



PTC Therapeutics, Inc.
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September 30, 2026

Dear Fabry Disease Community,

For over 25 years, PTC Therapeutics has focused on bringing innovative and meaningful therapies to children and adults affected by rare diseases. Today, we are proud to be able to provide six different approved therapies, including a gene therapy, to individuals in more than 50 countries around the world. We are excited to introduce ourselves to the Fabry community as we work to gain approval for the investigational gene therapy ST-920 (isargalgagene civaparvovec), following its recent acquisition from Sangamo Therapeutics.

At the heart of everything we do at PTC is an unwavering commitment to patients and families. We are guided and inspired by the experiences of those living with rare conditions and by the belief that meaningful progress is built through partnership with the communities. We do not take lightly the responsibility of advancing therapies and supporting patients and families who continue to navigate significant challenges.

Over the coming weeks, our teams will focus on the work necessary to complete the submission of the ST-920 approval application to the FDA. We expect the application submission to be completed before the end of 2026. We are excited by the opportunity to bring a one-time administered, safe and effective disease treatment option to the Fabry community. In addition, we will be assessing the potential for approval applications in other countries.

We look forward to meeting with the community and learning from your experiences. We will keep you updated as the FDA application process progresses. If you have any questions, please feel free to reach out to our patient engagement team at patientengagement@ptcbio.com.