Protara Therapeutics Announces Positive Interim Results Demonstrating Robust Responses in the Ongoing Phase 2 STARBORN-1 Trial of TARA-002 in Pediatric Patients with Lymphatic Malformations

November 19, 2025

- *80% of patients that completed treatment and 100% of patients that completed the eight-week response assessment achieved clinical success*
- *Clinical success achieved with one or two doses of TARA-002 in 88% of patients*
- *TARA-002 demonstrated favorable safety and tolerability profile with no serious adverse events reported*
- *Company to host conference call and webcast featuring Key Opinion Leader Dr. Jesse Jones at 8:30 a.m. ET*

NEW YORK, Nov. 19, 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 positive interim results from its ongoing Phase 2 open-label STARBORN-1 trial assessing intracystic injection of TARA-002, the Company's investigational cell-based therapy, in pediatric patients with macrocystic and mixed cystic lymphatic malformations (LMs).

"We are pleased to report these robust results from the STARBORN-1 trial that demonstrate TARA-002's expected significant clinical benefit in treating patients with macrocystic and mixed cystic LMs," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Treatment with TARA-002 resulted in clinically meaningful responses, with a favorable safety profile observed across all evaluable patients. The totality of available clinical data, including data from prior studies with TARA-002's predecessor compound OK-432, an established treatment for LMs in Japan, underscore our belief in the potential for TARA-002 to emerge as an important intervention for pediatric patients suffering from LMs."

"There are currently no approved therapies for LMs, with many patients turning to invasive surgical procedures that carry high rates of complication and recurrence, or off-label use of chemotherapies and chemicals, which can have challenging side effects, especially for pediatric patients," said Jesse G.A. Jones, M.D., Associate Professor, Department of Neurosurgery and Radiology, University of Alabama at Birmingham, and STARBORN-1 study investigator. "I am encouraged by the positive interim safety and efficacy data from TARA-002 and believe this promising candidate has the potential to help the many patients in need of FDA-approved therapeutic approaches for LMs."

STARBORN-1 Interim Results

The interim analysis includes a total of 12 patients who enrolled in the trial and received ≥ 1 dose of TARA-002 as of the November 12, 2025 data cutoff. Of those, eight patients were evaluable at an eight-week post-treatment assessment, two withdrew prior to the eight-week assessment and two remain in dosing. Patients receive up to four injections of TARA-002 spaced approximately six weeks apart. Of the eight patients who were evaluable, the majority (7/8) achieved clinical success with one or two doses. Only one patient, who presented with a 1,739 ml macrocystic LM, required all four doses, and achieved a complete response.

- 80% (8/10) of patients that completed treatment achieved clinical success
- 100% (8/8) of patients who completed the eight-week response assessment achieved clinical success
- 83% (5/6) of macrocystic patients achieved a complete response (90% to 100% reduction in total LM volume) and one patient achieved a substantial response (60% to less than 90% reduction in total LM volume)
- The only mixed cystic patient treated achieved a complete response
- Two LMs patients reached the 32-week post-treatment assessment and remain disease-free
- One patient deemed a complete response was subsequently diagnosed with a ranula (a different type of maxillofacial cyst from LMs)
- Two patients withdrew before the eight-week post-treatment assessment:
    - One patient was misdiagnosed and had a rare form of cancer and did not respond to treatment
    - One patient dropped out after achieving a notable resolution of the patient's macrocystic LM. The patient received two doses of TARA-002 with 160 ml aspiration at the first dose, which was reduced to a 10 ml aspiration at second dose.

Safety

The majority of adverse events (AEs) were mild to moderate, with no serious AEs reported. The most common AEs were swelling and fatigue. One patient discontinued treatment due to a Grade 2 AE of fatigue.

About STARBORN-1

STARBORN-1 is a Phase 2 single-arm, open-label, prospective clinical trial evaluating the safety and efficacy of intracystic injection of TARA-002 for the treatment of macrocystic and mixed cystic LMs (≥ 50% macrocystic disease) in 29 participants six months to less than 18 years of age. The trial includes age de-escalation safety lead-in cohorts of children ages six years to less than 18 years, two years to less than six years and six months to less than two years. Assessment of efficacy is based on the proportion of participants with macrocystic and mixed cystic LMs who demonstrate clinical success, defined as having either a complete response (90% to 100% reduction from baseline in total LM volume) or substantial response (60% to less than 90% reduction in total LM volume) as measured by axial imaging or via investigator assessment (physical exam, visual inspection and ultrasound). More information about the trial is available at clinicaltrials.gov (identifier: NCT05871970).

Conference Call and Webcast

Protara will host a conference call and webcast today at 8:30 am ET to review the data reported this morning, as well as provide an overview of LMs, the current treatment landscape and the TARA-002 program in LMs. Members of the management team will be joined by STARBORN-1 study investigator Jesse G.A. Jones, M.D., Associate Professor, Department of Neurosurgery and Radiology, University of Alabama at Birmingham. The live event and accompanying slides can be accessed by visiting https://protara-therapeutics-update-call.open-exchange.net/registration, or via the Events and Presentations section of the Company's website: https://ir.protaratx.com. A replay of the webcast will be archived for a limited time following the event.

About TARA-002 in LMs

TARA-002 is an investigational, genetically distinct strain of streptococcus pyogenes that is inactivated while retaining its immune-stimulating properties. It was developed from the same master cell bank as OK-432, which was originally granted marketing approval by the Japanese Ministry of Health for the treatment of LMs and has been the standard of care in Japan for 30 years. In addition, OK-432 was studied in a large Phase 2 trial in LMs in over 500 patients with significant clinical success. TARA-002 has been granted Rare Pediatric Disease designation by the U.S. Food and Drug Administration for the treatment of LMs.

About Lymphatic Malformations

Lymphatic malformations (LMs) are rare, congenital malformations of lymphatic vessels resulting in the failure of these structures to connect or drain into the venous system. Most LMs are present in the head and neck region and are diagnosed in early childhood during the period of active lymphatic growth, with more than 50% detected at birth and 90% diagnosed before the age of three years. The most common morbidities and serious manifestations of the disease include compression of the upper aerodigestive tract, including airway obstruction requiring intubation and possible tracheostomy dependence; intralesional bleeding; impingement on critical structures, including nerves, vessels and lymphatics; recurrent infection; and cosmetic and other functional disabilities.

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 who are unresponsive or naïve to treatment with Bacillus Calmette-Guérin, 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 the timing of any particular phases of such trials and the timing of the announcement of any data produced during such trials or phases thereof); 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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