Protara Therapeutics Announces Second Quarter 2021 Financial Results and Business Overview

August 5, 2021

- Successfully Completed IND-Enabling Studies for TARA-002 in Patients with NMIBC; On Track to Initiate Phase 1 Trial by Year-End -

- TARA-002 GMP Scale up and Comparability Ongoing and Expected to be Completed by Year-End -

- Company Plans to Engage with FDA to Determine Clinical Trial Design for TARA-002 in Lymphatic Malformations -

- Strong Cash, Cash Equivalents, and Investments Position of $145M as of June 30, 2021 -

NEW YORK, Aug. 05, 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 second quarter ended June 30, 2021 and provided a business update.

"We continue to make significant progress executing against our development plans for TARA-002 in non-muscle invasive bladder cancer (NMIBC) and lymphatic malformations (LMs), and we look forward to achieving multiple important milestones in the second half of this year," said Jesse Shefferman**,** Chief Executive Officer of Protara Therapeutics. "With the successful completion of required non-clinical studies for TARA-002, we remain on track to submit our Investigational New Drug (IND) application and initiate our Phase 1 clinical study by year-end. In addition, our Good Manufacturing Practice (GMP) scale up and comparability work remain on track to be completed by year-end, and we look forward to working with the U.S. Food and Drug Administration (FDA) on a clinical trial of TARA-002 in patients with LMs, a rare pediatric indication for which there are currently no U.S. FDA-approved therapies."

Recent Highlights and Upcoming Milestones

TARA-002 Comparability

- The Company remains on track to complete confirmatory, large-scale GMP manufacturing comparability by year-end.

TARA-002 in NMIBC

- Protara successfully completed required non-clinical IND-enabling studies to characterize local toxicity of intravesical administration of TARA-002. The Company remains on track to submit an IND application in the second half of 2021 and, subject to the FDA acceptance of the IND application, the Company plans to commence a Phase 1 trial 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

- Following the completion of confirmatory, large-scale GMP manufacturing comparability, the Company plans to align with the FDA on the design, and subsequently initiate a clinical trial in pediatric LM patients.

IV Choline Chloride in Intestinal Failure Associated Liver Disease (IFALD)

- The Company is currently executing 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 June 2021, the Company announced the appointment of Jane Huang, M.D., to its Board of Directors. Dr. Huang is an experienced biotech executive and proven leader throughout the development lifecycle of multiple oncology therapeutics globally and currently serves as Chief Medical Officer, Hematology at BeiGene, Ltd.

Second Quarter 2021 Financial Results

- As of June 30, 2021, cash, cash equivalents and investments totaled $145 million.

- Research and development expenses for the second quarter of 2021 increased to $5.9 million from $2.5 million during the second quarter of 2020. The increased R&D expenses were primarily due to increases in manufacturing and regulatory expenses associated with TARA-002.

- General and administrative expenses for the second quarter of 2021 increased to $6.9 million from $4.8 million during the

second quarter of 2020. The increase was primarily due to increases in stock-based compensation, headcount, and costs associated with the new Company headquarters in New York, NY.

- For the second quarter of 2021, Protara reported a net loss of $12.8 million, or $1.14 per share, compared with a net loss of $7.1 million, or $1.22 per share, for the second quarter of 2020. Net loss for the second quarter of 2021 included approximately $3.0 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 has 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.

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, including its plans with respect to certain clinical trials, GMP scale up and comparability work and anticipated timing, Protara's development plans for its product candidates and related expectations regarding interactions or upcoming filings with the FDA, including its planned IND filing for TARA-002 in NMIBC and plans regarding the timing or outcome of existing or future non-clinical studies and clinical trials, Protara's financial footing, 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.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)

	As of	
	June 30, 2021	December 31, 2020
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 46,836	$ 168,598
Restricted cash	-	50
Marketable debt securities, current	37,830	-
Prepaid expenses and other current assets	3,153	787
Total current assets	87,819	169,435
Non-current assets:		
Restricted cash, non-current	745	745
Marketable debt securities, non-current	60,378	-
Property and equipment, net	1,769	1,240
Operating lease right-of-use asset	7,648	1,060
Goodwill	29,517	29,517
Other assets, non-current	1,016	1,160
Total assets	$ 188,892	$ 203,157
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,403	$ 914
Accrued expenses	1,439	1,913
Operating lease liability, current	826	88
Total current liabilities	3,668	2,915
Non-current liabilities:		
Operating lease liability, non-current	6,819	999
Total liabilities	10,487	3,914
Commitments and Contingencies (Note 6)		
Stockholders' Equity		
Preferred Stock, $0.001 par value, authorized 10,000,000 shares:		
Series 1 Convertible Preferred Stock, 8,028 shares authorized at June 30, 2021 and December 31, 2020, 8,027 shares issued and outstanding as of June 30, 2021 and December 31, 2020.	-	-
Common Stock, $0.001 par value, authorized 100,000,000 shares:		
Common Stock, 11,233,856 and 11,211,840 shares issued and outstanding as of June 30, 2021 and December 31, 2020, respectively.	11	11
Additional Paid in Capital	251,502	245,992
Accumulated Deficit	(73,007)	(46,760)
Accumulated Other Comprehensive Income (Loss)	(101)	-
Total Stockholders' Equity	178,405	199,243
Total Liabilities and Stockholders' Equity	$ 188,892	$ 203,157

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

	For the three months ended June 30,		For the six months ended June 30,	
	2021	2020	2021	2020
Operating expense:				

Research & development	$ 5,887	$ 2,470	$ 12,927	$ 5,534
General & administrative	6,905	4,796	13,445	11,891
Total operating expenses	12,792	7,266	26,372	17,425
Operating loss	(12,792)	(7,266)	(26,372)	(17,425)
Other income, net				
Interest income, net	(10)	(126)	(125)	(225)
Total other income, net	(10)	(126)	(125)	(225)
Net loss	(12,782)	(7,140)	(26,247)	(17,200)
Other comprehensive gain (loss):				
Unrealized gains (losses) on available-for-sale marketable debt securities	63	-	(101)	-
Total other comprehensive gain (loss)	63	-	(101)	-
Comprehensive Loss	$ (12,719)	$ (7,140)	$ (26,348)	$ (17,200)
Weighted Average Shares Outstanding, basic and diluted	11,232,010	5,843,203	11,229,484	5,701,855
Net loss per share, basic and diluted	$ (1.14)	$ (1.22)	$ (2.34)	$ (3.02)

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