

October 2019

BioMarin statement on the out-licensing of tralesenidase alfa program (BMN 250) for Mucopolysaccharidosis Type IIIB (MPS IIIB, Sanfilippo Syndrome Type B)

In 2015, BioMarin launched a clinical trial program in ten countries around the world to evaluate tralesenidase alfa as a potential treatment for Sanfilippo Syndrome Type B. Since that time, the clinical program has made significant progress: many children now receive tralesenidase alfa in the fully-enrolled treatment studies, while a natural history study continues to enroll and deliver valuable insights into Sanfilippo Syndrome Type B disease manifestations and progression. Tralesenidase alfa remains under investigation and has not been determined to be safe or effective or approved for use.

On October 23rd, BioMarin announced its decision to out-license this program to Allievex. Allievex is a clinical stage biotechnology company developing novel therapies for the treatment of rare pediatric neurodegenerative diseases.

All clinical studies in the tralesenidase alfa program have been transferred to Allievex and will continue uninterrupted. All study participants in the treatment studies continue to receive tralesenidase alfa to evaluate the safety and efficacy as a potential treatment. BioMarin will provide necessary technical support and guidance throughout the transfer process to support a smooth transition. BioMarin will transfer full responsibility to Allievex for all clinical development activities, which we anticipate will be complete by end of the year.

BioMarin believes in the continued research of tralesenidase alfa as a potential treatment for Sanfilippo Syndrome Type B. We also believe Allievex can bring even more concentrated focus to continuing the research of tralesenidase alfa for the treatment of this rare and devastating disease.

BioMarin remains dedicated to advancing the care of the rare disease communities we serve through the use of our approved therapies.

BioMarin thanks all of those who have contributed to the research of tralesenidase alfa for their commitment, strength and dedication: our investigator teams, trial support staff, contract research organization partners, advocates, and most of all the families and children who have been part of this development program.

If you have questions regarding this statement, please contact your local BioMarin contact. Alternatively, you can also reach out to BioMarin Medical Information: Medinfo@biomarin.com.