Protara Therapeutics Announces Results from a Retrospective Study Evaluating the Prevalence of Cholestasis in Patients Dependent on Parenteral Nutrition

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-Study found that approximately 30% of patients who are dependent on parenteral nutrition have cholestasis-

-Results support significant unmet medical need in patients dependent on parenteral nutrition who have intestinal failure associated liver disease-

NEW YORK, Sept. 13, 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 the completion of a retrospective prevalence study designed to enhance understanding of the incidence of intestinal failure associated liver disease (IFALD) in patients dependent on parenteral nutrition (PN). The study found that approximately 30% of patients who are dependent on PN have cholestasis, a hallmark pathology of IFALD, despite the use of current medical management in these patients. The Company is currently developing intravenous (IV) Choline Chloride, an investigational phospholipid substrate replacement therapy, for the treatment of patients receiving PN who have IFALD.

“In addition to steatosis, we know that cholestasis is a core feature of IFALD and data from this prevalence study further underscore the significant need for an effective intervention for patients with IFALD, which carries a particularly poor prognosis in the absence of an intestine-liver transplant,” said Alan Buchman, M.D., Professor of Clinical Surgery and Medical Director, Intestinal Rehabilitation and Transplant Center, University of Illinois at Chicago. “Patients who are dependent on PN are unable to absorb sufficient levels of choline, an essential component of several metabolic processes, ultimately resulting in the development of IFALD. Results from the previously completed Phase 2 study of IV Choline Chloride support the clinical potential of choline substrate replacement therapy to treat IFALD.”

“Findings from this retrospective study reinforce that there are a significant number of patients dependent on PN who are suffering from IFALD who may potentially benefit from treatment with IV Choline Chloride,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We remain committed to strengthening the understanding of the unmet need in this devastating disease, for which there are no approved therapies.”

In order to further characterize the prevalence and needs of IFALD patients dependent on PN, the Company recently initiated a prospective, multi-center, cross-sectional observational study that will assess the prevalence of choline deficiency, as well as cholestasis and steatosis, in approximately 300 patients dependent on PN.

The Company expects to use the results from the completed retrospective study and ongoing prospective study to inform next steps for its IV Choline Chloride development program. As previously announced, the Company held a positive end of Phase 2 meeting with the U.S. Food and Drug Administration (FDA) and received feedback on the design of studies necessary to complete the registration package for IV Choline Chloride for the treatment of IFALD, including a Phase 1 pharmacokinetic study and a Phase 3 placebo-controlled study.

Retrospective Study Results

The retrospective, observational study was conducted in partnership with a large home health organization and examined data from 468 patients dependent on PN for six months or more. The primary endpoint of the study was to identify the proportion of patients dependent on PN with suspected liver disease defined as serum alkaline phosphatase (ALP) levels greater than 1.5 times the upper limit of normal (ULN). The study evaluated ALP levels from baseline up to 36 months to determine if there is a progressive component to cholestasis in IFALD and the degree to which medical management affected ALP levels. ALP is an established biomarker for cholestasis and a clinically meaningful indicator of IFALD severity and progression. Prolonged elevation of ALP is indicative of ongoing hepatocellular injury.

Key findings are summarized below:

- Approximately 31% of all patients, irrespective of baseline levels, presented with ALP levels greater than 1.5 times the ULN at any given time over a 30-month period.
- Approximately 28% of all patients had persistent ALP elevations greater than 1.5 times the ULN at 36 months.
- At baseline, approximately 23% of patients presented with ALP levels greater than 1.5 times the ULN.
    - In these patients, approximately 76% presented with greater than 1.5 times the ULN at any given time over a 30-month period and approximately 59% had persistent ALP elevations greater than 1.5 times the ULN at 36 months.
- While medical management demonstrated some improvement in ALP levels, it was not sufficient for managing ALP levels over the long term in patients on PN.
- Results support further exploration in this patient population to determine rates of choline deficiency and steatosis.

About IV Choline Chloride and Intestinal Failure-associated Liver Disease (IFALD)

IV Choline Chloride is an investigational, intravenous (IV) phospholipid substrate replacement therapy initially in development for patients receiving parenteral nutrition (PN) who have IFALD. Choline is a known important substrate for phospholipids that are critical for healthy liver function. Because PN patients cannot sufficiently absorb adequate levels of choline and no available PN formulations contain sufficient amounts of choline to correct this deficiency, PN patients often experience a prolonged progression to hepatic failure and death, with the only known intervention being a dual small bowel/liver transplant. If approved, IV Choline Chloride would be the first approved therapy for IFALD. It has been granted Orphan Drug Designations

(ODDs) by the FDA for the treatment of IFALD and the prevention of choline deficiency in PN patients.

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 plans with respect to the IV Choline Chloride program and the impact of the study results discussed in this press release on such plans, Protara's beliefs regarding the benefit of IV Choline Chloride for patients dependent on PN who are suffering from IFALD, Protara's development plans for its product candidates and related expectations regarding interactions or upcoming filings with the FDA and plans regarding the timing or outcome of existing or future non-clinical studies and clinical trials. 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 sales, revenue, expense and other 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; 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; 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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