An Open Letter to the Pompe Community

On October 28, Amicus Therapeutics issued a press release with an update on the progress of our Pompe program as we received information from the US Food & Drug Administration (FDA) on the regulatory status of our next-generation therapy for the treatment of late-onset Pompe disease. The entire Pompe community - from individuals and families to patient advocacy organizations and their leaders - are our valued partners and we want to share this information with you all within the context of the regulatory activities' timeline.

- Amicus submitted a Biologics License Application (BLA) for cipaglucosidase alfa and a New Drug Application (NDA) for miglustat in 2021. The original Prescription Drug User Fee Act (PDUFA) action dates for the NDA were May 29, 2022, and July 29, 2022, for the BLA.
- As announced in May 2022, the PDUFA action dates for miglustat and cipaglucosidase alfa were amended to August 29 and October 29, 2022, respectively, to allow additional time for the FDA to complete the routine pre-license approval inspections necessary at the WuXi Biologics manufacturing site in WuXi, China.
- As stated in this most recent press release, the FDA was unable to conduct the inspection of the WuXi Biologics manufacturing facility within the review period due to China's COVID-19 travel restrictions.

As a result, the FDA is deferring action on the applications until the manufacturing site inspection is complete. Amended anticipated action date(s) have not been provided as the agency monitors the public health situation and travel restrictions in China. Nevertheless, and rest assured, Amicus is actively engaged with the FDA on developing a plan for the pre-approval inspection.

Please note that access to AT-GAA continues for participants in any ongoing clinical studies, such as the open-label extension studies ATB200-02 and ATB200-03, and for anyone currently receiving AT-GAA through an expanded access program in the United States or elsewhere.

All of Team Amicus is deeply grateful to the entire Pompe community for your ongoing commitment to support and participate in clinical research. Even under the best of circumstances, let alone during a global pandemic, clinical research for rare diseases is a challenging endeavor that requires collaborative efforts to explore and advance new treatment options for people living with serious and life-threatening conditions. Staying involved and fulfilling the many requirements of clinical studies calls for sacrifice and selflessness from study participants, their families and support systems. As the ripple effects of COVID-19 continue to alter the way we approach our daily life, perseverance is essential to carry research initiatives forward.

If anyone in the community would like to review this information or has any questions, the Patient & Professional Advocacy team is available. To arrange a call, please email patientadvocacy@amicusrx.com, or call 1-866-9-AMICUS (926-4287) toll-free in the US or +1.609-662.2000. Please do not hesitate to reach out. We are happy to help.

Most sincerely,

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Chief Patient Advocate

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