

15 May 2019

**External message to SMA patient community: SMA EUROPE**

Dear Marie Christine,  
Dear Mencia,

We are writing to inform you that enrollment to the ongoing branaplam clinical study is now closed.

Branaplam is currently being assessed for safety and efficacy in a Phase 1/2 clinical study in Type 1 infantile-onset SMA. We are pleased to announce that we have enrolled 25 infants into Part 2 of the study. This is in addition to the 7 (out of 13 infants) who continue to receive branaplam in Part 1 of the study – exposure to treatment ranges from 3 to 4 years. Enrollment to the study is now closed.

The data from this Phase 1/2 study will inform the options we consider for future studies. We are grateful for the dedication of the investigators and their teams who are conducting this study, caring for the enrolled infants and gathering the data.

In addition, we would like to express our genuine gratitude to all the parents who have considered participation in this study for their children. We truly appreciate the commitment you have made. We would also like to thank the SMA community for your advice and support that has helped us achieve this important stage in branaplam's development program.

Novartis remains committed to our ongoing open communication with the SMA community, as well as to progressing science-based innovation to improve outcomes for SMA patients. We look forward to continuing dialogue with you – not only to keep you up to date on progress with branaplam, but also to gain your insights and feedback on how we can improve our clinical studies and outcomes for SMA patients.

Regards,  
The branaplam team

