Protara Therapeutics Announces Dosing of First Patients in Phase 1b ADVANCED-1EXP Trial of TARA-002 in NMIBC Patients with Carcinoma in Situ

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- *Dosing of first patients follows recent presentation of positive preliminary data from Phase 1a dose escalation portion of ADVANCED-1 trial*

- *Company advancing NMIBC clinical development program, with Phase 1b/2 trial in BCG-unresponsive CIS patients and in BCG-naïve and BCG-exposed CIS patients expected to initiate in 2H'2023*

NEW YORK, May 15, 2023 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced that patient dosing is now underway in its Phase 1b ADVANCED-1EXP expansion trial evaluating TARA-002, the Company's investigational cell-based therapy, for the treatment of patients with high-grade non-muscle invasive bladder cancer (NMIBC) who have carcinoma in situ (CIS).

"Following positive preliminary results from the ADVANCED-1 trial, we are pleased to have dosed the first patients in our ADVANCED-1EXP trial," said Jathin Bandari, M.D., Chief Medical Officer of Protara Therapeutics. "We believe TARA-002 has the potential to play a meaningful role in the NMIBC treatment landscape, as there continues to be limited treatment options for this highly recurrent disease. We look forward to gaining additional clinical experience with TARA-002 in NMIBC patients with CIS."

ADVANCED-1EXP is a Phase 1b open-label expansion trial, which is evaluating intravesical TARA-002 at the 40KE[1] dose in 12 CIS patients, including Bacillus Calmette-Guérin (BCG)-naïve, BCG-unresponsive and BCG-inadequately treated patients. Trial participants will receive six once-weekly intravesical instillations of TARA-002. The primary objective of the trial is to evaluate the safety, tolerability and signs of anti-tumor activity of TARA-002, and the planned primary endpoint is the complete response (CR) rate at three months.

In April 2023 at the American Urological Association Annual Meeting, the Company announced positive preliminary results from the Phase 1a dose-escalation component of the ADVANCED-1 clinical trial of TARA-002 for the treatment of patients with high-grade NMIBC. The clinical data indicate that TARA-002 was generally well tolerated and anti-tumor activity was observed, including tumor regression in all three evaluable patients with CIS, including one heavily pre-treated BCG-unresponsive patient who achieved a CR.

Based on these results, Protara plans to initiate ADVANCED-2, a Phase 1b/2 open-label trial evaluating intravesical TARA-002 in up to 102 patients with CIS. The Phase 1b trial is expected to enroll 27 patients with CIS (± Ta/T1), BCG-Naïve or BCG-exposed who have not received intravesical BCG for at least 24 months prior to CIS diagnosis. The Phase 2 trial is expected to enroll 75 patients with BCG-unresponsive CIS (± Ta/T1). ADVANCED-2 is expected to initiate in the second half of 2023.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of NMIBC and of LMs for which it has been granted Rare Pediatric Disease Designation by the U.S. Food and Drug Administration (FDA). TARA-002 was developed from the same master cell bank of genetically distinct group A *Streptococcus pyogenes* as OK-432, a broad immunopotentiator marketed as Picibanil® in Japan and approved in Taiwan by Chugai Pharmaceutical Co., Ltd. Protara has successfully shown manufacturing comparability between TARA-002 and OK-432.

When TARA-002 is administered, it is hypothesized that innate and adaptive immune cells within the cyst or tumor are activated and produce a strong immune cascade. Neutrophils, monocytes and lymphocytes infiltrate the abnormal cells and various cytokines, including interleukins IL-2, IL-6, IL-8, IL-10, IL-12, interferon (IFN)-gamma, and tumor necrosis factor (TNF)-alpha are secreted by immune cells to induce a strong inflammatory reaction and destroy the abnormal cells.

About Non-Muscle Invasive Bladder Cancer

Bladder cancer is the 6th most common cancer in the United States, with NMIBC representing approximately 80% of bladder cancer diagnoses. Approximately 65,000 patients are diagnosed with NMIBC in the United States each year. NMIBC is cancer found in the tissue that lines the inner surface of the bladder that has not spread into the bladder muscle.

About Protara Therapeutics, Inc.

Protara is committed to advancing transformative therapies for people with cancer and rare diseases. Protara's portfolio includes its lead program, TARA-002, an investigational cell-based therapy being developed for the treatment of non-muscle invasive bladder cancer and lymphatic malformations, and IV Choline Chloride, an investigational phospholipid substrate replacement for patients dependent on parenteral nutrition. For more information, visit www.protaratx.com.

Reference:

1. Klinische Einheit, or KE, is a German term indicating a specified weight of dried cells in a vial.

Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Protara may, in some cases, use terms such as “predicts,” “believes,” “potential,” “proposed,” “continue,” “designed,” “estimates,” “anticipates,” “expects,” “plans,” “intends,” “may,” “could,” “might,” “will,” “should” or other words or expressions referencing future events, conditions or circumstances that convey uncertainty of future events or outcomes to identify these forward-looking statements. Such forward-looking statements include but are not limited to, statements regarding Protara’s intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, Protara’s business strategy, including its development plans for its product candidates and plans regarding the timing or outcome of existing or future non-clinical studies and clinical trials, and statements regarding the anticipated safety or efficacy of Protara’s product candidates. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Factors that contribute to the uncertain nature of the forward-looking statements include: risks and uncertainties associated with: Protara’s development programs, including the initiation and completion of non-clinical studies and clinical trials and the timing of required filings with the FDA and other regulatory agencies; general market conditions; changes in the competitive landscape; changes in Protara’s strategic and commercial plans; Protara’s ability to obtain sufficient financing to fund its strategic plans and commercialization efforts; having to use cash in ways or on timing other than expected; the impact of market volatility on cash reserves; and the risks and uncertainties associated with Protara’s business and financial condition in general, including the risks and uncertainties described more fully under the caption "Risk Factors" and elsewhere in Protara's filings and reports with the United States Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. Protara undertakes no obligation to update any forward-looking statements, whether as a result of the receipt of new information, the occurrence of future events or otherwise, except as required by law.

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