Protara Therapeutics Announces Fourth Quarter and Full Year 2020 Financial Results and Business Overview

March 11, 2021

- Initiation of Phase 1 Trial of TARA-002 in Patients with Non-Muscle Invasive Bladder Cancer on Track to Commence by Year-End -

- In Discussions with FDA on Next Steps Needed to File BLA for TARA-002 in Lymphatic Malformations -

- On Track to Complete GMP Scale up and Comparability in 2H, 2021 -

- Strong Cash Position of $169M as of December 31, 2020 -

NEW YORK, March 11, 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 financial results for the fourth quarter and year ended December 31, 2020 and provided a business update.

"We believe 2021 will be a transformative year for Protara, and we are entering it with strong momentum," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We remain on track to commence a Phase 1 study of TARA-002 in patients with non-muscle invasive bladder cancer (NMIBC), a pressing area of unmet need, by the end of the year. We believe that TARA-002 has the opportunity to play a meaningful role in the current NMIBC treatment landscape."

Mr. Shefferman continued, "We are in discussions with the U.S. Food and Drug Administration's (FDA) Division of Vaccines and Related Products to establish a path forward to file our Biological License Application (BLA) for TARA-002 in lymphatic malformations (LMs). We are encouraged by the progress to date and, at the FDA's request, have submitted the full Clinical Study Report (CSR) of a randomized Phase 2 study of OK-432 (the originator compound of TARA-002) in LMs led by the University of Iowa. We look forward to continuing our dialogue with the FDA."

Recent Highlights and Upcoming Milestones

TARA-002 in NMIBC

- Protara remains on track to complete select non-clinical studies to characterize local toxicity of intravesical administration of TARA-002 in the first half of 2021, with an Investigational New Drug (IND) application submission anticipated in the second half of 2021. Subject to FDA acceptance of the IND application, the Company plans to commence a Phase 1 study by the end of 2021 to assess the safety and tolerability of TARA-002 in patients with NMIBC, including patients with carcinoma in situ (CIS).

TARA-002 in LMs

- Protara plans to utilize the robust dataset for OK-432 (the originator compound of TARA-002) in LMs to support a potential filing. In connection with Protara's request to discuss a potential BLA submission for TARA-002 in LMs, the FDA Division of Vaccines and Related Products has requested a CSR summarizing the totality of a randomized Phase 2 study of OK-432 in LMs led by the University of Iowa. The Company has submitted the CSR to the FDA and continues to prepare for a potential BLA filing in the second half of 2021, or to initiate additional clinical work as required.

IV Choline Chloride in intestinal failure associated liver disease (IFALD)

- Following a successful meeting with the FDA in 2018 regarding the registration package for IV Choline Chloride, the Company is currently undertaking a prevalence study in partnership with a large home health organization in the U.S. to enhance understanding of the appropriate patient population and will use this information to define the next steps for the development program.

Corporate Update

- In February 2021, the Company announced the appointment of Cynthia Smith to its Board of Directors. Ms. Smith brings to Protara over 20 years of diverse leadership experience within the healthcare industry, most recently serving as Chief Commercial Officer at ZS Pharma.

Fourth Quarter and Full Year 2020 Financial Results

- As of December 31, 2020, cash, cash equivalents and restricted cash were $169.4 million.

- Research and development expenses for the fourth quarter of 2020 increased to $3.7 million from $0.7 million for the prior

year period, and for the full year increased to $12.0 million compared to $3.9 million for 2019. The fourth quarter and full year increases were primarily due to increases in personnel and related costs, manufacturing and regulatory expenses as the company advanced its clinical programs supporting TARA-002.

- General and administrative expenses for the fourth quarter of 2020 increased to $5.3 million from $1.8 million for the prior year period, and for the full year increased to $22.5 million compared to $4.0 million for 2019. The fourth quarter and full year increases were primarily due to increases in stock-based compensation expense, insurance expense and personnel and related costs supporting the company's growth.

- For the fourth quarter of 2020, Protara reported a net loss of $8.8 million, or $0.79 per share, compared with a net loss of $2.5 million, or $0.96 per share, for the same period in 2019. Net loss for the year ended December 31, 2020 was $34.0 million, or $4.70 per share, compared with a net loss of $7.8 million, or $3.04 per share, for the year ended December 31, 2019. Net loss for the fourth quarter included approximately $2.3 million of stock-based compensation expenses. Net loss for the year ended December 31, 2020 included $9.7 million of stock-based compensation expenses.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of non-muscle invasive bladder cancer (NMIBC) and lymphatic malformations (LMs) for which it has been granted Rare Pediatric Disease Designation by the U.S. Food and Drug Administration. 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.

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 3 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 and related expectations regarding interactions or upcoming filings with the FDA, including its plans regarding the timing or outcome of existing or future non-clinical studies and clinical trials, Protara's financial footing, the impact of the COVID-19 pandemic and related governmental responses on Protara's business and clinical programs, 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 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.

PROTARA THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)

	As of December 31,	
	2020	2019
Assets		
Current assets:		
Cash and cash equivalents	$ 168,598	$ 564
Restricted cash	50	-
Deferred offering costs	-	122
Prepaid expenses and other current assets	787	78
Total current assets	169,435	764
Non-current assets:		
Restricted cash, long-term	745	-
Property and equipment, net	1,240	459
Goodwill	29,517	-
Other assets	2,220	-
Total assets	$ 203,157	$ 1,223
Liabilities and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 914	$ 716
Accrued expenses	1,913	2,635
Operating lease liability, current	88	-
Total current liabilities	2,915	3,351
Non-current liabilities:		
Operating lease liability, long-term	999	-
Total liabilities	3,914	3,351
Commitments and Contingencies (Note 7)		
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 December 31, 2020 and 2019, respectively, 8,027 and 0 shares issued and outstanding as of December 31, 2020 and 2019, respectively.	-	-
Common Stock, $0.001 par value, authorized 100,000,000 shares:		
Common Stock, 11,211,840 and 2,627,533 shares issued and outstanding as of December 31, 2020 and 2019, respectively.	11	3
Additional Paid in Capital	245,992	10,651
Accumulated Deficit	(46,760)	(12,782)
Total Stockholders' Equity (Deficit)	199,243	(2,128)
Total Liabilities and Stockholders' Equity (Deficit)	$ 203,157	$ 1,223

PROTARA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except share and per share data)

	For the Years Ended December 31,	
	2020	2019
Operating expense:		
Research & development	$ 11,982	$ 3,878
General & administrative	22,462	3,952
Total operating expenses	34,444	7,830
Operating loss	(34,444)	(7,830)
Other income, net		
Interest income, net	(466)	-
Total other income, net	(466)	-
Net Loss	$ (33,978)	$ (7,830)
Weighted Average Shares Outstanding, basic and diluted	7,233,913	2,577,493
Net loss per share, basic and diluted	$ (4.70)	$ (3.04)

PROTARA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)
(unaudited)

	For the Three Months Ended December 31,	
	2020	2019
Operating expense:		
Research & development	$ 3,651	$ 715
General & administrative	5,305	1,804
Total operating expenses	8,956	2,519
Operating loss	(8,956)	(2,519)
Other income, net		
Interest income, net	(148)	-
Total other income, net	(148)	-
Net Loss	$ (8,808)	$ (2,519)
Weighted Average Shares Outstanding, basic and diluted	11,174,340	2,627,533
Net loss per share, basic and diluted	$ (0.79)	$ (0.96)

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