BIOMARIN UPDATE FOR THE POMPE COMMUNITY: 22 JUNE 2016

BioMarin greatly appreciates the many patients, families, and physicians who have participated in our Pompe program, as well as your ongoing support of the entire patient community. After much deliberation, BioMarin has decided to discontinue the clinical development of the BMN 701 (reveglucosidase alfa) Pompe program. However, BioMarin remains open to external opportunities for the development of this compound. This decision is not based on concerns for patients' safety or efficacy.

Does my doctor know about this decision?
Yes, your study doctor has been made aware of this decision.

What will happen to patients currently receiving drug in the clinical trials?
BioMarin plans to stop providing investigational drug to study sites within the month of July.

What are patients' options when the trial is stopped at their study site?
All options regarding present and future treatment should first be discussed with your physician. In contrast to many other rare diseases, there is an approved treatment option for Pompe disease. As with any treatment option, this should be discussed with your physician.

What about compassionate use?
BioMarin's policy for treatment under compassionate use requires that the drug be under development. Since BioMarin's plans are to discontinue development of BMN 701, we are not planning a compassionate use program at this time.

Who is the best source for additional information?
Your physician remains the best source of support and information for you and your family. You should contact them with any questions you have regarding treatment options. BioMarin remains in close contact with all investigators and study teams and will provide updates when new information becomes available.