Protara Therapeutics Announces Presentation of Additional Encouraging Data from Phase 1a ADVANCED-1 Trial of TARA-002 in NMIBC at the 24th Annual Meeting of the Society of Urologic Oncology

November 30, 2023

- *New results from six patients with HGTa papillary tumors show five of six patients with HGRFS at Week 12*
- *Data continue to support favorable tolerability of TARA-002 in patients with NMIBC*
- *As previously reported, anti-tumor activity observed in all three evaluable patients with CIS, including one heavily pre-treated BCG-unresponsive patient who achieved a complete response*
- *Company remains on track to report preliminary results from the expansion portion of the ADVANCED-1 trial in NMIBC patients with CIS in the first half of 2024*

NEW YORK, Nov. 30, 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 additional encouraging data 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). In addition, the study design for its ADVANCED-2 trial will be presented. The data will be featured during a poster session at the 24th Annual Meeting of the Society of Urologic Oncology (SUO) being held in Washington, D.C. from November 28, 2023 through December 1, 2023.

"We are pleased to share additional data from the ADVANCED-1 trial which continue to support the potential for TARA-002 to provide a meaningful benefit to patients with NMIBC," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We look forward to reporting preliminary results from the expansion portion of the ADVANCED-1 trial of TARA-002, which we expect in the first half of 2024, and remain focused on continuing to execute on the totality of our NMIBC program, including the ongoing ADVANCED-2 trial."

Study Results

- TARA-002 was generally well tolerated at all three dose levels evaluated in the trial, and no dose limiting toxicities were observed. While a maximum tolerated dose was not determined, 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 carcinoma in situ (CIS) who reached the three-month efficacy assessment. Of those three patients with CIS, one heavily pre-treated Bacillus Calmette-Guérin (BCG)-unresponsive patient achieved a complete response (CR) at the 20KE dose, and tumor regression was observed in the other two patients.
- New results from six patients with high-grade, non-invasive papillary (HGTa) tumors showed five of six patients with high-grade recurrence free survival (HGRFS) at week 12. The patient who did not achieve HGRFS was dosed at 10KE, the lowest dose of TARA-002 offered in the trial.

The Company remains on track to report preliminary results from the expansion portion of the ADVANCED-1 trial in the first half of 2024.

Supported by data from the Phase 1a study, the Company commenced ADVANCED-2 (NCT05951179), a Phase 2 open-label trial evaluating intravesical TARA-002 in up to 102 NMIBC patients with CIS (± Ta/T1) who are BCG-naïve (n=27) and BCG-unresponsive (n=75). Trial subjects received 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. Additional details on the trial design will be featured in a Trial in Progress poster at the SUO meeting.

A copy of the SUO posters will be available in the Events and Presentations section of the Company's website: https://ir.protaratx.com.

About Non-Muscle Invasive Bladder Cancer (NMIBC)

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

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 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.

References

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 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.

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