



PTC Therapeutics Enters into Agreement to Sell Priority Review Voucher for \$150 Million

November 27, 2024

WARREN, N.J., Nov. 27, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that it has entered into an agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV) for \$150 million upon the closing of the transaction.

PTC was granted the PRV on November 13, 2024 along with the U.S. Food and Drug Administration (FDA) approval of KEBILIDI™ (eladocagene exuparvec-tneq) for the treatment of children and adults with AADC deficiency, including the full spectrum of disease severity.

The transaction is subject to customary closing conditions, including expiration of the applicable waiting period under the Hart-Scott Rodino (HSR) Antitrust Improvements Act.

About the Rare Pediatric Disease Priority Review Voucher Program

The FDA awards priority review vouchers to sponsors of rare pediatric disease product applications that meet certain criteria. The program is intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A PRV can be redeemed to receive priority review of a subsequent marketing application for a different product, or it can be sold or transferred.

About PTC Therapeutics, Inc.

PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to children and adults living with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and X.

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Forward-Looking Statement:

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 statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected closing of the transaction and any expectations with respect to the timing for such closing and HSR clearance of the transaction and the timing of any such clearance; 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", 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: 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; 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; and the factors discussed in the "Risk Factors" section of PTC's most recent 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.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful.

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.

