Protara Therapeutics Announces Third Quarter 2020 Financial Results and Business Overview

November 12, 2020

- On Track to Complete GMP Batch Runs in Mid-2021 to Confirm Comparability Between TARA-002 and OK-432 -
- Expect to Initiate Phase 1 Trial for TARA-002 in Patients with Non-Muscle Invasive Bladder Cancer in 2021 -
- Requested Meeting with FDA to Discuss Path Forward for TARA-002 in Lymphatic Malformations -
- Strong Cash Position of $166M as of September 30, 2020 -

NEW YORK, Nov. 12, 2020 (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 financial results for the third quarter ended September 30, 2020.

"The third quarter marked a highly productive time for the Company, notably with the Food and Drug Administration's (FDA) confirmation of initial comparability between TARA-002 and OK-432, and separately the Company's identification of an acceptable development path for TARA-002 in non-muscle invasive bladder cancer (NMIBC), an oncology indication with high unmet need," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We believe TARA-002 has the potential to serve as a much needed intervention for NMIBC patients lacking alternative therapeutic options, and we look forward to the expected commencement of our clinical program in NMIBC in 2021. Finally, we have requested a meeting with the FDA Division of Vaccines and Related Products, the review division which the Investigational New Drug (IND) application was initially opened by the University of Iowa, to discuss a potential Biological License Application (BLA) filing for TARA-002 in lymphatic malformations (LMs)."

Recent Highlights

TARA-002

In September 2020, Protara announced the following updates for the TARA-002 program:

- **FDA Confirmation of Initial Comparability Between TARA-002 and OK-432**. Following a pre-IND engagement with the Office of Tissues and Advanced Therapies (OTAT) division of the Center for Biologics Evaluation and Research (CBER), the FDA agreed that Protara has successfully demonstrated initial manufacturing comparability between TARA-002 and OK-432 and that the in-process and release protocols employed by Protara to demonstrate initial comparability are appropriate to utilize for GMP-Scale comparability testing. Good Manufacturing Practice (GMP) scale-up is currently in process and the Company expects to complete three such large-scale batch runs to confirm comparability in mid-2021.

- **Clinical Development Path in NMIBC.** The Company reached alignment with the FDA on a proposed clinical development plan to evaluate TARA-002 in patients with NMIBC. Advancement into the clinic will be supported by existing and ongoing non-clinical studies as well as historical clinical safety and efficacy data for OK-432. Subject to the successful completion of select non-clinical studies to characterize local toxicity of intravesical administration of TARA-002, as well as acceptance of an IND filing, the Company plans to commence a Phase 1 study in 2021 to assess the safety and tolerability of TARA-002 in patients with NMIBC, including patients with carcinoma in situ (CIS), with results expected in 2022.

- **Regulatory Path in LMs.** Protara has requested a meeting with the FDA Division of Vaccines and Related Products Applications to discuss the regulatory path for TARA-002 in LMs. The Company plans to utilize the robust dataset for OK-432 in LMs to support a BLA filing for TARA-002 in LMs.

IV Choline Chloride

- **Prevalence Study Underway to Assess Incidence of Liver Disease in Patients Dependent on Parenteral Nutrition (PN).** The Company recently commenced a prevalence study to assess the incidence of liver disease in patients dependent on PN in the home care setting. The Company believes the study will enhance its understanding of the population of patients who may potentially benefit from IV Choline Chloride, its investigational phospholipid substrate replacement therapy currently in development for the treatment of patients receiving PN who have intestinal failure-associated liver disease (IFALD).

Corporate Update

- **Raised $151 Million in Concurrent Public Offerings.** Protara recently announced the closing of concurrent but separate underwritten public offerings of 4,600,000 shares of its common stock, at a public offering price of $16.87 per share, and 4,148 shares of non-voting Series 1 Convertible Preferred Stock, at a public offering price of $16,873.54 per share. In

addition, the underwriters exercised their overallotment option in full to purchase an additional 690,000 shares of common stock at a public offering price of $16.87. Aggregate net proceeds to Protara were approximately $151 million, after deducting underwriting discounts and offering expenses.

Third Quarter 2020 Results from Operations

- As of September 30, 2020, cash and restricted cash were $166.0 million.
- Protara reported a net loss of $8.0 million for the third quarter of 2020 as compared to a net loss of $2.4 million for the three months ended September 30, 2019. The third quarter of 2020 included approximately $2.8 million of stock-based compensation expense.
- Research and Development expenses were $2.8 million for the third quarter of 2020, an increase of $1.7 million as compared to the three months ended September 30, 2019. The increase was primarily due to an increase of (i) $0.6 million in personnel and related costs, (ii) $0.8 million in chemistry manufacturing and controls (CMC) expenses and (iii) $0.3 million in regulatory expenses.
- General and Administrative expenses were $5.3 million for the third quarter of 2020, which represented an increase of $4.0 million as compared to the three months ended September 30, 2019. The increase was primarily related to an increase of (i) $2.6 million in stock-based compensation expense, (ii) $0.6 million in insurance expense and (iii) $0.7 million in personnel and related costs.

A Form 10-Q containing the full financial statements was filed this morning and is available for viewing on Protara's website at www.protaratx.com or www.sec.gov.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of lymphatic malformations (LMs) and non-muscle invasive bladder cancer (NMIBC). TARA-002 was developed from the same master cell bank of genetically distinct group A Streptococcus pyogenes as OK-432, a broad immunopotentiator marketed as Picibanil® in Japan and Taiwan by Chugai Pharmaceutical Co., Ltd. Protara successfully demonstrated initial manufacturing comparability between TARA-002 and OK-432.

When TARA-002 is administered, it is hypothesized that innate and adaptive immune cells within the cyst or tumor are activated and produce a strong immune cascade. Neutrophils, monocytes and lymphocytes infiltrate the abnormal cells and various cytokines, including interleukins IL-6, IL-8, IL-12, interferon (IFN)-gamma, tumor necrosis factor (TNF)-alpha, and vascular endothelial growth factor (VEGF) are secreted by immune cells to induce a strong local inflammatory reaction and destroy the abnormal cells. TARA-002 has been granted Rare Pediatric Disease Designation by the U.S. Food and Drug Administration for the LMs indication.

About Non-Muscle Invasive Bladder Cancer

Bladder cancer is the 6th most common cancer in the United States, with non-muscle invasive bladder cancer (NMIBC) representing approximately 80% of bladder cancer diagnoses. Approximately 65,000 patients are diagnosed with NMIBC in the United States each year. NMIBC is cancer found in the tissue that lines the inner surface of the bladder that has not spread into the bladder muscle. The current standard of care for high-grade NMIBC includes intravesical Bacillus Calmette-Guerin (BCG), which has been the subject of multiple global supply shortages in the past decade.

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 2 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 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, Protara’s development plans for its product candidates , including its plans regarding the timing or outcome of existing or future non-clinical studies and clinical trials, Protara’s expectations regarding interactions or upcoming filings with the FDA, statements regarding the anticipated safety or efficacy of Protara’s product candidates and, Protara’s financial footing. 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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PROTARA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS

	As of September 30, 2020 (unaudited)	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 165,904,797	$ 564,124
Restricted cash	50,000	-
Deferred offering costs	-	121,712
Prepaid expenses and other current assets	1,160,257	78,057
Total current assets	167,115,054	763,893
Non-current assets:		
Property and equipment, net	760,548	458,591
Goodwill	29,367,213	-
Other assets	1,664,442	-
Total assets	$ 198,907,257	$ 1,222,484
Liabilities and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 1,912,553	$ 715,653
Accrued expenses	1,569,348	2,634,790
Short-term debt	370,793	-
Right-of-use liability, current	34,079	-
Total current liabilities	3,886,773	3,350,443
Non-current liabilities:		
Right-of-use liability, long-term	394,721	-
Total liabilities	4,281,494	3,350,443
Commitments and Contingencies (Note 6)		
Stockholders' Equity (Deficit)		

Preferred Stock, $0.001 par value, authorized 10,000,000 shares:		
Series 1 Convertible Preferred Stock, 8,028 and 0 shares authorized at September 30, 2020 and December 31, 2019, respectively, 8,027 and 0 shares issued and outstanding as of September 30, 2020 and December 31, 2019, respectively.	8	-
Common Stock, $0.001 par value, authorized 100,000,000 shares:		
Common Stock, 10,521,840 and 2,627,533 common shares issued and outstanding as of September 30, 2020 and December 31, 2019, respectively.	10,522	2,628
Additional Paid in Capital	232,567,265	10,651,073
Accumulated Deficit	(37,952,032)	(12,781,660)
Total Stockholders' Equity (Deficit)	194,625,763	(2,127,959)
Total Liabilities and Stockholders' Equity (Deficit)	$ 198,907,257	$ 1,222,484

PROTARA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(unaudited)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expense:				
Research & development	$ 2,796,214	$ 1,098,617	$ 8,330,727	$ 3,163,179
General & administrative	5,265,965	1,255,466	17,156,952	2,147,635
Total operating expenses	8,062,179	2,354,083	25,487,679	5,310,814
Operating loss	(8,062,179)	(2,354,083)	(25,487,679)	(5,310,814)
Other income, net				
Interest income, net	(92,094)	-	(317,307)	-
Total other income, net	(92,094)	-	(317,307)	-
Net Loss	$ (7,970,085)	$ (2,354,083)	$ (25,170,372)	$ (5,310,814)
Weighted Average Shares Outstanding, basic and diluted	6,324,295	2,564,429	5,910,849	2,560,444
Net loss per share, basic and diluted	$ (1.26)	$ (0.92)	$ (4.26)	$ (2.07)

Source: Protara Therapeutics