Protara Therapeutics Announces Positive Preliminary Data from ADVANCED-1 Phase 1a Dose Escalation Trial of TARA-002 in NMIBC Supporting Advancement into Phase 2 Clinical Development

April 28, 2023

- *Favorable tolerability observed in patients with NMIBC treated with TARA-002 in ADVANCED-1 trial*
- *Anti-tumor activity was observed in all three evaluable patients with CIS, including one heavily pre-treated BCG-unresponsive patient who achieved a complete response*
- *Company's current resources are expected to fund operations into 2025 supporting the initiation of the ADVANCED-2 trial in BCG-naïve CIS patients and BCG-unresponsive CIS patients in 2H'2023*

NEW YORK, April 28, 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 positive preliminary results from the Phase 1a dose-escalation component of its ongoing ADVANCED-1 clinical trial of TARA-002, the Company's investigational cell-based therapy, for the treatment of patients with high-grade non-muscle invasive bladder cancer (NMIBC). The clinical data indicate that TARA-002, a novel intravesical monotherapy, was generally well tolerated and showed anti-tumor activity in high-grade NMIBC patients. The data will be featured during a moderated poster session at the American Urological Association (AUA) 2023 Annual Meeting being held in Chicago from April 28, 2023 to May 1, 2023.

"These promising results suggest TARA-002 may provide meaningful benefit to patients with NMIBC, who currently have limited treatment options," said Neal Shore, M.D., Medical Director, Carolina Urologic Research Center, Chief Medical Officer, GenesisCare US, and study investigator. "These data show favorable tolerability and initial evidence of anti-tumor activity, thus serving as an impetus to advance TARA-002 into larger, later-stage trials."

"We are highly encouraged by preliminary results from the dose-escalation component of ADVANCED-1 and look forward to deepening our understanding of TARA-002's potential in the ongoing expansion trial, which is currently enrolling NMIBC patients with carcinoma in situ (CIS)," said Jathin Bandari, M.D., Chief Medical Officer of Protara Therapeutics. "We plan to initiate larger clinical trials in NMIBC patients with CIS who are Bacillus Calmette-Guérin (BCG)-naïve and BCG-unresponsive in the second half of this year."

Preliminary Results

- TARA-002 was generally well tolerated at all three dose levels evaluated in the trial, and no dose limiting toxicities were observed. A maximum tolerated dose was not determined, and dose escalation remains ongoing in exploratory cohorts. The Company has selected the 40KE[1] dose for use in subsequent clinical trials.
- The majority of reported adverse events were Grades 1 and 2 across all dose levels, and treatment-related adverse events, as assessed by study investigators, were in line with typical responses to bacterial immunopotentiation, and included fatigue, headache, fever, and chills. The most common urinary symptoms were urinary urgency, urinary frequency, urinary tract pain/burning, incomplete emptying, and bladder spasm. Most bladder irritations resolved soon after administration or in a few hours to a few days.
- A total of nine patients were enrolled in the study, including three patients with CIS who reached the three-month efficacy assessment. Of those three patients with CIS, one heavily pre-treated BCG-unresponsive patient achieved a complete response (CR) at the 20KE dose, and tumor regression was observed in the other two patients.

A copy of the AUA poster will be available in the Events and Presentations section of the Company's website: https://ir.protaratx.com. The Company plans to present complete results from the ADVANCED-1 study at a subsequent medical conference.

Patient enrollment is ongoing in the open-label expansion trial (ADVANCED-1EXP), which is evaluating intravesical TARA-002 at the 40KE dose in 12 CIS patients, including BCG-naïve, BCG-unresponsive, and BCG-inadequately treated patients.

Clinical Development Plan Update

Based on these results, Protara is advancing the clinical development of TARA-002 for the treatment of NMIBC. The Company 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-experienced, 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 ADVANCED-1

ADVANCED-1 is a Phase 1 dose-finding, open-label trial (NCT05085977) evaluating TARA-002 in treatment-naïve and treatment-experienced NMIBC patients with carcinoma in situ (CIS) and high-grade papillary tumors (Ta). In the initial dose escalation phase of the trial, patients received six weekly intravesical doses of TARA-002 evaluating the 10KE, 20KE and 40KE doses. The primary objective of the trial was to evaluate the safety, tolerability

and preliminary signs of anti-tumor activity of TARA-002, with the goal of establishing a recommended dose for a future Phase 2 clinical trial.

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. 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 number 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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