PTC to Expand Rare Disease Portfolio with Acquisition of BLA-Stage ST-920 Fabry Disease Program

August 12, 2026

– Planned acquisition leverages existing regulatory and commercial infrastructure and leadership's experience in Fabry therapy commercialization –

– ST-920 is a one-time administered AAV gene therapy for the enzyme deficient in Fabry disease with demonstrated long-term clinical benefits and safety profile –

– BLA submission expected to be completed in Q4 2026 with potential for commercial launch in 2027 –

– PTC will host a conference call today, Aug. 12, at 5 p.m. ET –

WARREN, N.J., Aug. 12, 2026 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced that it was selected as the winning bidder to acquire ST-920 – a BLA-stage one-time administered AAV gene therapy for Fabry disease – from Sangamo Therapeutics in a competitive bankruptcy auction. The terms include $111 million upfront and up to $100 million in contingent milestone payments based on certain regulatory approvals. A rolling BLA submission to FDA for accelerated approval of ST-920 is expected to be completed in Q4 2026. The BLA is based on evidence of meaningful favorable clinical effect on renal function and safety and tolerability profile over 52 weeks in the Phase 1/2 STAAR study.

"This transaction advances our strategy of leveraging our accomplished existing rare disease global commercial infrastructure to accelerate short- and intermediate-term revenue growth," said Matthew B. Klein, M.D., Chief Executive Officer. "The ST-920 gene therapy program puts another innovative and valuable product in the demonstrated capable hands of our customer-facing teams. This was a unique opportunity with the potential for significant return on investment without the need for any development or commercial build and without impacting our objective of reaching cashflow break even in 2026. We look forward to working to bring ST-920 to all individuals who may benefit from this therapy as quickly as possible."

Fabry disease is a rare, inherited lysosomal storage disorder caused by mutations in the GLA gene, resulting in deficiency of the alpha-galactosidase A (α-Gal A) enzyme and causing a range of serious signs and symptoms that require lifelong treatments. It is estimated that there are 11,000 people living with Fabry disease in the United States with similar prevalence rates in markets where PTC has the potential to commercialize.

ST-920 is designed as a one-time administered AAV gene therapy that enables long-term production of the deficient α-Gal A enzyme and significant reduction in globotriaosylceramide (Gb3) levels with demonstrated durable clinical benefit and reduction of the burden associated with chronic Enzyme Replacement Therapy (ERT). ST-920 has received Regenerative Medicine Advanced Therapy (RMAT) designation as well as Orphan Drug and Fast Track designations from FDA.

The Phase 1/2 STAAR study demonstrated positive mean annualized estimated glomerular filtration rate (eGFR) slope at 52 weeks following ST-920 administration, as well as evidence of favorable effect on other aspects of Fabry disease including cardiac function and quality of life. The finding of improved eGFR over 52 weeks is differentiated from other Fabry therapies which demonstrated improved renal function but still negative eGFR slope from baseline. Furthermore, all study participants on ERT at study start were withdrawn from ERT. Durability of effect has been demonstrated with sustained increased α-Gal A activity maintained for up to 4.5 years for the earliest treated study participant, and evidence of maintained improvements in renal function across the study population. In addition, ST-920 has demonstrated an encouraging safety and tolerability profile and there is no requirement for routine prophylactic or post-infusion systemic immunosuppressive agents.

The BLA submission for accelerated approval is based on the intermediate clinical endpoint of annualized eGFR at Week 52 as aligned with FDA, with 104-week results from the STAAR study planned to provide confirmatory evidence to support traditional approval. The nonclinical and clinical BLA modules have already been submitted as part of a rolling submission, with the CMC package expected to be submitted in Q4 2026. PTC will also pursue regulatory approval outside of the United States, again leveraging existing regulatory and commercial rare disease infrastructure.

The acquisition remains subject to definitive documentation, bankruptcy court approval, antitrust review, and other customary closing conditions. It is expected to close in late Q3 or early Q4 2026.

Conference Call and Webcast Details
PTC will hold a conference call today at 5 p.m. ET to discuss this news. To access the live webcast, please visit Events & Presentations within the Investors section of the PTC website. A replay of the webcast will be available on the PTC website for 30 days following the event. To participate via phone, please register in advance here to receive dial-in details.

About the STAAR Study
The Phase 1/2 STAAR study was a global open-label, single-dose, dose-ranging, multicenter clinical study designed to evaluate isaralgagene civaparvovec, or ST-920, a gene therapy product candidate in patients with Fabry disease. Isaralgagene civaparvovec requires a one-time infusion without preconditioning. The STAAR study enrolled patients who were on ERT, were ERT pseudo-naïve (defined as having been off ERT for six or more months), or who were ERT-naïve. The FDA has granted Orphan Drug, Fast Track, and RMAT designations to isaralgagene civaparvovec, which has also received Orphan Medicinal Product designation and PRIME eligibility from the European Medicines Agency and Innovative Licensing and Access Pathway from the U.K. Medicines and Healthcare products Regulatory Agency.

About Fabry Disease
Fabry disease is a lysosomal storage disorder caused by mutations in the galactosidase alpha gene (GLA), which leads to deficient alpha-galactosidase A (α-Gal A) enzyme activity, which is necessary for metabolizing globotriaosylceramide (Gb3). The buildup of Gb3 in the cells can cause serious damage to vital organs, including the kidney, heart, nerves, eyes, gut and skin. Symptoms of Fabry disease can include decreased or absent sweat production, heat intolerance, angiokeratoma (skin blemishes), vision problems, kidney disease, heart failure, gastrointestinal disturbance, mood disorders, neuropathic pain and tingling in the extremities.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company dedicated to the discovery, development and commercialization of clinically differentiated medicines for children and adults living with rare disorders. PTC is advancing a robust and diversified pipeline of transformative medicines as part of its mission to provide access to best-in-class treatments for patients with unmet medical needs. The company's strategy is to leverage its scientific expertise and global commercial infrastructure to optimize value for patients and other stakeholders. To learn more about PTC, please visit www.ptcbio.com and follow us on LinkedIn, X, Facebook and Instagram.

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Forward-Looking Statements
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including the Company's expectations regarding the proposed acquisition, including the expectation of finalizing definitive documentation for the transaction and the entry of an bankruptcy court order approving the transaction; the Company's ability to complete the acquisition; the anticipated benefits of ST-920; the timing of and potential for regulatory submissions and potential commercial launch for ST-920, if acquired; and PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance," "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," "aim," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: uncertainty surrounding the bankruptcy's court entry of an order approving the acquisition and the possibility that the acquisition is not completed; the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to Sephience, including commercialization and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in geographies in which it has been approved and the effect of the European Commission's adoption of the negative opinion from the Committee for Medicinal Products for Human Use (CHMP) on Translarna and the withdrawal of the Translarna NDA in the US on other regulatory bodies; expectations with respect to PTC's license and collaboration agreement with Novartis Pharmaceuticals Corporation for votoplam for the treatment of Huntington's disease including its right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis, the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses, including potential accelerated approval; expectations with respect to Upstaza/Kebilidi, including commercialization, manufacturing capabilities, and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to vatiquinone, including with respect to the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses and potential approvals and other matters; expectations with respect to the commercialization of Evrysdi under PTC's SMA collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; expectations regarding PTC's product candidates, including the timing of clinical trials and studies; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of its lease agreements; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's Annual Report on Form 10-K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

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