

July 21, 2021

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

Dear SMA Community:

To ensure you have the latest information about branaplam's development program, we are providing this update. Novartis has made the decision to discontinue development of branaplam, an investigational oral, once-weekly RNA splicing modulator, for the treatment of spinal muscular atrophy (SMA). This was a difficult decision that was made as the result of rapid advancements in the SMA treatment landscape in recent years.

Knowing the progressive nature of this devastating, genetic disease, maintaining continuity of care for all children enrolled in the branaplam SMA clinical trial is our top priority. As this decision was not driven by the safety or efficacy of branaplam, we encourage patients to continue branaplam treatment until an alternate solution is arranged for ongoing care. In partnership with trial investigators, we will ensure treatment solutions are determined on a case-by-case basis, tailored to the individual needs of each child. We sincerely thank the SMA community for their support as well as the families who enrolled their children in our clinical trial.

Currently there are three approved treatments available for SMA, including two RNA splicing modulators and our own, one-time gene therapy. The wide-spread availability of these treatments impacts the feasibility of conducting a registration-enabling clinical trial for branaplam in this small patient population. We also recognize that branaplam would not offer a highly differentiated treatment solution for the SMA community. We are continuing to advance the investigation of branaplam for Huntington's Disease, a rare, genetic disease without any approved, disease-modifying therapies.

Novartis remains unwavering in our commitment to use science-based innovation to improve outcomes for SMA patients. We would again like to thank the parents who have children in this study for their courage and commitment. We understand that participating in any study is one made with both heart and mind and we are truly thankful for the dedication. We are also grateful to the whole SMA community for your continued support and generous advice.

Yours sincerely,

The branaplam team