1] https://www.novartis.com/news/novartis-gene-therapies-recommits-global-managed-access-program-2021
[2] https://www.novartis.com/our-company/novartis-pharmaceuticals/novartis-gene-therapies/novartis-gene-therapies-managed-access-program