Protara Announces Positive Results from the Ongoing Phase 2 ADVANCED-2 Trial of TARA-002 in Patients with NMIBC

December 5, 2024

- *TARA-002 demonstrates 72% six-month landmark complete response rate and 70% complete response rate at any time across BCG exposures*
- *100% six-month landmark complete response rate and 80% complete response rate at any time observed in BCG-Unresponsive patients*
- *64% six-month landmark complete response rate and 67% complete response rate at any time observed in BCG-Naïve patients*
- *80% reinduction salvage rate and compelling durability observed with 100% of patients maintaining a complete response from three months to six months across BCG exposures*
- *Favorable safety and tolerability profile with no Grade 2 or greater treatment-related adverse events*
- *Company to host conference call and webcast today at 8:30 a.m. ET*

NEW YORK, Dec. 05, 2024 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced results from its ongoing Phase 2 open-label ADVANCED-2 trial. The trial is assessing intravesical TARA-002, the Company's investigational cell-based therapy, in high-risk Non-Muscle Invasive Bladder Cancer (NMIBC) patients with carcinoma in situ or CIS (± Ta/T1) who are Bacillus Calmette-Guérin (BCG)-Unresponsive or BCG-Naïve. The complete response (CR) rate across BCG exposures was 72% (13/18) at six months and 70% (14/20) at any time with 100% (9/9) of patients maintaining a CR from three months to six months. In addition, two of three patients maintained a CR at nine months. These results will be featured today during a poster session at the 25th Annual Meeting of the Society of Urologic Oncology (SUO) in Dallas, Texas.

The dataset includes 20 patients who were evaluable at three months, 18 patients who were evaluable at six months and three patients who were evaluable at nine months with a data cutoff of November 19, 2024. In the pivotal cohort of the ADVANCED-2 trial in BCG-Unresponsive patients, the CR rate was 100% (4/4) at six-months and 80% (4/5) at any time. In the proof-of-concept cohort of BCG-Naïve patients, the CR rate was 64% (9/14) at six months and 67% (10/15) at any time. TARA-002 demonstrated a favorable safety and tolerability profile with no Grade 2 or greater treatment-related adverse events (TRAEs), and no patients discontinued due to adverse events.

"These impressive TARA-002 results demonstrate meaningful activity in a difficult to treat patient population," said Brian Mazzarella, MD, Vice President of Research for Urology America, and ADVANCED-2 study investigator. "The activity of TARA-002 across BCG exposures, coupled with its ease of use and low procedural burden for physicians, make it an exciting potential treatment option for NMIBC patients."

"We are thrilled with these positive six-month data, which reinforce TARA-002's potential in NMIBC, while offering a compelling product profile for physicians and patients," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We believe these encouraging data together with our international site expansion will accelerate patient enrollment, and we look forward to reporting initial data from 12-month evaluable patients in mid-2025."

The majority of adverse events were Grade 1 and transient with no Grade 2 or greater TRAEs as assessed by study investigators. No patients discontinued treatment due to adverse events. The most common adverse events were in line with typical responses to bacterial immunopotentiation, such as flu-like symptoms. The most common urinary symptoms reflect urinary tract instrumentation effects, such as bladder spasm, burning sensation, and urinary tract infection. Most bladder irritations resolved shortly after administration or within a few hours to a few days.

Conference Call and Webcast

Protara will host a conference call and webcast to discuss the data today at 8:30 am ET. The live call can be accessed by registering as a participant here. Upon registration, participants will receive conference call dial-in information. A live webcast of the event can be accessed by visiting the Events and Presentations section of the Company's website: https://ir.protaratx.com. The webcast will be archived for a limited time following the presentation.

About ADVANCED-2

The Phase 2 open-label ADVANCED-2 trial is assessing intravesical TARA-002 in NMIBC patients with carcinoma in situ or CIS (± Ta/T1) who are Bacillus Calmette-Guérin (BCG)-Unresponsive (n≈100) and BCG-Naïve (n=27). The BCG-Unresponsive cohort has been designed to be registrational in alignment with the FDA's 2024 BCG-Unresponsive Non-muscle Invasive Bladder Cancer: Developing Drugs and Biological Products for Treatment Draft Guidance for Industry.

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

pro-inflammatory response with release of cytokines such as tumor necrosis factor (TNF)-alpha, interferon (IFN)-gamma, IL-1b, IL-6, IL-12, granulocyte-macrophage colony-stimulating factor (GM-CSF) and natural killer cells. TARA-002 also directly kills tumor cells and triggers a host immune response by inducing immunogenic cell death, which further enhances the antitumor immune response.

About Non-Muscle Invasive Bladder Cancer (NMIBC)

Bladder cancer is the sixth 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 a clinical-stage biotechnology company committed to advancing transformative therapies for people with cancer and rare diseases. Protara's portfolio includes its lead candidate, TARA-002, an investigational cell-based therapy in development for the treatment of non-muscle invasive bladder cancer (NMIBC) and lymphatic malformations (LMs). The Company is evaluating TARA-002 in an ongoing Phase 2 trial in NMIBC patients with carcinoma in situ (CIS) who are unresponsive or naïve to treatment with Bacillus Calmette-Guérin (BCG), as well as a Phase 2 trial in pediatric patients with LMs. Additionally, Protara is developing IV Choline Chloride, an investigational phospholipid substrate replacement for patients on parenteral nutrition who are otherwise unable to meet their choline needs via oral or enteral routes. 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 (including reporting initial data from 12-month evaluable patients in mid-2025); 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 and future periods. 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; failure to attract and retain management and key personnel; 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.

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