Protara Therapeutics Announces Positive Interim Results Demonstrating Durable Responses in the Ongoing Phase 2 ADVANCED-2 Trial of TARA-002 in Patients with NMIBC

April 26, 2025

- *TARA-002 demonstrates 100% complete response rate at any time and 67% 12-month landmark complete response rate in BCG-Unresponsive patients*
- *TARA-002 demonstrates 76% complete response rate at any time and 43% 12-month landmark complete response rate in BCG-Naïve patients*
- *Favorable safety and tolerability profile with no Grade 3 or greater treatment-related adverse events*
- *On track to present updated interim data from approximately 25 six-month evaluable BCG-Unresponsive patients by the end of 2025*
- *Company to host conference call and webcast on Monday, April 28, 2025, at 8:30 a.m. ET*

NEW YORK, April 26, 2025 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced updated results from its ongoing Phase 2 open-label ADVANCED-2 trial 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 results will be featured today during an interactive poster session at the American Urological Association 2025 Annual Meeting in Las Vegas.

"For patients with high-risk NMIBC, there are few effective and durable therapies available other than radical cystectomy, which we know is quite difficult for patients to tolerate," said Tom Jayram, M.D., Director of the Advanced Therapeutics Center at Urology Associates, and ADVANCED-2 study investigator. "TARA-002 has shown impressive efficacy, safety profile, and 12-month durability in its Phase 2 trial. In the clinic, we have seen TARA-002 become easily integrated into workflow without major hurdles for the patients or staff. This combination of clinical activity and ease of use makes me optimistic about TARA-002 having a meaningful impact in clinical practice."

Interim Results

BCG-Unresponsive Cohort

The BCG-Unresponsive dataset includes a total of five patients, all of whom were six- and nine-month evaluable, and three of whom were evaluable at 12 months as of an April 16, 2025 data cutoff.

- The complete response (CR) rate at any time in BCG-Unresponsive patients was 100% (5/5).
- The CR rate in BCG-Unresponsive patients was 100% (5/5) at six months, 80% (4/5) at nine months, and 67% (2/3) at 12 months.

BCG-Naïve Cohort

The BCG-Naïve dataset includes a total of 21 patients, including 16 evaluable at six months, eight at nine months, and seven at 12 months as of an April 16, 2025 data cutoff.

- The CR rate at any time in BCG-Naïve patients was 76% (16/21).
- The CR rate in BCG-Naïve patients was 63% (10/16) at six months, 63% (5/8) at nine months, and 43% (3/7) at 12 months.

Safety

The majority of adverse events were Grade 1 and transient with no Grade 3 or greater treatment-related adverse events (TRAEs) as assessed by study investigators. No patients discontinued treatment due to TRAEs. 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.

"The durable results shared today continue to support our conviction that TARA-002 has the potential to make a meaningful difference in the lives of patients with NMIBC," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Notably, we are particularly pleased with the competitive 12-month CR rates observed in the registrational BCG-Unresponsive cohort as well as the BCG-Naïve cohort. We look forward to continuing to advance this important trial as we work toward our mission of bringing transformative therapies to patients."

The Company continues to expect to present an interim update with results from approximately 25 six-month evaluable BCG-Unresponsive patients by the end of 2025.

Conference Call and Webcast

Protara will host a conference call and webcast to discuss the data on Monday, April 28, 2025, 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

ADVANCED-2 (NCT05951179) is a Phase 2 open-label trial 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=31). The BCG-Unresponsive cohort has been designed to be registrational in alignment with the U.S. Food and Drug Administration'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 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-6, IL-10, IL-12. 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 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 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 data from approximately 25 6-month evaluable BCG-Unresponsive patients by the end of 2025); statements related to expectations regarding interactions with the U.S. Food and Drug Administration (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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