Protara Therapeutics Announces Dosing of First Patient in Phase 2 STARBORN-1 Trial of TARA-002 in Pediatric Patients with Lymphatic Malformations

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NEW YORK, Oct. 23, 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 dosing is now underway in the Phase 2 STARBORN-1 trial evaluating TARA-002, an investigational cell-based immunopotentiator, for the treatment of pediatric patients with lymphatic malformations (LMs).

“There is a pressing need for an effective FDA-approved therapy to treat LMs, a rare condition mainly affecting children. There are currently no such agents that are FDA approved specifically for this indication,” said Nancy Bauman, MD, Children's National Medical Center: Children's Research Institute, Washington DC, and investigator for the STARBORN-1 trial. “Having witnessed first-hand in prior studies the significant benefit observed with OK-432, the predecessor of TARA-002, I firmly believe that TARA-002 has the potential to play a meaningful role in this vulnerable patient population.”

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. Including 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.

“Initiation of the STARBORN-1 trial marks an important step forward for our LMs program and, more importantly, the young patients impacted by this rare condition,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We are committed to bringing TARA-002 to patients suffering from this devastating disease and look forward to advancing this study.”

The primary endpoint of the trial is 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.

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 with this rare condition. 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.

TARA-002 has been granted Rare Pediatric Disease designation by the U.S. Food and Drug Administration (FDA) 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, lymphatics; recurrent infection, and cosmetic and other functional disabilities.

About TARA-002 in LMs

TARA-002 is an investigational cell therapy in development for the treatment of non-muscle invasive bladder cancer 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.

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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