LMI070 (branaplam) in Spinal Muscular Atrophy (SMA)

Update on behalf of the branaplam program team

Dear SMA community

For years, our researchers have been working hard to develop a new treatment for SMA. One promising compound in our neuroscience pipeline is LMI070, which started development for the treatment of SMA a few years back. In 2016, we had to pause enrollment in our first clinical study CLMI070X2201, in type 1 SMA, because signs of nerve injury appeared in some animal tests.

Since then, we have been working together with experts in the field to understand these findings better. We are now pleased to share some good news with you: we are expanding the clinical investigation of this important molecule, which will now be called *branaplam*.

Effective immediately, following the respective Health Authorities and Ethics Committee approvals, we are resuming enrollment in the ongoing study in type 1 SMA (CLMI070X2201) at our existing sites in Belgium, Germany, Denmark and Italy. CLMI070X2201 is an open-label study (all patients assigned to branaplam) where different doses are being tested in patients with type 1 SMA who are younger than 6 months of age. You can find more information about the ongoing clinical trial available on our clinical trials website, on clinicaltrials.gov and the EU Clinical Trials Register.

Now that recruitment has resumed, we are seeking to expand to additional sites and countries. We will keep you updated as new sites open.

Specifically for patients in the US, we can also share that we have recently opened an Investigational New Drug (IND) application with the FDA. This means we are allowed to enroll US patients in the study. We will notify the SMA community as US sites open.

Beyond resuming recruitment, we have also made some changes to the way the study is being conducted. As a reminder, branaplam is designed to be orally administered. From now on, patients will have the option to have the weekly drug dose administered orally rather than via a feeding tube only. We made this change – and others – in response to feedback from patients' families and investigators. We sincerely hope they will ease some of the burden of participating in the trial.

The well-being of every patient in the study is of paramount importance to us. Therefore, in order to monitor the safety of enrolled patients, additional nerve tests were added to the study protocol.

In parallel, we are also working closely with regulatory authorities to define the best way to expand branaplam clinical trial program beyond type 1 SMA and make the trials available for patients as quickly as possible. Recently the US FDA granted a "fast-track"[1] designation in type 1 SMA. It is still a long road, but we are pleased to be back on track.

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We believe that the best way to bring a product that is most beneficial to SMA patients and their families is by working in close partnership with you. So, moving forward, we will make sure that the SMA community is informed regularly as development for branaplam advances.

We would like to thank all of you who are or who will be participating in the study for branaplam, and those of you who support them – your contribution to bringing new and innovative treatments for SMA patients is invaluable.

Best regards,

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Reference

1. https://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm