

2021 Global Managed Access Program Community Update

Jan 24, 2022

Dear SMA community,

In early 2020, we launched a global Managed Access Program (MAP), the first for a one-time gene therapy, to make Zolgensma® (onasemnogene abeparvovec) available to eligible patients with spinal muscular atrophy (SMA) in countries where it has not received regulatory approval or formal access approval. To date, our efforts have enabled nearly 250 babies and children across 24 regions and countries to receive Zolgensma free-of-charge – a meaningful portion of the more than 1,600 patients treated with our gene therapy to-date. We are honored to provide free access to this life-changing therapy to so many families. Today, we are proud to announce we have renewed the global MAP program with up to 100 doses again planned in 2022.

This program benefits patients and families who otherwise would not have access to our gene therapy or other SMA treatments and is a particular point of pride for our organization. As the MAP continues to evolve, we will partner with the community to strategically review the program's scope and consider additional ways in which it may be improved in the future.

"The SMA community remains at the heart of Novartis' mission to reimagine medicine," said Shephard Mpofu, Chief Medical Officer, Novartis Gene Therapies. "The continuation of the Zolgensma global MAP, especially in the face of the ongoing pandemic, is just one part of our commitment to ensuring babies and young children who may benefit from this transformative therapy are able to do so."

Zolgensma is now approved in 42 countries around the world, with additional approvals expected in several markets in 2022. We remain committed to broadening access to Zolgensma as quickly as possible, and to continuing to work closely with local governments and payors to craft innovative access solutions that allow early reimbursement.

The Zolgensma global MAP has successfully reached families in nearly every continent thanks to the collaboration and insights we have gained from the global SMA community. We achieve the best results when we work together. We thank you for your ongoing partnership and look forward to seeing the future results of our shared endeavors.

Sincerely,

The Novartis Gene Therapies Team

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