PTC Therapeutics Announces Strategic Pipeline Prioritization

May 23, 2023

- Preclinical and early research gene therapy programs discontinued -
- Expected reductions of approximately 15% in residual 2023 OPEX -

SOUTH PLAINFIELD, N.J., May 23, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today the discontinuation of preclinical and early research programs in gene therapy as part of a strategic portfolio prioritization. PTC will continue its development and global commercialization of Upstaza, the first-ever approved gene therapy directly administered to the brain. As a result of the prioritization, estimated reductions of approximately fifteen percent in residual 2023 operating expenses (OPEX) are expected. Additional 2023 OPEX guidance will be provided as part of second quarter earnings.

"We are incredibly proud of PTC's accomplishments in the field of gene therapy, including the pioneering work associated with the approval of Upstaza. We believe that the decision to discontinue our pipeline gene therapy programs enables PTC to focus R&D efforts on our other innovative and differentiated scientific platforms and strongly positions us for long-term growth and success," said Chief Executive Officer Dr. Matthew Klein. "Where possible, we will work to ensure that the discontinued gene therapy programs can be developed by other parties so that the therapies have the potential to benefit patients."

The decision to deprioritize early-stage gene therapy programs reflects the company's desire to focus resources on areas of the pipeline likely to deliver significant return on investment and transformative therapies for patients with high unmet medical need. The discontinuation of the gene therapy programs will also result in a reduction in workforce which will be conducted in accordance with appropriate labor regulations.

The discontinued gene therapy programs include preclinical stage programs in Friedreich ataxia and Angelman syndrome as well as several other programs targeting rare CNS and ophthalmological disorders of high unmet medical need at various stages of preclinical development. The prioritization decision will allow for additional focus on PTC's proprietary splicing platform as well as additional CNS and metabolic disorders that leverage its differentiated and innovative scientific expertise.

In addition, PTC Therapeutics also announced today that PTC's Chief Financial Officer Emily Hill has been relieved of her responsibilities and will be leaving the organization.

Conference Call and Webcast Details:
PTC will hold a conference call at 5:00 pm EDT today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About PTC Therapeutics, Inc.
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients 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 Twitter at @PTCBio.

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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 press release, other than statements of historic fact, are forward-looking statements, including statements regarding the extent, timing and financial aspects of the discontinuation of our preclinical and early research programs in gene therapy and reduction in workforce; the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters, future 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; the timing of and actual expenses incurred in connection with the discontinuation of our preclinical and early research programs in gene therapy and reduction in workforce, which may be in different periods and may be materially higher than we estimate; the savings that may result from the discontinuation of our preclinical and early research programs in gene therapy and reduction in workforce, which may be materially less than we expect; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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; 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 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.

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