Protara Therapeutics Provides Comparability and U.S. Regulatory Updates for TARA-002 Supporting Advancement in Oncology and Rare Disease Indications

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- Company Demonstrates Initial Comparability Between TARA-002 and OK-432, Advancing to Final GMP Comparability Runs -

- Reaches Alignment with FDA on Development Path Forward for TARA-002 in Non-Muscle Invasive Bladder Cancer; Company Plans to Initiate Clinical Trials in 2021 -

- Company Expects to Request Meeting with FDA by Year End to Discuss Path to BLA for TARA-002 in Lymphatic Malformations -

NEW YORK, Sept. 08, 2020 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases with significant unmet needs, today announced its development plans for TARA-002 in both non-muscle invasive bladder cancer (NMIBC) and Lymphatic Malformations (LMs) following recent interactions with the U.S. Food and Drug Administration (FDA). TARA-002 is the Company's lead investigational cell therapy based on the broad immunopotentiator OK-432, which is approved in Japan and Taiwan for the treatment of LMs and multiple oncologic indications.

"The outcome of our recent interaction with the FDA represents a significant milestone for the TARA-002 program, confirming initial comparability between TARA-002 and OK-432 and expanding the potential market opportunity of TARA-002 beyond LMs to include a potentially significant oncology indication," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Importantly, we believe that we have identified an efficient path forward that allows us to address the population of patients suffering from NMIBC who are lacking alternative therapeutic options. Patients suffering from NMIBC have limited approved treatment options, and with the current standard-of-care facing a historical long-term supply shortage, there is a dire need for novel and effective therapies for these patients."

Mr. Shefferman added, "In addition to the opportunity in NMIBC, as we near completion of our IND update and make progress toward finalizing GMP comparability of TARA-002 to OK-432, we continue to expect to request a meeting with the FDA by year end to discuss a potential near-term path to a BLA filing for TARA-002 in LMs. TARA-002 was recently granted a Rare Pediatric Disease Designation for LMs, a rare pediatric indication with an urgent need for new therapeutic approaches."

FDA Confirmed Initial Comparability Between TARA-002 and OK-432; Final GMP Comparability Expected to be Completed in Mid-2021

Following a recent pre-Investigational New Drug (IND) engagement with the Office of Tissues and Advanced Therapies division of the Center for Biologics Evaluation and Research (CBER), the FDA agreed that Protara has successfully demonstrated initial manufacturing comparability between TARA-002 and OK-432 and that the Company was on track with its plans to conduct three large-scale batch runs to confirm comparability. Good Manufacturing Practice (GMP) scale up is currently in process and the Company will initiate GMP comparability runs with an expected completion date in mid-2021.

Clinical Development Path in NMIBC

In addition, the Company reached alignment with the FDA on a proposed clinical development plan to evaluate TARA-002 in patients with NMIBC. Advancement into the clinic will be supported by existing and ongoing non-clinical studies as well as the historical safety and efficacy data for OK-432.

Subject to the successful completion of select non-clinical studies to characterize local toxicity of intravesical administration of TARA-002 as well as acceptance of an IND filing, the Company plans to commence a Phase 1 study in 2021 to assess the safety and tolerability of TARA-002 in patients with NMIBC, including patients with carcinoma in situ (CIS), with results expected in 2022.

The Phase 2 development program, which Protara plans to commence in 2022, is expected to include NMIBC patients with CIS +/- Ta and/or T1 papillary tumors and high-grade Ta and/or T1 papillary tumors without CIS.

Regulatory Path in LMs

Protara plans to request a meeting with the FDA Division of Vaccines and Related Products Applications by year end to discuss the regulatory path for TARA-002 in LMs. The Company plans to utilize the robust dataset for OK-432 in LMs to support a Biological License Application (BLA) filing for TARA-002 in LMs. In a randomized, Phase 2 clinical trial of OK-432 in LMs conducted in the U.S., 68% of patients treated with OK-432 (>90% pediatric) in the immediate treatment group experienced a complete or substantial response. Long-term control of LMs was favorable, with more than 90% of patients treated with OK-432 having no regrowth over a median follow-up period of approximately three years following treatment.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of lymphatic malformations (LMs) and non-muscle invasive bladder cancer (NMIBC). 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 successfully demonstrated initial 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-6, IL-8, IL-12, interferon (IFN)-gamma, tumor necrosis factor (TNF)-alpha, and vascular endothelial growth factor (VEGF) are secreted by immune cells to induce a

strong local inflammatory reaction and destroy the abnormal cells. TARA-002 has been granted Rare Pediatric Disease Designation by the U.S. Food and Drug Administration for the LMs indication.

About Non-Muscle Invasive Bladder Cancer

Bladder cancer is the 6th most common cancer in the United States, with non-muscle invasive bladder cancer (NMIBC) representing approximately 80% of bladder cancer diagnoses. Approximately 65,000 patients are diagnosed with NMIBC in the United States each year. NMIBC is cancer found in the tissue that lines the inner surface of the bladder that has not spread into the bladder muscle. The current standard of care for high-grade NMIBC includes intravesical Bacillus Calmette-Guerin (BCG), which has been the subject of multiple global supply shortages in the past decade.

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 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 2 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: statements regarding Protara's business strategy, Protara's manufacturing and development plans for its product candidates and related interactions with the FDA. 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 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, clinical supply chain, clinical trials and the global economy; 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; the loss of key members of management; 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:

Blaine Davis
Protara Therapeutics
Blaine.Davis@protaratx.com
646-844-0337

Source: Protara Therapeutics