Protara Therapeutics Receives Both FDA Breakthrough Therapy and Fast Track Designations for TARA-002 in Pediatric Patients with Lymphatic Malformations

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TARA-002 selected for FDA manufacturing development and readiness pilot program

NEW YORK, Jan. 05, 2026 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted both Breakthrough Therapy and Fast Track designations for TARA-002, the Company's investigational cell-based therapy, for the treatment of pediatric patients with macrocystic and mixed cystic lymphatic malformations (LMs). In addition, the FDA has selected TARA-002 to participate in the Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) Program, which aims to support CMC development of products with expedited clinical development timeframes and provide patients with earlier access.

"Receiving these important FDA designations and invitation to participate in the CDRP program highlights the significant unmet need among pediatric patients with LMs and underscores our belief that TARA-002 could serve as a meaningful treatment option for this underserved patient population," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We look forward to continuing to work with the Agency to bring this promising therapy to patients as expeditiously as possible and expect to meet with the FDA to define the path to registration for TARA-002 in LMs in the first half of this year."

The FDA's Breakthrough Therapy designation is a process designed to expedite the development and regulatory review of drugs or biologics that are intended to treat serious conditions where preliminary clinical evidence indicates that the drug or biologic may demonstrate substantial improvement on at least one clinically significant endpoint over available therapy. The FDA's Fast Track program is intended to facilitate the development and expedite the review of new drugs and biologics designed to treat serious conditions with unmet medical needs.

TARA-002 was previously granted Rare Pediatric Disease designation for the treatment of LMs.

The FDA created the CDRP Program to facilitate CMC development for therapies with compressed clinical development timeframes based on the anticipated clinical benefits of earlier patient access to the therapy. The initiative is designed to promote earlier and more structured engagement between sponsors and the FDA on CMC development strategies, and since its inception, has led to increased collaboration with the FDA so that sponsors can confidently scale up manufacturing capacity while clinical development is ongoing. TARA-002 has been accepted into this program, which will involve manufacturing for both the company's LMs and non-muscle invasive bladder cancer (NMIBC) programs.

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, Breakthrough Therapy and Fast Track designations by the U.S. Food and Drug Administration for the treatment of LMs.

About 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 (CIS) who are unresponsive or naïve to treatment with Bacillus Calmette-Guérin, as well as a Phase2 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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