Protara Therapeutics Receives Fast Track Designation from U.S. FDA for Intravenous Choline Chloride for the Treatment of Intestinal Failure Associated Liver Disease

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NEW YORK, May 26, 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 Fast Track designation to Intravenous (IV) Choline Chloride for the treatment of intestinal failure-associated liver disease (IFALD). IV Choline Chloride is the Company's Phase 3-ready investigational phospholipid substrate replacement therapy for patients receiving parenteral nutrition (PN) who have IFALD.

"Receiving Fast Track designation from the FDA further supports the potential for IV Choline Chloride to serve as the much-needed first approved therapy for IFALD patients," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We look forward to collaborating with FDA and utilizing the benefits associated with Fast Track designation to make this important therapy available for patients with IFALD."

Data from the randomized, controlled, Phase 2 trial in IFALD patients dependent on PN demonstrated that treatment with IV Choline Chloride resulted in normalization of plasma-free choline concentrations, reversal of hepatic steatosis, and a clinically meaningful and statistically significant improvement in cholestasis.

Protara held an end of Phase 2 meeting with the FDA in late 2018 and received the FDA's support to advance IV Choline Chloride into a registration-enabling study for the treatment of IFALD.

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 (NDA) for review on a rolling-submission basis.

About Intravenous (IV) Choline Chloride for IFALD

Intravenous (IV) Choline Chloride is a Phase 3-ready investigational phospholipid substrate replacement therapy initially in development for patients receiving parenteral nutrition (PN) who have intestinal failure-associated liver disease (IFALD). Protara believes that IV Choline Chloride has the potential to be the first approved therapy for IFALD. Choline is a known important substrate for phospholipids that are critical for healthy liver function, yet currently available PN formulations do not contain sufficient amounts of choline. IV Choline Chloride has been granted Orphan Drug Designation for the treatment of IFALD and prevention of choline deficiency in PN patients. IV Choline Chloride has also been granted Fast Track Designation for the treatment of IFALD.

About IFALD

IFALD is uniquely characterized by the presence of both steatosis (toxic fat accumulation in liver cells) and cholestasis (damage to the biliary system in the liver) in patients who are chronic (greater than six months) PN users.

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 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 Protara's business strategy, Protara's development plans for IV Choline Chloride and Protara's plans for interactions with the FDA for this product candidate. 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; uncertainties related to the actual impacts and length of such impacts caused by the COVID-19 pandemic; having to use cash in ways or on timing other than expected; and the impact of market volatility on cash reserves. 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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