Protara Therapeutics Announces First Quarter 2024 Financial Results and Provides Business Update

May 2, 2024

- *Reported positive data from three-month evaluable CIS patients treated across ongoing TARA-002 clinical program in NMIBC*
- *Preliminary data from six-month evaluable patients in ADVANCED-2 trial of TARA-002 in NMIBC expected in 2H 2024*
- *Reached alignment with FDA on registrational path forward for IV Choline Chloride in patients dependent on parenteral nutrition*
- *Current cash resources, together with approximately $45.0 million in gross proceeds from April 2024 private placement, expected to fund operations into 2026*

NEW YORK, May 02, 2024 (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, 2024 and provided a business update.

"We have made significant progress thus far in 2024, and with cash resources expected to fund operations into 2026, we are well positioned to execute our programs in oncology and rare disease," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "We are pleased with the positive three-month data announced last month from our clinical program in patients with non-muscle invasive bladder cancer (NMIBC), which support the potential for TARA-002 to play a meaningful role in the treatment landscape. Looking ahead, we are on track to present interim data from our ADVANCED-2 trial of TARA-002 in patients with NMIBC in the second half of this year."

Mr. Shefferman continued, "We also recently aligned with the U.S. Food and Drug Administration (FDA) on a path forward for intravenous (IV) Choline Chloride in patients dependent on parenteral nutrition (PN). We continued to enroll pediatric patients in our Phase 2 STARBORN-1 trial of TARA-002 in lymphatic malformations (LMs), an underserved population with no FDA-approved therapies."

Recent Progress and Highlights

Corporate Update

- In April 2024, Protara closed a $45.0 million private placement. The offering was led by RA Capital Management and Acorn Bioventures and included participation from new and existing investors such as Boxer Capital, Woodline Partners LP, Catalio Capital Management, StemPoint Capital, Armistice Capital, Velan Capital and a healthcare fund.

TARA-002 in NMIBC

- In April 2024, the Company announced positive data from three-month evaluable carcinoma in situ (CIS) patients treated across its ongoing clinical program of TARA-002 in high-risk NMIBC, including Bacillus Calmette-Guérin (BCG)-unresponsive, BCG-experienced and BCG-naïve patient populations. The overall three-month complete response (CR) rate prior to reinduction for 16 evaluable patients treated across three trials with varying BCG status was 38%, with a CR rate of 63% in CIS-only patients and 13% in patients with CIS +Ta/T1. A 43% CR rate was observed in BCG-Unresponsive/Experienced patients. TARA-002 demonstrated a favorable safety and tolerability profile, with no Grade 3 or greater treatment-related adverse events.
- The Company expects to share preliminary results from a pre-planned risk-benefit analysis of the ongoing Phase 2 open-label ADVANCED-2 trial in the second half of 2024. The Phase 2 open-label ADVANCED-2 trial is assessing intravesical TARA-002 in NMIBC patients with CIS (± Ta/T1) who are BCG-Unresponsive (n=75-100) and BCG-Naïve (n=27). The BCG-Unresponsive cohort has been designed to be registrational aligned with the FDA's 2018 BCG-Unresponsive Non-muscle Invasive Bladder Cancer: Developing Drugs and Biologics for Treatment Guidance for Industry. Trial subjects will receive an induction course of six weekly intravesical instillations, and following mandatory biopsy at three months, will either receive a reinduction course of six weekly intravesical instillations of TARA-002, or the first maintenance course of three weekly installations every three months for an additional 12 months.
- In addition to the ADVANCED-2 trial, the Company intends to assess higher dosing at an 80KE[1] dose and systemic priming prior to initiation of intravesical administration, as well as the combination of TARA-002 with a checkpoint inhibitor in NMIBC patients with CIS.

IV Choline Chloride for Patients on PN

- In April 2024, the Company announced alignment with the FDA on a registrational path forward for IV Choline Chloride in patients dependent on PN. Previously, the Company had been pursuing an indication in intestinal failure-associated liver disease (IFALD) and following feedback from the FDA, is pursuing a broader indication in patients on PN who are or may

become unable to synthesize choline from oral or enteral nutrition sources. The Company expects to advance the development of IV Choline Chloride as a source of choline for adult and adolescent patients on long-term PN.

TARA-002 in LMs

- Dosing continues to progress in STARBORN-1, a Phase 2 clinical trial of TARA-002 in pediatric patients with macrocystic and mixed-cystic LMs. Including an age de-escalation safety lead-in, the trial will enroll approximately 30 patients who will receive up to four injections of TARA-002 spaced approximately six weeks apart. The primary endpoint of the trial is the proportion of participants with macrocystic and mixed cystic LMs who demonstrate clinical success, defined as having either a CR (90% to 100% reduction from baseline in total LM volume) or substantial response (60% to less than 90% reduction in total LM volume) as measured by axial imaging.

First Quarter 2024 Financial Results

- As of March 31, 2024, cash, cash equivalents and investments in marketable debt securities totaled $55.2 million. The Company expects its cash, cash equivalents, and investments in marketable debt securities, together with approximately $42.0 million in net proceeds from its April 2024 private placement, will be sufficient to fund its planned operations and data milestones into 2026.
- Research and development expenses for the first quarter of 2024 increased to $7.7 million from $5.1 million for the prior year period. The increases were primarily due to an increase in expenses related to clinical trial and non-clinical activities for TARA-002 of $1.8 million as well as an increase of $1.1 million in personnel-related expenses, partially offset by a reduction in clinical development activities for Choline of $0.3 million.
- General and administrative expenses for the first quarter of 2024 decreased to $4.1 million from $4.6 million for the prior year period. This decrease was primarily due to a reduction of $0.5 million in personnel-related expenses, inclusive of $0.3 million of stock-based compensation.

- For the first quarter of 2024, Protara incurred a net loss of $11.1 million, or $0.97 per share, compared with a net loss of $9.0 million, or $0.80 per share, for the same period in 2023. Net loss for the first quarter of 2024 included approximately $1.2 million of stock-based compensation expenses.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of NMIBC and of 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 approved in Taiwan by Chugai Pharmaceutical Co., Ltd. Protara has successfully shown 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 pro-inflammatory response with release of cytokines such as tumor necrosis factor (TNF)-alpha, interferon (IFN)-gamma, IL-1b, IL-6, IL-12, granulocyte-macrophage colony-stimulating factor (GM-CSF) and natural killer cells. TARA-002 also directly kills tumor cells and triggers a host immune response by inducing immunogenic cell death, which further enhances the antitumor immune response.

About Non-Muscle Invasive Bladder Cancer (NMIBC)

Bladder cancer is the sixth 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 three 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

IV Choline Chloride is an investigational, intravenous phospholipid substrate replacement therapy initially in development for patients receiving parenteral nutrition. Choline is a known important substrate for phospholipids that are critical for healthy liver function and also plays an important role in modulating gene expression, cell membrane signaling, brain development and neurotransmission, muscle function, and bone health. PN patients are unable to synthesize choline from enteral nutrition sources, and there are currently no available PN formulations containing choline. Approximately 80 percent of PN-dependent patients are choline-deficient and have some degree of liver damage, which can lead to hepatic failure. There are currently no available PN formulations containing choline. In the U.S. alone, there are approximately 40,000 patients on long-term parenteral nutrition who would benefit from an IV formulation of choline. IV Choline Chloride has the potential to become the first FDA approved IV choline formulation for PN patients. IV Choline Chloride has been granted Orphan Drug Designation by the FDA for the prevention of choline deficiency in PN patients. The Company was issued a U.S. patent claiming a choline composition with a term expiring in 2041.

About Protara Therapeutics, Inc.

Protara is a clinical-stage biotechnology company committed to advancing transformative therapies for people with cancer and rare diseases. Protara's portfolio includes its lead candidate, TARA-002, an investigational cell-based therapy in development for the treatment of non-muscle invasive bladder cancer (NMIBC) and lymphatic malformations (LMs). The Company is evaluating TARA-002 in an ongoing Phase 2 trial in NMIBC patients with carcinoma in situ (CIS) who are unresponsive or naïve to treatment with Bacillus Calmette-Guérin (BCG), as well as a Phase 2 trial in pediatric patients with LMs. Additionally, Protara is developing IV Choline Chloride, an investigational phospholipid substrate replacement for patients on parenteral nutrition who are otherwise unable to meet their choline needs via oral or enteral routes. For more information, visit www.protaratx.com.

References

1. Klinische Einheit, or KE, is a German term indicating a specified weight of dried cells in a vial.

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; Protara's financial position; 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; 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; failure to attract and retain management and key personnel; the impact of general U.S. and foreign, economic, industry, market, regulatory, political or public health 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. AND SUBSIDIARIES
Unaudited Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)

	As of	
	March 31, 2024	**December 31, 2023**
Assets		
Current assets:		
Cash and cash equivalents	$ 52,231	$ 39,586
Marketable debt securities	2,992	25,994
Prepaid expenses and other current assets	2,690	3,125
Total current assets	57,913	68,705
Restricted cash, non-current	745	745
Property and equipment, net	1,213	1,296
Operating lease right-of-use asset	5,018	5,264
Other assets	3,245	2,944
Total assets	$ 68,134	$ 78,954
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 972	$ 2,434
Accrued expenses and other current liabilities	3,529	2,732
Operating lease liability	1,000	983
Total current liabilities	5,501	6,149
Operating lease liability, non-current	4,227	4,484
Total liabilities	9,728	10,633
Commitments and contingencies (Note 9)		
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, 2024 and December 31, 2023, 7,991 shares issued and outstanding as of March 31, 2024 and December 31, 2023.	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares: Common stock, 11,433,837 and 11,364,903 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively.	11	11
Additional paid-in capital	269,875	268,725
Accumulated deficit	(211,479)	(200,384)
Accumulated other comprehensive income (loss)	(1)	(31)
Total stockholders' equity	58,406	68,321
Total liabilities and stockholders' equity	$ 68,134	$ 78,954

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

	For the Three Months Ended March 31,	
	2024	**2023**
Operating expenses:		
Research and development	$ 7,748	$ 5,143
General and administrative	4,103	4,589
Total operating expenses	11,851	9,732
Loss from operations	(11,851)	(9,732)
Other income (expense), net:		
Interest and investment income	756	687
Other income (expense), net	756	687
Net loss	$ (11,095)	$ (9,045)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.97)	$ (0.80)
Weighted-average shares outstanding, basic and diluted	11,420,948	11,303,869
Other comprehensive income (loss):		
Net unrealized gain (loss) on marketable debt securities	30	219
Other comprehensive income (loss)	30	219
Comprehensive loss	$ (11,065)	$ (8,826)

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