Protara Therapeutics Announces Dosing of First Patient in Phase 1b/2 ADVANCED-2 Trial of TARA-002 in NMIBC Patients with High Grade Carcinoma in Situ

September 20, 2023

Dosing is progressing on schedule in ongoing ADVANCED-1EXP trial evaluating TARA-002 in NMIBC patients with CIS; preliminary results expected in 1H24

NEW YORK, Sept. 20, 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 dosing is now underway in its Phase 1b/2 ADVANCED-2 trial evaluating intravesical instillation of TARA-002, the Company's investigational cell-based therapy, for the treatment of high-grade non-muscle invasive bladder cancer (NMIBC) patients with Bacillus Calmette-Guérin (BCG)-naïve and BCG-unresponsive carcinoma in situ (CIS). In addition, dosing is progressing in the ongoing ADVANCED-EXP trial started earlier this year with preliminary results expected in the first half of 2024.

"The ADVANCED-2 trial in NMIBC is an exciting opportunity to build on the favorable anti-tumor and safety data of TARA-002 presented earlier this year," said Tom Jayram, M.D., Director, Advanced Therapeutics Center, Urology Associates, P.C., TN and study investigator for the ADVANCED-2 trial. "There is significant need for new intravesical approaches for NMIBC, which can recur and progress to invasive disease with current treatments. This trial looks at the potential clinical benefit of TARA-002 as a possible new therapy for NMIBC, across both BCG-naïve and BCG-unresponsive patient populations."

The initiation of this trial follows the positive results of the ADVANCED-1 clinical trial of TARA-002 presented in April 2023 at the American Urological Association Annual Meeting. In the dose-escalation component of that study, 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 complete response (CR).

"Following positive preliminary results from the ADVANCED-1 trial, we are pleased to have dosed the first patient in the ADVANCED-2 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 and look forward to progressing the trial in parallel with our ongoing ADVANCED-1EXP study."

ADVANCED-2 (NCT05951179) is a Phase 1b/2 open-label trial evaluating intravesical TARA-002 in up to 102 patients with CIS (± Ta/T1) who are BCG-naïve (N=27) and BCG-unresponsive (N=75). Trial subjects will receive an induction with or without a reinduction course of six weekly intravesical instillations of TARA-002, followed by a maintenance course of three weekly installations every three months in the BCG-unresponsive cohort.

The ongoing ADVANCED-1EXP trial is a Phase 1b open-label expansion trial evaluating intravesical TARA-002 in 12 CIS patients, including BCG-naïve, BCG-unresponsive, and BCG-inadequately treated patients.

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.

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 clinical trials; statements related to expectations regarding interactions with the FDA; Protara's financial position; statements regarding the anticipated safety or efficacy of Protara's product candidates; and Protara's outlook for the remainder of the year. 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 that Protara's financial guidance may not be as expected, as well as 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; the loss of key members of management; the impact of general U.S. and foreign, economic, industry, market, regulatory, political or public health conditions; 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.

Company Contact:

Justine O'Malley
Protara Therapeutics
Justine.OMalley@protaratx.com
646-817-2836

Source: Protara Therapeutics