Protara Highlights Recent Updates and Anticipated 2025 Milestones

January 13, 2025

Reported positive six-month data from ADVANCED-2 trial of TARA-002 in patients with NMIBC

Completed approximately $100 million public offering, extending runway into 2027

Initial data from 12-month evaluable NMIBC patients in ADVANCED-2 trial expected in mid-2025; Results from a futility analysis of approximately 25 six-month evaluable BCG-Unresponsive patients expected by the end of 2025

Dosing of first patient in THRIVE-3 registrational trial of IV Choline Chloride in patients dependent on parenteral support expected in 1H 2025

Results from additional cohorts of Phase 2 STARBORN-1 trial of TARA-002 in pediatric LMs patients expected by the end of 1H 2025

NEW YORK, Jan. 13, 2025 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today highlighted recent updates and anticipated 2025 milestones.

“Following a highly productive 2024 marked by positive data in our non-muscle invasive bladder cancer (NMIBC) program and with the funds from a successful financing, we are well positioned to accelerate our development programs and deliver on our mission to provide novel therapies to patients impacted by cancer and rare diseases,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “Looking ahead, we expect several key milestones in 2025, including reporting data on 12-month evaluable patients in our ADVANCED-2 trial of TARA-002 in NMIBC mid-year. On the heels of our recently reported positive interim data from six-month evaluable patients, we continue to believe that TARA-002 could represent a meaningful and differentiated addition to the NMIBC treatment paradigm with an attractive product profile for both physicians and patients.”

“In addition, we expect to begin the pivotal THRIVE-3 study of intravenous (IV) Choline Chloride in the first half of 2025. We also expect data from our ongoing Phase 2 STARBORN-1 trial of TARA-002 in lymphatic malformations (LMs) by the end of the first half of 2025.”

Recent Company Updates and Planned 2025 Milestones

TARA-002 in NMIBC

- In December 2024, the Company reported positive interim results from its ongoing Phase 2 open-label ADVANCED-2 trial in in NMIBC patients with carcinoma in situ or CIS (± Ta/T1) who are Bacillus Calmette-Guérin (BCG)-Unresponsive and BCG-Naïve at the 25th Annual Meeting of the Society of Urologic Oncology (SUO) in Dallas, Texas. 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. TARA-002 showed a favorable safety profile, with no Grade 2 or greater treatment-related adverse events and no treatment discontinuations due to adverse events.

- The Company expects to report data on 12-month evaluable patients in the ADVANCED-2 trial in mid-2025 and results from a futility analysis of approximately 25 six-month evaluable BCG-Unresponsive patients are expected by the end of 2025. As previously announced, the BCG-Unresponsive cohort is designed to be registrational in alignment with the 2024 BCG-Unresponsive Non-muscle Invasive Bladder Cancer: Developing Drugs and Biological Products for Treatment Draft Guidance for Industry issued by the U.S. Food and Drug Administration (FDA).

- The Company expects to provide an update on the design of its planned BCG-Naïve registrational trial by the end of the first half of 2025 following regulatory alignment.

- The Company continues to explore the administration of systemic priming dosing prior to initiation of intravesical administration, as well as combination therapy with TARA-002 in NMIBC patients with CIS. Given TARA-002’s mechanism of action and safety profile, the Company believes it has strong potential for use in combination therapy and is working to finalize various opportunities for the clinical program.

IV Choline Chloride for Patients on Parenteral Support (PS)

- The Company expects to commence the THRIVE-3 registrational trial of IV Choline Chloride, an investigational phospholipid substrate replacement, in adolescents and adults on long-term PS when oral or enteral nutrition is not possible, insufficient, or contraindicated, in the first half of 2025. IV Choline Chloride was previously granted Fast Track designation by the FDA as a source of choline for this patient population.

- In September 2024, the Company announced results from THRIVE-1, a prospective, observational study, which found that 78% of PS-dependent patients were choline deficient, and 63% of these patients demonstrated liver dysfunction, including steatosis, cholestasis, and hepatobiliary injury.

TARA-002 in LMs

- Protara remains on track to report initial results from additional cohorts in the Phase 2 STARBORN-1 trial of TARA-002 in pediatric patients with macrocystic and mixed cystic LMs by the end of the first half of 2025. The Company previously announced completion of the study's first safety cohort, in which TARA-002 demonstrated encouraging efficacy and was generally well-tolerated.

Financial Guidance

- The Company today provided updated financial guidance. Protara believes its approximately $81.5 million of cash, cash equivalents, and investments in marketable debt securities as of September 30, 2024, together with approximately $100 million gross proceeds from its December 2024 public offering, will be sufficient to fund its planned operations into 2027.

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 support 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.

Company Contact:

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

Source: Protara Therapeutics