Protara Therapeutics Granted FDA Fast Track Designation for Intravenous Choline Chloride for Patients Receiving Parenteral Support

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- *Remain on track to initiate registrational THRIVE-3 trial in 1Q' 2025*

NEW YORK, Oct. 21, 2024 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to Intravenous (IV) Choline Chloride, the Company's investigational IV phospholipid substrate replacement therapy, as a source of choline for adult and adolescent patients on parenteral support (PS) for whom oral or enteral nutrition is not possible, insufficient, or contraindicated. In the U.S. alone, there are approximately 40,000 patients on long-term parenteral support.

"Receipt of Fast Track designation underscores the urgent need in these patients and our belief that IV Choline Chloride has the potential to serve as the first FDA-approved IV choline therapy for patients dependent on PS," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Approximately 80% of patients dependent on PS experience choline deficiency, the long-term consequences of which can lead to serious hepatic injury, neuropsychological impairment, muscle damage, and thrombotic abnormalities, yet there are no currently approved IV choline products for patients dependent on PS globally. Looking ahead, we remain on track to initiate our registrational THRIVE-3 clinical trial in the first quarter of 2025."

IV choline is recommended for patients receiving PS by the American Society for Parenteral and Enteral Nutrition in its Recommendations for Changes in Commercially Available Parenteral Multivitamin and Multi–Trace Element Products, as well as by the European Society for Clinical Nutrition and Metabolism Congress in its Guideline on Home Parenteral Nutrition.

Based on feedback from the FDA, Protara intends to assess the safety and efficacy of IV Choline Chloride in THRIVE-3, a seamless registrational Phase 2b/3 trial with dose confirmation followed by a double-blinded, randomized, placebo-controlled trial in adolescents and adults receiving parenteral support. The primary endpoint of the trial is the change in plasma choline concentration from baseline compared to placebo.

About FDA Fast Track Designation

The FDA's Fast Track program facilitates the development and expedites the review of drugs that treat serious conditions and have the potential to address an unmet medical need. Programs with Fast Track designation may benefit from early and frequent interactions with the FDA over the course of drug development. In addition, the Fast Track designation program allows for the eligibility for accelerated approval and priority review if relevant criteria are met and enables a company to submit individual sections of a New Drug Application for review on a rolling-submission basis.

About IV Choline Chloride

IV Choline Chloride is an investigational, intravenous phospholipid substrate replacement therapy in development for patients receiving parenteral support (PS). Choline is a known important substrate for phospholipids that are critical for healthy liver function that also play an important role in modulating gene expression, cell membrane signaling, brain development and neurotransmission, muscle function, and bone health. PS patients are unable to synthesize choline from enteral nutrition sources, and there are currently no available PS formulations containing choline. Approximately 80% of patients dependent on PS are choline-deficient and have some degree of liver damage, which can lead to hepatic failure. In the U.S. alone, there are approximately 40,000 patients on long-term parenteral support who could benefit from an IV formulation of choline. IV Choline Chloride has the potential to become the first U.S. Food and Drug Administration (FDA) approved IV choline formulation for PS patients. IV Choline Chloride has been granted Orphan Drug Designation by the FDA for the prevention of choline deficiency in PS patients. The Company was issued a U.S. patent claiming a choline composition with a term expiring in 2041.

Protara recently presented results from THRIVE-1, a prospective, observational study evaluating the prevalence of choline deficiency and liver injury in patients dependent on PS, which found that 78% of patients who are dependent on PS were choline deficient, with 63% of these patients demonstrating liver dysfunction including steatosis, cholestasis, and signs of hepatobiliary injury.

About Protara Therapeutics, Inc.

Protara is a clinical-stage biotechnology company committed to advancing transformative therapies for people with cancer and rare diseases. Protara's portfolio includes its lead candidate, TARA-002, an investigational cell-based therapy in development for the treatment of non-muscle invasive bladder cancer (NMIBC) and lymphatic malformations (LMs). The Company is evaluating TARA-002 in an ongoing Phase 2 trial in patients with NMIBC with carcinoma in situ (CIS) who are unresponsive or naïve to treatment with Bacillus Calmette-Guérin, as well as a Phase 2 trial in pediatric patients with LMs. Additionally, Protara is developing IV Choline Chloride, an investigational phospholipid substrate replacement for patients on parenteral support who are otherwise unable to meet their choline needs through oral or enteral routes. 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; Protara's financial position; 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; general market conditions; changes in the competitive landscape; changes in Protara's strategic and development 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; failure to attract and retain management and key personnel; the impact of general U.S. and foreign, economic, industry, market, regulatory, political or public health 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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