SMA Community Update from Novartis Gene Therapies, Region Europe
September 2022

Dear SMA Community:

All of us at Novartis Gene Therapies are looking forward to this year's 3rd International Scientific Congress on Spinal Muscular Atrophy (SMA) that will take place in Barcelona, Spain this coming October. Novartis Gene Therapies will host a side event about Pediatric Physiotherapy and Rehabilitation on Thursday, October 20th in the afternoon. We will also host the symposium " Exploring patient care in the era of newborn screening (NBS)" in the morning of Saturday, October 22nd. We are really looking forward to hosting these events as we appreciate the SMA community's insights, partnership, and continued engagement.

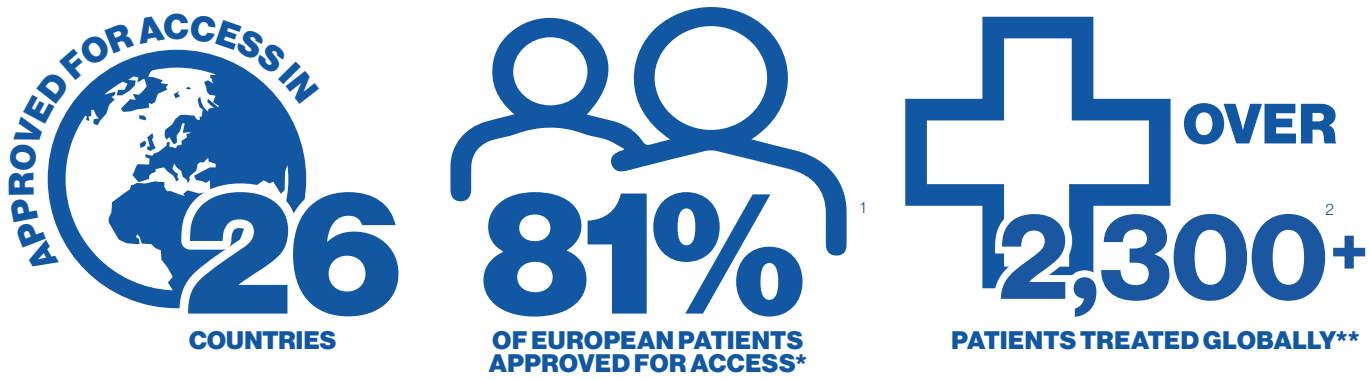
Below, we are sharing with you the most updated information about our clinical trials, long-term studies, and some recent publications.

We continue to be inspired by the work you do to drive care and treatment for patients with SMA.

With gratitude,

YOUR NOVARTIS GENE THERAPIES TEAM

European Access Snapshot for onasemnogene abeparvovec, for intravenous infusion:



*Covered via formal access or early access pathway
**As of June 2022, including clinical trials, commercially, and through the managed access programs

SMA Clinical Trial Program

Completed Clinical Studies

START

Phase 1 study that evaluated safety and efficacy of onasemnogene abeparvovec in symptomatic patients

STR1VE

Phase 3 study that evaluated safety and efficacy of onasemnogene abeparvovec in symptomatic patients

SPR1NT

Phase 3 study that evaluated safety and efficacy of onasemnogene abeparvovec in presymptomatic patients

STRONG

Phase 1 study that evaluated safety and tolerability of investigational intrathecal gene therapy (OAV101)

SMA Clinical Trial Program

Investigational Studies

SMART

SMART is a Phase 3b clinical study to further evaluate safety, tolerability, and efficacy of intravenous onasemnogene abeparvovec in patients with SMA weighing ≥8.5 kg and ≤21 kg. The global study has completed enrollment of 24 symptomatic children at SMA sites in Europe, North America, Australia and Taiwan, and will follow patients for a period of 12 months. For the latest information, please visit clinicaltrials.gov.

STRENGTH

STRENGTH is a global, Phase 3b, open-label study to evaluate safety and tolerability of our investigational intrathecal gene therapy (OAV101) in patients aged 2-12 years with SMA after discontinuing treatment with nusinersen or risdiplam. The study is not yet recruiting. For the latest information, please visit clinicaltrials.gov.

STEER

STEER is a global Phase 3 clinical study of our investigational intrathecal gene therapy (OAV101) in patients with type 2 SMA. The STEER trial will include treatment naive patients aged 2 to <18 years, able to sit, but who have never walked. Recruitment has begun. For the latest information, please visit clinicaltrials.gov.

Long-Term Follow-Up Studies

Three long-term follow-up studies to monitor safety and efficacy in participants from Phase 1 and 3 OAV101 intravenous (IV) and intrathecal (IT) clinical trials

LT-001
for participants from START

LT-002
for participants from IV and IT clinical trials

A12308
for participants from IV and IT clinical trials

New Webpage for the STEER Clinical Trial

Information on the study, including study design, endpoints and eligibility criteria, can be found on **Novartis.com**.

Recently Published Data

Nature Medicine
Published the SPR1NT trial of Onasemnogene abeparvovec in presymptomatic infants with two copies of *SMN2*.[3]

Nature Medicine
Published the SPR1NT trial of onasemnogene abeparvovec in presymptomatic infants with three copies of *SMN2*.[4]

Pediatric Neurology
Published a paper about safety of onasemnogene abeparvovec for patients with SMA who were 8.5 kg or heavier in a global managed access program.[5]

References

1. Data on file. Novartis Gene Therapies, Region Europe, 2022. **2.** Data on file. Novartis Gene Therapies, Inc. 2022. **3.** Strauss KA, Farrar MA, Muntoni F, et al. Onasemnogene abeparvovec for presymptomatic infants with two copies of *SMN2* at risk for spinal muscular atrophy type 1: the Phase III SPR1NT trial. *Nat Med.* 2022;28:1381-1389. **4.** Strauss KA, Farrar MA, Muntoni F, et al. Onasemnogene abeparvovec for presymptomatic infants with three copies of *SMN2* at risk for spinal muscular atrophy: the Phase III SPR1NT trial. *Nat Med.* 2022;28:1390-1397. **5.** Chand DH, Mitchell S, Sun R, LaMarca N, Reyna SP, Sutter T. Safety of onasemnogene abeparvovec for patients with spinal muscular atrophy 8.5 kg or heavier in a global managed access program. *Pediatr Neurol.* 2022;132:27–32.

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