Protara Therapeutics Receives Rare Pediatric Disease Designation for TARA-002 for the Treatment of Lymphatic Malformations

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NEW YORK, July 28, 2020 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical stage company developing treatments for rare and specialty diseases with significant unmet needs, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease designation for TARA-002 for the treatment of Lymphatic Malformations (LMs). TARA-002 is an investigational cell-based therapy based on the broad immunopotentiator OK-432, which is approved in Japan and Taiwan for the treatment of LMs. LMs are rare, typically congenital, malformations of the lymphatic vasculature.

“Receipt of Rare Pediatric Disease designation from the FDA for TARA-002 in LMs further underscores the significant unmet medical need for the children that are affected by this rare and serious disease,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We are encouraged about the potential of TARA-002 to enhance the treatment paradigm of LMs for this underserved population, and we look forward to working with the agency to determine next steps for the program in the coming months.”

About FDA Rare Pediatric Disease Designation

The FDA grants Rare Pediatric Disease designation for serious diseases that primarily affect children ages 18 years or younger and fewer than 200,000 persons in the United States. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application or biologics license application for a product for the prevention or treatment of a rare pediatric disease may be eligible for a voucher, which can be redeemed to obtain priority review for any subsequent marketing application or may be sold or transferred.

About TARA-002 for Lymphatic Malformations

TARA-002, Protara’s lead product candidate, is an investigational cell-based therapy based on the broad immunopotentiator OK-432. OK-432 is approved in Japan and Taiwan for lymphatic malformations (LMs), which are rare, congenital malformations of lymphatic vessels resulting in the failure of these structures to connect or drain into the venous system. In a randomized, Phase 2 clinical trial of OK-432 in LMs, 86% of patients treated with OK-432 (>90% pediatric) 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 three years following treatment.

TARA-002 has been granted Rare Pediatric Disease designation by the U.S. Food and Drug Administration for the treatment of LMs.

About Protara Therapeutics, Inc.

Protara is committed to identifying and advancing transformative therapies for people with rare and specialty diseases who have limited treatment options. Protara’s portfolio includes its lead program, TARA-002, an investigational cell-based therapy being developed for the treatment of lymphatic malformations, and IV Choline Chloride, an investigational phospholipid substrate replacement therapy for the treatment of IFALD. 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. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, statements regarding the potential that Protara will receive a priority review voucher from the FDA. Risks that contribute to the uncertain nature of the forward-looking statements include: uncertainties related to 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; and uncertainties related to the actual impacts and length of such impacts caused by the COVID-19 pandemic. These and other risks and uncertainties are 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. Protara undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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