LMI070 (branaplam) in Spinal Muscular Atrophy (SMA)

Update on behalf of the branaplam program team

Dear SMA Community,

As part of our ongoing commitment to communicate more transparently and openly with you as we progress with the development program for branaplam, we wanted to provide you with a short update on the program.

The development of branaplam is important to Novartis and we continue our efforts to bring it to patients. Branaplam, formerly known as LMI070, is an oral treatment under investigation to treat Spinal Muscular Atrophy (SMA), currently in a Phase 1/2 clinical trial (safety and efficacy) in Type 1 SMA. This trial is for untreated Type 1 SMA patients who are less than 6 months of age.

In total, 13 patients with Type I SMA were enrolled in the first part of the Phase 1/2 trial across 6 sites in Belgium, Denmark, Germany and Italy, and 8 patients continue to receive branaplam as of today. For the second part of the trial, we are very happy to announce that we are expanding the number of countries and sites. Specifically, we are about to initiate sites into three new countries by end of June 2018. Other countries and sites are in our plans and should follow soon; however, we prefer to wait to announce them until we have received formal health authority approval and formal approval from each site before making any announcement. Physicians in other European countries have also reached out to us to participate, which is extremely encouraging. Recruiting continues at all of our existing trial sites. Site selection and initiation in the US is also ongoing.

We are pleased to announce that we recently reached an important milestone: On March 23, we received the written positive opinion from the European Commission confirming Orphan Drug Designation for branaplam. This follows the Orphan Drug Designation from the US FDA, which was granted on January 25.

The granting of orphan status in the US and EU is intended to support development of medicines for the treatment of rare diseases (less than 5 in 10,000 in the EU). It provides companies certain benefits to encourage the continued development of medicines, like branaplam, that treat small numbers of patients with severe diseases.

Novartis continues to be very active in broader scientific research for SMA. We introduced a potential biomarker to assess disease severity at the SMA Europe International Scientific Conference in Krakow, Poland. As we communicated at the conference, this biomarker seems to be elevated in Type 1 SMA patients as compared to healthy children. We are continuing to explore this biomarker and its potential in SMA.

We continue to work to share information and talk regularly with the SMA community. Novartis recently launched our Novartis Commitment to Patients and Caregivers. Co-created with over 40 patient organizations, this is a powerful statement that the company commits to embed patient perspectives in its daily work and champion the patient voice in internal discussions and decision making.

Based on this, we look forward to continuing our ongoing constructive dialogue with you – not only to keep you up to date on progress with branaplam, but also to get your insights and feedback on how we can improve our clinical trials and outcomes for SMA patients.

Best regards,

The branaplam team

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