# Preliminary Results Presented at ASCO Demonstrated Promising Clinical Efficacy with Unesbulin in Leiomyosarcoma Study

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*- Results highlight strength of PTC Therapeutics' scientific platform in targeting difficult-to-treat rare cancer types -*

SOUTH PLAINFIELD, N.J., June 3, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced encouraging preliminary safety and efficacy results from its Phase 1B study of unesbulin (PTC596) in advanced leiomyosarcoma (LMS) patients. The results demonstrated that treated patients achieved an 18.2 percent objective response rate and a 51.5 percent disease control rate. In addition, unesbulin was generally well tolerated. The results from the dose escalation study which evaluated unesbulin in combination with dacarbazine (DTIC), will be presented on Saturday, June 4, during the Sarcoma Oral Abstract Session beginning at 1:15 p.m. CDT at the 2022 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago.

"The results demonstrated with unesbulin in LMS patients are very promising," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "Leiomyosarcoma is an aggressive soft tissue sarcoma that has significant high unmet medical need, especially for those patients who have relapsed or are refractory to current treatments. We are excited to bring a new chemical entity to the fight against cancer."

The primary objectives of the study were to determine the recommended Phase 2 dose (RP2D) of unesbulin in combination with DTIC and to characterize the safety profile of the combination. In addition, the secondary objectives included progression free survival and duration of response. Unesbulin 300 mg taken twice a week in combination with DTIC 1,000 mg/m$^2$ every 21 days was established as the RP2D.

"Leiomyosarcoma is a particularly aggressive tumor type that desperately requires new treatment options for patients," said Brian Van Tine, M.D., Ph.D., Sarcoma Program, Washington University and lead clinical investigator. "The preliminary results demonstrate promising early efficacy in patients with advanced leiomyosarcoma."

Based on the preliminary data from the Phase 1B study, PTC has initiated the SUNRISE LMS study. SUNRISE LMS is an international, placebo-controlled, registration-directed study comparing the efficacy and safety of unesbulin and DTIC versus placebo and DTIC in patients with advanced LMS who have received ≥1 prior line of systemic therapy. The study is ongoing (ClinicalTrials.gov identifier: NCT05269355).

## About Unesbulin (PTC596)

Unesbulin is an investigational oral tubulin binding agent that arrests tumor cells in G2/M phase, including cancer stem cells, through the action of inhibiting tubulin polymerization. Unesbulin was discovered through PTC's proprietary discovery platform.

## About Leiomyosarcoma

Leiomyosarcoma (LMS) is a rare and aggressive cancer found in smooth muscle tissue. LMS is one of the more aggressive sarcoma subtypes representing 10 to 28 percent[1] of all soft tissue sarcomas and has a high risk of recurrence leading to decreased disease-specific survival.[2] Approximately 4,000 patients are diagnosed each year in the United States and there is a 2:1 incidence in females compared to males.[3]

## 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 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 timing of clinical trials and studies, availability of data, regulatory submissions and responses, licensing or commercialization of its products and products candidates and other matters; 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; 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.

[1] Toro 2006, Ducimetière 2011, Ferrari 2011, Friedman 2018, Nagar 2018, Parikh 2018, Saltus 2018, Bessen 2019

[2] Pisters 1996, Svarvar 2007, Wang 2011, Gladdy 2013, Miettinen 2014, Worhunsky 2015, George 2018

[3] FDA Updates Highlighting the Latest Cancer Treatment. *Onc Times.* 2020;42:88–89. 5. The Liddy Shriver Sarcoma Institute. Leiomyosarcoma of the Bone and Soft Tissue

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