



For Immediate Release

July 4, 2016

As the International Pompe Association (IPA) has previously announced, on Thursday, June 9<sup>th</sup>, Biomarin Pharmaceuticals stated it was planning to out-license the further development of BMN-701 during Goldman Sachs 37<sup>th</sup> Annual Global Healthcare Conference. BMN-701 (also known as Reveglucosidase alfa) is an investigational enzyme replacement therapy for Pompe disease that is currently in Phase II/III trials. The IPA was disappointed to learn of this development, but remained hopeful for the continuation of the program.

Unfortunately, this announcement was followed by a Community Update from Biomarin on Wednesday, June 22<sup>nd</sup> that announced that Biomarin was discontinuing “the clinical development of the BMN 701 (reveglucosidase alfa) Pompe program.” (<http://worldpompe.org/index.php/news/591-biomarin-update-for-the-pompe-community>). Biomarin has stated: “This decision is not based on concerns for patients’ safety or efficacy.”

The IPA Board appreciates that many factors must have gone into Biomarin’s decision to discontinue its Pompe program. Clinical development of a treatment is difficult in the best of circumstances; it is even more difficult in the rare disease field. The IPA believes that the Pompe community is fortunate to have as much interest in developing new therapies and treatments as we do. Many rare diseases do not have any treatments on the horizon.

However, the IPA also believes that the development of a treatment is, and should be, a community effort. It takes close collaboration between the scientific/medical community and the patient community to understand the disease in question, and how to treat it. Then, when the time is right, it takes industry to move potential treatments from the scientist’s laboratory to the patient in the form of clinical trials and market approvals. However, successful clinical development of a therapy is impossible without the contribution and collaboration of all three parties in our experience.

The Board of the IPA is disappointed in Biomarin’s decision to discontinue its Pompe program. We are disappointed that a potential new therapeutic option will not be pursued, but more than that we are disappointed with the way the global patient community and the patients in the trial were informed about this development.

In our opinion, industry members have a moral and ethical obligation to be transparent and forthright with the patients in their trials. Depending on the nature of the trial, when patients agree to participate in clinical trials they may be agreeing to regular travel commitments, and invasive and non-invasive testing. Travel for a healthy person can be difficult, but for a patient already burdened by illness, it takes an additional toll. That has not stopped the Pompe patient community from being willing and enthusiastic participants in numerous studies—including the various studies and trials conducted by Biomarin.

The IPA is disappointed that despite the patient community’s commitment to participating in the clinical development of Biomarin’s Pompe program they were given minimal to no information about the fate of the program for nearly two weeks after the announcement was made that Biomarin was seeking to out-license its program. Further, once information was

made available it was only to say that the program was being stopped in the immediate future.

We can't help but feel that this information could have, and should have, been communicated in a different and more respectful manner. Ultimately, the patient should be the priority in any communication strategy, as it is the patient's life that is the most affected by decisions related to clinical development of treatments—especially in a progressive, and potentially fatal disease like Pompe. Going forward, we will continue to work with all industry and continue to advocate that the patient's access to timely, transparent, and accurate information is a priority.

In the coming weeks and months, the IPA remains committed to supporting the Pompe community. If the discontinuation of Biomarin's clinical program is directly affecting you or a family member, please contact us if we can be of assistance ([info@worldpompe.org](mailto:info@worldpompe.org)).

The IPA Board