July 24, 2024

Dear Parents, Families, Children, Caregivers and Clinicians,

As you are aware, Allievex terminated its business operations in October 2023, to include all clinical studies treating children with Sanfilippo syndrome type B with our enzyme replacement therapy, tralesinidase alfa (AX 250). At the same time, we laid off all seven employees as we sought a buyer for our program. During this time, the former employees of Allievex worked tirelessly to ensure that we were able to initiate investigator sponsored treatments of some children who were previously receiving treatment in our clinical program. However, these treatments are dependent on the remaining drug product supply, which is very limited and will be exhausted before the end of this year. Yesterday, Allievex entered an Assignment for the Benefit of Creditors under the supervision of the Chancery Court of Delaware. This is ultimately a formal liquidation of our assets to pay our creditors.

Since 2019, the lack of alignment with the U.S. Food and Drug Administration on a development pathway towards an Accelerated Approval left the company with very little options to raise the necessary capital or to be acquired. On March 15, 2024, former Allievex employees met with the FDA, and the Center for Drug Evaluation and Research completely changed course and encouraged Allievex to file its Biologics License Application for consideration under Accelerated Approval Pathway. I credit the pragmatism of Dr. Patrizia Cavazzoni, the Director of CDER, in this change at FDA. While this late breaking good news was not enough to allow us to solve our business situation, we do take great pride in our advocacy at FDA and your commitment to our clinical trials. Together, we have now enabled multiple other sponsored programs in development in MPS disorders to move rapidly forward to potential Accelerated Approval for diseases like MPS I, MPS II and MPS III. You have seen these recent announcements from companies like Denali, Ultragenyx and RegenxBio.

Former Allievex employees continue to support the marketing of our AX 250 program to potential acquirors during this process. It is important that we maintain the institutional knowledge of this complex program to adequately transfer to a new company. To give you a sense of scale of what is needed, we believe it will take $60 million to $75 million of new investment to allow for a submission and review of AX 250 for approval under Accelerated Approval. Almost all this investment will focus on manufacturing and starting a new confirmatory clinical study prior to filing for approval. To date, we have already invested $72 million in the program. But now, there is a path forward for a potential acquiror where we did not have one.

I will continue to communicate with the patient foundations as we go through this process. Know that we are devastated by this outcome, and we have done everything we possibly can to preserve the potential of AX 250 to be accessible by all MPS IIIB patients worldwide.

God bless all of you, and especially all our Sanfilippo children.

Respectfully,

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