SMA Community Update from Novartis Gene Therapies:
June 2022

Dear SMA Community:

The past few months have continued to show the strength of the Spinal Muscular Atrophy (SMA) community: from Rare Disease Day, where the SMA community joined its voice with other rare disease communities to raise awareness, to region-wide support to SMA patients and families in war-torn Ukraine. This highlights important topics for the SMA community: Raising awareness & supporting its community.

In this community update, we share the latest educational resources and provide clinical updates.
We thank all for your dedication and commitment to the SMA community. The work you do to drive care and treatment for patients with SMA continues to inspire us.

With gratitude,

YOUR NOVARTIS GENE THERAPIES TEAM

Connecting with the Community

Raising awareness has been key to much of the work we strive to do. We joined voices with patients in Rare disease Day to bring attention and generate change to the 300 million people living with rare diseased worldwide. SMA Europe coordinated a relief effort and we were committed to help. Novartis Gene Therapies donated 15,000 Euros to SMA Europe to assist SMA patients needing to leave Ukraine.

Gene Therapy Education Resources

Several new resources are available on our Explore Gene Therapy website to help you better understand cell and gene therapy including a fact sheet; information about gene addition, inhibition and editing; and details about the next era of medicine with cell and gene therapies. Take a look and share with your communities to encourage meaningful conversations about the potential of gene therapy.

Recently Published Data

Drug Safety published a paper that described the safety data for onasemnogene abeparvovec.[1]

Nature Medicine published a paper on the biodistribution of onasemnogene abeparvovec.[2]

Access Snapshot for onasemnogene abeparvovec suspension, for intravenous infusion

APPROVED

FOR ACCESS IN OVER 24 EUROPEAN COUNTRIES

82%[3] **OF THE EUROPEAN POPULATION HAS APPROVED ACCESS***

* covered via formal access or early access pathway

OVER 2,000+[4] **PATIENTS TREATED GLOBALLY****

** As of March 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)

Long-Term Follow-Up Studies

LT-001

Monitoring ongoing safety in START study patients

LT-002

Evaluating long-term safety and efficacy in patients from clinical trials for SMA who were treated with onasemnogene abeparvovec

SMA Clinical Trial Program

Ongoing/Upcoming Studies

SMART

SMART is a Phase 3b clinical study to further evaluate the safety, tolerability, and efficacy of intravenous onasemnogene abeparvovec in patients with SMA weighing ≥8.5 kg and ≤21 kg. The global study has completed recruitment and is expected to enroll 24 symptomatic children with SMA across 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.

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.

STRENGTH

STRENGTH is a global, Phase 3b, open-label study to evaluate the safety and tolerability of our investigational intrathecal gene therapy (OAV101) in patients aged 2-12 years with SMA after discontinuing treatment with nusinersen and/or risdiplam. The study is early in development and specific details including the study protocol are still ongoing.

Intravenous (IV) is a method of administering medicines directly into the vein. **Intrathecal administration (IT)** is a method of administering medicines into the spinal canal so that it reaches the **cerebrospinal fluid (CSF)**.

References

1. Day JW, Mendell JR, Mercuri E, et al. Clinical Trial and Postmarketing Safety of Onasemnogene Abeparvovec Therapy. *Drug Saf*. 2021;44:1109–1119. doi: 10.1007/s40264-021-01107-6.
2. Thomsen G, Burghes AHM, Hsieh C, et al. Biodistribution of onasemnogene abeparvovec DNA, mRNA and SMN protein in human tissue. *Nat Med*. 2021;27:1701-1711.
3. Data on file as of May 2022. Novartis Gene Therapies, EMEA. 2022.
4. Data on file. Novartis Gene Therapies, Inc. 2022.

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