Protara Therapeutics Presents Results from Retrospective Analysis of Randomized and Open-Label Studies Evaluating the Safety and Efficacy of OK-432 in Patients with Lymphatic Malformations

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Data showed OK-432 was clinically successful in treating lymphatic malformations and support a favorable safety profile

NEW YORK, May 12, 2022 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced the results of a retrospective analysis of OK-432, the originator compound for TARA-002, Protara's investigational therapy in development for the treatment of lymphatic malformations (LMs). LMs are serious, rare, congenital malformations of lymphatic vessels. The results from the analysis, which were presented during a poster presentation at the International Society for the Study of Vascular Anomalies (ISSVA) World Congress 2022, showed that OK-432 was clinically successful and generally well-tolerated in the treatment of both macrocystic and mixed-cystic LMs.

"We are pleased to share these compelling results, which are consistent with the robust body of approximately 30 years of patient experience with OK-432," said Richard Smith, M.D., Department of Otolaryngology, Carver College of Medicine, University of Iowa, and author of the study. "There are currently no FDA-approved treatments for LMs, which are usually diagnosed in early childhood and can lead to serious complications. These data provide continued support for the potential of TARA-002 to ultimately serve as an effective intervention in this highly underserved area."

The retrospective analysis included 246 patients from a Phase 2 randomized study, and 275 patients from an open-label study. The majority of participants in both studies were six months to 18 years of age. In the first study, patients were randomized 2:1 to receive treatment immediately (immediate treatment group [ITG]) or delayed by six months (delayed treatment group [DTG]). In the open-label study, patients received four doses of OK-432 approximately six weeks apart. The primary efficacy endpoint was clinical success (defined as complete [90%-100%] or substantial [60%-89%] reduction in LM volume measured radiographically) in the ITG versus spontaneous resolution of the LM in the DTG. Efficacy was assessed two weeks post-treatment in the randomized study, and one to six months post-treatment in the open-label study.

Key findings are summarized below:

- Approximately 69% of patients in the randomized study ITG achieved clinical success after six months, while only 7.5% of patients in the DTG showed spontaneous resolution of LMs in the same time period ($p<0.0001$).
- 73.1% of patients in the open-label study achieved clinical success.
- In the randomized and open-label studies, 10 of 219 (4.6%) and 5 of 275 (1.8%) subjects, respectively, were reported to have treatment emergent serious adverse events that were assessed by the investigator as related to study drug. The most severe adverse events (SAE) were airway obstruction and facial paralysis due to swelling post-injection that required tracheostomy and hospitalization. Both of these events were reported as resolved. One SAE related to OK-432 led to discontinuation (proptosis of the eye).
- Local/systemic reactions peaked in the first few days and resolved within two weeks.
- Patients were followed for up to three years post treatment with no significant safety concerns.

"We are encouraged by the growing amount of positive data supporting OK-432 in helping patients with LMs," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We look forward to utilizing the robust data set for OK-432 to support further development of TARA-002 as we work toward our goal of delivering the first approved medication for LMs in the U.S."

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of NMIBC and 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 Taiwan by Chugai Pharmaceutical Co., Ltd. Protara has successfully demonstrated 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, tumor necrosis factor (TNF)-alpha, granulocyte colony-stimulating factor, and granulocyte-macrophage colony-stimulating factor, are secreted by immune cells to induce a strong local 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 identifying and advancing transformative therapies for people with cancer and rare diseases with limited treatment options. 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 the treatment of intestinal failure-associated liver disease. 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 footing; 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; the impact of the COVID-19 pandemic on Protara’s business and the global economy as well as the impact on Protara’s contract research organizations, study sites or other clinical partners; 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 or political 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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