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Community Statement from Novartis Gene Therapies

Dear SMA Community,

Novartis Gene Therapies is committed to working with our partners in the SMA community to make a difference in the diagnosis, treatment, and care of those affected by SMA. We would like to announce that we will initiate SMART, a Phase 3b clinical study to further evaluate the safety and efficacy of Zolgensma® (onasemnogene abeparvovec) in patients with spinal muscular atrophy (SMA) weighing ≥ 8.5 kg and ≤ 21 kg, following a single intravenous (IV) infusion. Clinical data from this study will supplement emerging real-world evidence and use of onasemnogene abeparvovec.

The global study is expected to enroll 24 symptomatic children with SMA from sites in Europe, North America, Australia and Taiwan, and will follow patients for a period of 12 months. All study site locations are still being finalized, with enrollment anticipated to start in September 2021. We expect only a few patients in each country to be enrolled and anticipate enrollment to be filled quickly. Recruitment will be highly targeted to meet the enrollment criteria and led by the individual sites. Physicians and caregivers looking for more information about the SMART study should refer to https://clinicaltrials.gov/ct2/show/NCT04851873 or contact the Novartis Medical Information team at medinfo.gtx@novartis.com.

We continue to invest in new research and development programs to better serve the SMA community. We thank you for your engagement and will continue to keep you informed of our progress.

Sincerely,
Your Novartis Gene Therapies Team

About the SMART trial

- SMART is a Phase 3b, open-label, single-arm, multicenter study designed to further evaluate the safety, tolerability and efficacy of a single IV infusion of onasemnogene abeparvovec in patients who have symptomatic SMA with bi-allelic mutations in the *SMN1* gene and any copy number of the *SMN2* gene and weigh ≥ 8.5 kg and ≤ 21 kg.

- The global study is expected to enroll 24 patients and will follow participants for a period of 12 months. After study completion, participants will be invited to enroll into a long-term, follow-up study to collect additional safety and efficacy data.

Questions & Answers

1. **What is the purpose of the SMART study?**
   The SMART study will further expand the clinical evidence beyond the patient population studied in clinical trials conducted by Novartis Gene Therapies to date.

2. **How can patient families enroll in the study? Where can they go for more information?**
   All site locations are still being finalized and we expect to have additional details to share towards the middle of this year. It is important to note that enrollment is not anticipated to start until September 2021. Enrollment will be for 24 symptomatic children across sites in Europe, North America, Australia and Taiwan. As such, we expect only a few patients in each country to be enrolled and anticipate enrollment to be filled quickly. Recruitment will be highly targeted to meet the enrollment criteria and led by the individual sites. Physicians and caregivers looking for more information about the SMART study should refer to
   https://clinicaltrials.gov/ct2/show/NCT04851873
   or contact the Novartis Medical Information team at medinfo.gtx@novartis.com.

3. **Why is this study limited to 24 patients?**
   It is important to keep in mind this is a clinical study designed to extend the clinical evidence beyond the patient population studied in onasemnogene abeparvovec trials conducted by Novartis Gene Therapies to date. The purpose of the study is to get additional data on the use of onasemnogene abeparvovec, in a timely fashion, which then can be used to inform physicians and caregivers.

   We remain unwavering in our commitment to advancing global access for babies and children with SMA, but the SMART study is not designed as an access pathway for onasemnogene abeparvovec. Currently, onasemnogene abeparvovec is approved in 39 countries and regions around the world and we continue to collaborate with all stakeholders, including health authorities and reimbursement bodies to enable access to onasemnogene abeparvovec for babies and children with SMA. Earlier this year, we also reaffirmed our commitment to continue the global Managed Access Program (MAP) with up to 100 doses planned in 2021 to make onasemnogene abeparvovec available to eligible patients with SMA who are under the age of two in countries where onasemnogene abeparvovec has not received regulatory approval. More information about MAP can be found here.

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