Zolgensma Global Managed Access Program in 2023

Jan 09, 2023

Since it first launched, we have worked to broaden global access to Zolgensma® (onasemnogene abeparvovec), closely collaborating with local governments and payors to craft innovative access solutions that enable eligible spinal muscular atrophy (SMA) patients to obtain the gene therapy.

Along with these innovative access solutions, we also launched in early 2020 a global Managed Access Program (gMAP), the first for a one-time gene therapy. Through that program, which has been available in countries where Zolgensma has not yet received approval or no access pathway exists, along with our previous US managed access program, we have provided nearly 300 children in 36 countries with our gene therapy free of charge.

During this time, the global SMA treatment landscape has progressed, with three treatments now approved. So too, has our work in obtaining sustainable access to Zolgensma – now approved in 45 countries, with more than 2 500 patients treated globally across clinical trials, managed access programs, and in the commercial setting.

With these advancements in mind, we are redefining the geographic scope of the global Managed Access Program to countries where it is currently possible to make Zolgensma available as a future long-term sustainable solution. These changes, which we believe will ultimately benefit more patients over time, will take effect January 9, 2023.

We recognize this decision will be disappointing to patient families in countries no longer eligible. It is important to note that we will honor our commitment to patients already enrolled in the program, who will continue in the program as long as they remain medically eligible under the gMAP. While we don't anticipate adding additional countries in the future as the program will naturally conclude upon obtaining remaining health authority approvals in the countries where global Managed Access Program remains open, we will continue to work with stakeholders in exploring all alternative potential access solutions for interested countries.

We remain committed to the SMA community and will continue to advance clinical trials and innovative access solutions so the full SMA community may benefit from our transformative gene therapy.