Protara Therapeutics Announces First Quarter 2021 Financial Results and Business Overview

May 6, 2021

- On Track to File IND and Initiate Phase 1 Trial of TARA-002 in Patients with NMIBC by Year-End -

- On Track to Complete TARA-002 GMP Scale up and Comparability in 2H, 2021 -

- Plan to Engage with FDA and Initiate Clinical Study of TARA-002 in Lymphatic Malformations -

- Strong Cash, Cash Equivalents and Investments Position of $155M as of March 31, 2021 -

NEW YORK, May 06, 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 first quarter ended March 31, 2021 and provided a business update.

"We remain on track to file an Investigational New Drug (IND) application and initiate our Phase 1 clinical study for TARA-002 in patients with non-muscle invasive bladder cancer (NMIBC) by the end of the year. Patients suffering from NMIBC have limited approved treatment options, and with the current standard-of-care facing a long-term supply shortage, there is an urgent need for effective therapies for these patients," said Jesse Shefferman**,** Chief Executive Officer of Protara Therapeutics. "We look forward to completing GMP scale up and comparability and then aligning with the Food and Drug Administration (FDA) on the design of a clinical study for TARA-002 in patients with Lymphatic Malformations (LMs). This new study, together with the extensive data for OK-432, the originator compound for TARA-002, should provide a robust data package for TARA-002 in this rare pediatric indication for which there is no U.S. FDA-approved therapy."

Recent Highlights and Upcoming Milestones

TARA-002 in NMIBC

- Protara remains on track to submit an IND application in the second half of 2021. Subject to the successful completion of select non-clinical studies, which are expected to be completed in the first half of 2021, and 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

- Based on feedback from the FDA, the Company intends to complete confirmatory, large-scale, GMP manufacturing comparability in the second half of 2021 and, upon alignment with FDA on study design, subsequently initiate a clinical study in pediatric LM patients.

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

- 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 April 2021, the Company announced the appointment of Martín Sebastian Olivo, M.D. as Chief Medical Officer. Dr. Olivo brings to Protara more than 15 years of experience in oncology translational and clinical research and global drug development, most recently serving as Vice President, Breast Cancer Clinical Development Lead at Gilead Sciences, Inc. (formerly Immunomedics, Inc.).

First Quarter 2021 Financial Results

- As of March 31, 2021, cash, cash equivalents and investments totaled $155 million.

- Research and development expenses for the first quarter of 2021 increased to $7.0 million from $3.1 million during the first 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 first quarter of 2021 decreased to $6.5 million from $7.1 million for the prior year period. The decrease was primarily due to one-time expenses related to the reverse merger, which occurred in the first quarter of 2020, off-set by an increase in personnel and related costs supporting the Company's growth.

- For the first quarter of 2021, Protara reported a net loss of $13.5 million, or $1.20 per share, compared with a net loss of $10.1 million, or $1.81 per share, for the same period in 2020. Net loss for the first quarter of 2021 included approximately $2.7 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).

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 clinical studies and anticipated timing, 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, 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	
	March 31, 2021	December 31, 2020
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 58,201	$ 168,598
Restricted cash	50	50
Marketable debt securities, current	26,144	-
Prepaid expenses and other current assets	3,302	787
Total current assets	87,697	169,435
Non-current assets:		
Restricted cash, non-current	745	745
Marketable debt securities, non-current	70,824	-
Property and equipment, net	1,498	1,240
Goodwill	29,517	29,517
Other assets	2,132	2,220
Total assets	$ 192,413	$ 203,157
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,853	$ 914
Accrued expenses	1,368	1,913
Operating lease liability, current	92	88
Total current liabilities	3,313	2,915
Non-current liabilities:		
Operating lease liability, non-current	974	999
Total liabilities	4,287	3,914
Commitments and Contingencies (Note 5)		
Stockholders' Equity		
Preferred Stock, $0.001 par value, authorized 10,000,000 shares:		
Series 1 Convertible Preferred Stock, 8,028 shares authorized at March 31, 2021 and December 31, 2020, 8,027 shares issued and outstanding as of March 31, 2021 and December 31, 2020.	-	-
Common Stock, $0.001 par value, authorized 100,000,000 shares:		
Common Stock, 11,228,606 and 11,211,840 shares issued and outstanding as of March 31, 2021 and December 31, 2020, respectively.	11	11
Additional Paid in Capital	248,504	245,992
Accumulated Deficit	(60,225)	(46,760)
Accumulated Other Comprehensive Income (Loss)	(164)	-
Total Stockholders' Equity	188,126	199,243
Total Liabilities and Stockholders' Equity	$ 192,413	$ 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 March 31,	
	2021	2020
Operating expense:		
Research & development	$ 7,040	$ 3,065
General & administrative	6,540	7,095

Total operating expenses	13,580	10,160
Operating loss	(13,580)	(10,160)
Other income, net		
Interest income, net	(115)	(100)
Total other income, net	(115)	(100)
Net loss	(13,465)	(10,060)
Other comprehensive loss:		
Unrealized losses on available-for-sale marketable debt securities	(164)	-
Total other comprehensive loss	(164)	-
Comprehensive Loss	$ (13,629)	$ (10,060)
Weighted Average Shares Outstanding, basic and diluted	11,226,929	5,560,507
Net loss per share, basic and diluted	$ (1.20)	$ (1.81)

Company Contact:

Justine O'Malley
Protara Therapeutics
Justine.OMalley@protaratx.com
646-817-2836

Source: Protara Therapeutics