Protara Therapeutics Announces FDA Clearance of Investigational New Drug Application for TARA-002 for the Treatment of Non-Muscle Invasive Bladder Cancer

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- Company Plans to Initiate Phase 1 Study of TARA-002 by Year-End -

- IND Application Included Submission of Confirmatory Large-Scale GMP Comparability Data -

NEW YORK, Oct. 12, 2021 (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 U.S. Food and Drug Administration (FDA) has cleared the Company's Investigational New Drug (IND) application for TARA-002, an investigational cell-based therapy being developed for the treatment of non-muscle invasive bladder cancer (NMIBC). Protara expects to initiate a Phase 1 clinical trial of TARA-002 in adults with high-grade NMIBC by the end of 2021.

"We are thrilled to have reached this important milestone and look forward to quickly initiating our Phase 1 study in patients with NMIBC," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "There is an urgent need for new treatments for NMIBC. We are seeing significant increases in recurrence and disease progression, as well as an escalating number of patients requiring cystectomies. Supported by the strength of the existing clinical data in NMIBC for OK-432, the originator therapy for TARA-002, we believe this treatment represents a promising new option for NMIBC patients."

The Phase 1 dose-finding, open-label trial will evaluate TARA-002 in treatment-naïve and treatment-experienced NMIBC patients with high-grade carcinoma in situ (CIS) and high-grade papillary tumors (Ta). In the initial dose escalation phase of the trial, patients will receive six weekly intravesical doses of TARA-002. The primary objective of the trial is to evaluate the safety, tolerability and preliminary signs of anti-tumor activity of TARA-002, with the goal of establishing a maximum tolerated dose and recommended dose for a future Phase 2 clinical trial.

TARA-002 is manufactured from the same cell bank as OK-432, an approved therapy in Japan and Taiwan for multiple oncologic indications. In 2020, Protara successfully demonstrated initial manufacturing comparability between TARA-002 and OK-432. The confirmatory, GMP-scale comparability data for TARA-002 in relation to OK-432 have been completed and were reviewed by FDA as part of the clearance of the IND.

About TARA-002
TARA-002 is an investigational cell therapy in development for the treatment of non-muscle invasive bladder cancer and lymphatic malformations (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-6, IL-8, IL-12, interferon-gamma (IFN-γ)-, tumor necrosis factor-alpha (TNF-α), and vascular endothelial growth factor (VEGF) are secreted by immune cells to induce a strong local inflammatory reaction and destroy the abnormal cells.

About Non-Muscle Invasive Bladder Cancer
Bladder cancer is the 6th most common cancer in the United States, with 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.

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 non-clinical studies and clinical trials, and statements regarding the anticipated safety or efficacy of Protara's product candidates. 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 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; having to use cash in ways or

on timing other than expected; the impact of market volatility on cash reserves; 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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