Protara Therapeutics Receives Regulatory Clearance from FDA to Commence Phase 2 STARBORN-1 Trial of TARA-002 in Pediatric Patients with Lymphatic Malformations

May 2, 2023

- *STARBORN-1 trial leverages data from TARA-002 predecessor therapy OK-432, which is approved and has treated thousands of pediatric patients with Lymphatic Malformations in Japan and Taiwan as well as over 500 patients in a compassionate use study completed in the U.S.*
- *TARA-002 previously granted Rare Pediatric Disease Designation by the FDA for the treatment of Lymphatic Malformations*
- *Study startup activities ongoing; trial initiation expected in Q423*

NEW YORK, May 02, 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 that it has received regulatory clearance from the U.S. Food and Drug Administration (FDA) on its planned Phase 2 STARBORN-1 trial evaluating TARA-002, an investigational cell-based immunopotentiator, for the treatment of pediatric patients with lymphatic malformations (LMs). Trial initiation is expected in the fourth quarter of 2023.

“There are currently no FDA-approved treatments for LMs, a rare, serious condition mostly affecting children,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We are pleased to take the next step on our journey to deliver TARA-002 to these young patients and look forward to initiating this Phase 2 trial in pediatric patients with LMs.”

“LMs is a devastating disease for young children and there is a significant need for treatment options. Current management of these malformations often includes surgical excision, which can be a difficult procedure and have a high rate of complication and recurrence, especially in very young children,” said Richard Smith, M.D., Professor of Otolaryngology in the University of Iowa Carver College of Medicine and Director of Molecular Otolaryngology and Renal Research Laboratories and the Iowa Institute of Human Genetics. “As the lead investigator of the largest trial ever completed in LMs using OK-432, the predecessor therapy to TARA-002, I am very encouraged by the progress of TARA-002 as a potential treatment for this serious disease.”

STARBORN-1 is a Phase 2 single-arm, open-label, prospective clinical trial to evaluate the safety and efficacy of intracystic injection of TARA-002 for the treatment of macrocystic and mixed cystic LMs (≥ 50% macrocystic disease) in participants six months to less than 18 years of age. Following completion of an age de-escalation safety lead-in, the trial will enroll approximately 30 patients who will receive up to four injections of TARA-002 spaced approximately six weeks apart.

The primary endpoint of the trial is the proportion of participants with macrocystic LMs and mixed cystic LMs who demonstrated 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) eight weeks after the last injection, as measured by axial imaging.

TARA-002 is an investigational cell therapy based on the broad immunopotentiator, OK-432, which was originally granted marketing approval by the Japanese Ministry of Health and Welfare as an immunopotentiating cancer therapeutic agent. This cell therapy is currently approved in Japan and Taiwan for LMs and has been used to successfully treat thousands of pediatric patients. In addition, OK-432 was studied in the largest ever conducted Phase 2 trials in LMs, in which the therapy was administered via a now-closed compassionate use program led by the University of Iowa to over 500 pediatric and adult patients in the United States.

TARA-002 has been granted Rare Pediatric Disease designation by the FDA for the treatment of LMs.

About TARA-002 in LMs

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 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, lymphatics; recurrent infection, and cosmetic and other functional disabilities.

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 therapy for patients dependent on parenteral nutrition. 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; statements related to expectations regarding interactions with the FDA, including potential alignment with the FDA on a development path for TARA-002 in pediatric LM patients; 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.

Company Contact:

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

Source: Protara Therapeutics