Protara Therapeutics Announces First Quarter 2022 Financial Results and Business Overview

May 5, 2022

- Patient Dosing Underway in Phase 1 ADVANCED-1 Study of TARA-002 for the Treatment of Non-Muscle Invasive Bladder Cancer -

- Strong Cash, Cash Equivalents and Investments Position of $119M as of March 31, 2022 Expected to Fund Operations into Mid-2024 -

NEW YORK, May 05, 2022 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced financial results for the first quarter ended March 31, 2022 and provided a business update.

"The recent commencement of patient dosing in the Phase 1 ADVANCED-1 trial of TARA-002 for non-muscle invasive bladder cancer (NMIBC) marked an important step toward our goal of bringing a much needed, novel therapeutic option to this underserved population," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Beyond NMIBC, we are encouraged to have recently held a meeting with the U.S. Food and Drug Administration (FDA), where we received preliminary guidance regarding a development path for TARA-002 in lymphatic malformations (LMs). We are currently planning to initiate a Phase 2 clinical trial in this indication subject to alignment with FDA on a clinical trial protocol. We also remain very engaged with the LMs treating community as we work to design a clinical study that will further progress our program for this rare pediatric indication that currently lacks any FDA-approved therapies."

Recent Highlights

TARA-002 in NMIBC

- In March 2022, the Company announced that the first patient was dosed in its Phase 1 ADVANCED-1 clinical trial evaluating TARA-002, an investigational cell-based immunopotentiator, for the treatment of NMIBC.

TARA-002 in LMs

- The Company continues to engage with the Vaccines and Related Products Division of the FDA and recently received preliminary guidance regarding a potential development path for TARA-002 in LMs. The Company is planning to initiate a Phase 2 clinical trial of TARA-002 in this indication subject to alignment with the agency on a clinical trial protocol.

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

- Protara's prospective study to enhance understanding of the incidence of IFALD in patients dependent on parenteral nutrition remains ongoing. The Company expects to use results from the prospective study, as well as its previously completed retrospective study, to inform next steps for the IV Choline Chloride development program.
- With respect to the IV Choline Chloride program, in April 2022, the U.S. Patent and Trademark Office issued to the Company a patent claiming a sterile aqueous choline salt composition with a term expiring in 2041. The Company expects such patent to be listed in the FDA's "Orange Book" in the event of the FDA's approval of the Company's IV Choline Chloride product candidate.

First Quarter 2022 Financial Results

- As of March 31, 2022, cash, cash equivalents and restricted cash were $118.5 million. The Company expects its current cash and cash equivalents will be sufficient to fund its planned operations into mid-2024.
- Research and development (R&D) expenses for the first quarter of 2022 decreased to $5.3 million from $7.0 million during the first quarter of 2021. The decreased R&D expenses were primarily due to decreases in manufacturing and non-clinical expenses associated with TARA-002.
- General and administrative expenses for the first quarter of 2022 decreased to $5.6 million from $6.5 million for the prior year period. The decrease was primarily due to decreases in stock-based compensation.
- For the first quarter of 2022, Protara reported a net loss of $10.8 million, or $0.96 per share, compared with a net loss of $13.5 million, or $1.20 per share, for the same period in 2021. Net loss for the first quarter of 2022 included approximately $1.9 million of stock-based compensation expenses.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of NMIBC and 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 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-2, IL-6, IL-8, IL-10, IL-12, interferon (IFN)-gamma, tumor necrosis factor (TNF)-alpha, granulocyte colony-stimulating factor, and granulocyte-macrophage colony-stimulating factor are secreted by immune cells to induce a strong local inflammatory reaction and destroy the abnormal cells.

About Non-Muscle Invasive Bladder Cancer (NMIBC)

Bladder cancer is the 6th most common cancer in the United States, with 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 (LMs)

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 development plans for its product candidates and plans regarding the timing or outcome of existing or future clinical trials; statements related to expectations regarding interactions with the FDA, including potential alignment with the FDA on clinical trial design for TARA-002 in pediatric LM patients; 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 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 as well as the impact on Protara's contract research organizations, study sites or other clinical partners; 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; the impact of general U.S. and foreign, economic, industry, market, regulatory or political conditions; 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.
Unaudited Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)

	As of	
	March 31, 2022	**December 31, 2021**
Assets		
Current assets:		
Cash and cash equivalents	$ 25,436	$ 35,724
Marketable debt securities	62,155	55,505
Prepaid expenses and other current assets	3,098	1,883
Total current assets	90,689	93,112

Restricted cash, non-current	745	745
Marketable debt securities, non-current	30,889	39,467
Property and equipment, net	1,676	1,719
Operating lease right-of-use asset	6,953	7,171
Goodwill	29,517	29,517
Other assets	787	865
Total assets	$ 161,256	$ 172,596
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 541	$ 954
Accrued expenses	1,450	2,489
Operating lease liability	870	855
Total current liabilities	2,861	4,298
Operating lease liability, non-current	6,160	6,384
Total liabilities	9,021	10,682
Commitments and contingencies (Note 8)		
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, 2022 and December 31, 2021, 8,027 shares issued and outstanding as of March 31, 2022 and December 31, 2021.	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares:		
Common stock, 11,251,927 and 11,235,731 shares issued and outstanding as of March 31, 2022 and December 31, 2021, respectively.	11	11
Additional paid-in capital	257,933	256,126
Accumulated deficit	(104,767)	(94,012)
Accumulated other comprehensive income (loss)	(942)	(211)
Total stockholders' equity	152,235	161,914
Total liabilities and stockholders' equity	$ 161,256	$ 172,596

PROTARA THERAPEUTICS, INC.
Unaudited Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)

	For the Three Months Ended March 31,	
	2022	**2021**
Operating expenses:		
Research and development	$ 5,269	$ 7,040
General and administrative	5,605	6,540
Total operating expenses	10,874	13,580
Loss from operations	(10,874)	(13,580)
Other income (expense), net:		
Interest and investment income	119	115
Other income (expense), net	119	115
Net loss	(10,755)	(13,465)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.96)	$ (1.20)
Weighted-average shares outstanding, basic and diluted	11,250,127	11,226,929
Other comprehensive income (loss):		
Net unrealized (loss) gain on marketable debt securities	(731)	(164)
Other comprehensive income (loss)	(731)	(164)
Comprehensive Loss	$ (11,486)	$ (13,629)

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Source: Protara Therapeutics