

December 16, 2019

**Community update:
Status of ongoing study of branaplam/LMI070 in SMA**

Dear SMA Community

To ensure you have the latest information about branaplam's development program, we are pleased to provide this update on our Phase 1/2 study in Type 1 infantile-onset SMA. The study is progressing well, with a total of 29 infants receiving the study treatment. Infants who were enrolled into Part 1 of the study have been receiving branaplam for between approximately 4 and 5 years.

Earlier this year we were able to share the good news that Part 2 of the study had achieved its recruitment target. Participants in Part 2 have been on study treatment for between 7 and 19 months.

Part 3 of the study is assessing the long-term safety, tolerability and effectiveness of extended treatment with branaplam. Infants participating in Parts 1 and 2 who have received branaplam for at least 52 weeks are eligible for this extension study.

We anticipate being able to share the 12-month data from Part 2 of the study in the second half of next year (2020). This data will help define branaplam's potential in SMA and inform future development options.

We would again like to thank the parents who have children in this study for their courage and commitment. What we are learning about this weekly oral investigational treatment is only possible because of you and your children. We are also grateful to the whole SMA community for your continued support and generous advice.

We look forward to sharing more updates through our patient advocacy partners and at next year's conferences, starting with SMA Europe's 2nd International Scientific Congress in February.

As the year draws to a close, we wish you a joyful festive season with family and friends.

Yours sincerely,

The branaplam team