Protara Therapeutics Provides Regulatory Update for TARA-002 for the Treatment of Lymphatic Malformations

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Company to conduct additional clinical study to support submission of a Biologics License Application for TARA-002 in Lymphatic Malformations

NEW YORK, April 23, 2021 (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 a path forward related to TARA-002 for the treatment of Lymphatic Malformations (LMs), which are rare malformations of the lymphatic vasculature for which there is no U.S. Food and Drug Administration (FDA)-approved treatment. Based on feedback from the FDA, the Company intends to complete confirmatory, large-scale, GMP manufacturing comparability in the second half of 2021 and subsequently initiate a clinical study in pediatric LM patients pending alignment with FDA on study design.

“With the benefit of the recent FDA feedback, we will work with the agency to align on a clinical study in pediatric LM patients, which, we believe, combined with the existing dataset for OK-432 (the originator compound for TARA-002), which demonstrated treatment effect and support for strong safety profile in over 500 LM patients, should provide a robust data package for this rare disease,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We have already begun preparation to initiate a clinical study in LMs and we look forward to continued collaboration with FDA to achieve our goal of delivering the first approved medication for LMs to these patients and their physicians.”

TARA-002 is derived from the same cell bank as OK-432, a broad immunopotentiator approved in Japan and Taiwan for the treatment of LMs, where it is currently the standard of care. In 2020, Protara successfully demonstrated initial manufacturing comparability between TARA-002 and the originator compound OK-432, which has been studied in more than 500 patients in one of the largest Phase 2 trials ever conducted in LMs. TARA-002 has been granted Rare Pediatric Disease designation by the 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 3 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

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, including its plans with respect to clinical studies and anticipated timing, Protara’s development objectives 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 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.

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