Protara Therapeutics Announces First Quarter 2025 Financial Results and Provides Business Update

May 8, 2025

- *Reported positive interim results demonstrating durable responses in the ongoing Phase 2 ADVANCED-2 trial of TARA-002 in NMIBC*
- *Results from planned interim analysis of approximately 25 six-month evaluable BCG-Unresponsive patients expected by the end of 2025*
- *Dosing of first patient in THRIVE-3 registrational trial of IV Choline Chloride in patients dependent on parenteral support expected in Q3 2025*
- *Strengthened leadership team with key appointments of Leonardo Viana Nicacio, M.D., as Chief Medical Officer, and Shane Williams, Ph.D., as VP, Head of Human Resources, Chief People Officer*
- *Cash, cash equivalents and investments of $158 million as of March 31, 2025, expected to support planned operations into 2027*

NEW YORK, May 08, 2025 (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, 2025, and provided a business update.

“We have made significant progress thus far in 2025, notably with the recent presentation of positive interim results from our ADVANCED-2 trial of TARA-002 in BCG-Unresponsive and BCG-Naïve patients which demonstrated durable 12-month landmark responses,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We believe TARA-002 is well positioned to make a meaningful difference in the lives of patients with non-muscle invasive bladder cancer (NMIBC). In addition to our NMIBC program, we are pleased with the continued progress we have made across our rare disease programs and look forward to several exciting data milestones in the coming months.”

Recent Progress and Highlights

TARA-002 in NMIBC

- At the American Urological Association (AUA) 2025 Annual Meeting in April, the Company announced positive updated interim results from the ADVANCED-2 trial in evaluable NMIBC patients with carcinoma in situ or CIS (± Ta/T1) who are Bacillus Calmette-Guérin (BCG)-Unresponsive and BCG-Naïve. As of the April 16, 2025 data cutoff:
  - TARA-002 demonstrated a complete response (CR) rate at any time of 100% (5/5) and 67% (2/3) at 12 months in the cohort of BCG-Unresponsive patients. As previously communicated, the BCG-Unresponsive cohort is designed to be registrational in alignment with the 2024 BCG-Unresponsive Non-muscle Invasive Bladder Cancer: Developing Drugs and Biological Products for Treatment Draft Guidance for Industry issued by the U.S. Food and Drug Administration (FDA).
  - In the proof-of-concept BCG-Naïve cohort of patients, TARA-002 demonstrated a CR rate at any time of 76% (16/21) and a CR rate of 43% (3/7) at 12 months.
  - The majority of adverse events were Grade 1 and transient, with no Grade 3 or greater treatment-related adverse events as assessed by study investigators.
- Interim results from approximately 25 six-month evaluable BCG-Unresponsive patients are expected to be announced by the end of 2025.
- Following regulatory alignment with the FDA, the Company expects to provide an update on the design of its planned BCG-Naïve registrational trial in the second half of 2025.
- Protara continues to investigate subcutaneous dosing through priming and maintenance combined with intravesical dosing, as well as exploring combination treatment with TARA-002 in NMIBC patients with CIS.

IV Choline Chloride for Patients on Parenteral Support (PS)

- The Company plans to initiate THRIVE-3, a registrational Phase 3 clinical trial, in the third quarter of 2025. THRIVE-3 is a seamless Phase 2b/3 trial with a dose confirmation portion (n=24) followed by a double-blinded, randomized, placebo-controlled portion to assess the efficacy and safety of IV Choline Chloride over 24 weeks in adolescents and adults on

long-term PS when oral or enteral nutrition is not possible, insufficient, or contraindicated (n=105). IV Choline Chloride was previously granted Fast Track designation by the FDA.

TARA-002 in LMs

- Dosing continues to progress in the Phase 2 STARBORN-1 trial of TARA-002 in pediatric patients with macrocystic and mixed cystic LMs and the Company intends to provide an interim update from the trial in the second half of 2025. The Company previously announced the completion of the study's first safety cohort, in which TARA-002 showed promising results and was generally well-tolerated.

Corporate Update

- In April 2025, Protara strengthened its leadership team with the appointments of Leonardo Viana Nicacio, M.D., as Chief Medical Officer, and Shane Williams, Ph.D., as Vice President, Head of Human Resources, Chief People Officer. Dr. Nicacio brings to Protara nearly 20 years of broad oncology, drug development, regulatory and commercial experience across leading biopharmaceutical and health technology companies, and most recently served as Head of Clinical Development and Global Medical Affairs at Stemline Therapeutics. Dr. Williams brings a strong track record of driving growth, leading transformational change, and building high-performing teams across innovative life science organizations. He most recently served as Chief People Officer at Century Therapeutics.

First Quarter 2025 Financial Results

- As of March 31, 2025, unrestricted cash and cash equivalents and investments in marketable debt securities totaled $157.5 million. The Company expects its cash, cash equivalents, and investments in marketable debt securities will be sufficient to fund operations into 2027.

- Research and development expenses for the first quarter of 2025 increased to $9.1 million from $7.7 million for the prior year period. The increase was primarily due to a $2.6 million increase in clinical trial activities for TARA-002 and IV Choline, offset by a $1.2 million decrease in indirect expenses.

- General and administrative expenses for the first quarter of 2025 increased to $5.0 million from $4.1 million for the prior year period. This increase was primarily due to a $0.4 million increase in personnel-related costs as well as an increase of professional fees of $0.4 million.

- For the first quarter of 2025, Protara incurred a net loss of $11.9 million, or $0.29 per share, compared with a net loss of $11.1 million, or $0.97 per share, for the same period in 2024. Net loss for the first quarter of 2025 included approximately $0.8 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 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-6, IL-10, IL-12. 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 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 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 in development for patients receiving parenteral support (PS). Choline is a known important substrate for phospholipids that are critical for healthy liver function that also play an important role in modulating gene expression, cell membrane signaling, brain development and neurotransmission, muscle function, and bone health. PS patients are

unable to synthesize choline from enteral nutrition sources, and there are currently no available PS formulations containing choline. Approximately 80% of patients dependent on PS are choline-deficient and of those approximately 63% have some degree of liver dysfunction, which can lead to hepatic failure. Every year in the U.S. there are approximately 90,000 people who require PS at home and of those approximately 30,000 are on long-term PS. IV Choline Chloride has the potential to become the first U.S. Food and Drug Administration (FDA) approved IV choline formulation for PS patients. It has been granted Orphan Drug Designation by the FDA for the prevention and/or treatment of choline deficiency in patients on long-term PN and been granted Fast Track Designation as a source of choline when oral or enteral nutrition is not possible, insufficient, or contraindicated. The U.S. Patent and Trademark Office has issued us a U.S. patent claiming a choline composition and a U.S. patent claiming a method for treating choline deficiency with a choline composition, each 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.

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 (including reporting data from approximately 25 6-month evaluable BCG-Unresponsive patients by the end of 2025); statements related to expectations regarding interactions with the U.S. Food and Drug Administration (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 and future periods. 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, 2025	**December 31, 2024**
Assets		
Current assets:		
Cash and cash equivalents	$ 91,461	$ 162,798
Marketable debt securities	32,902	7,494
Prepaid expenses and other current assets	2,169	1,863
Total current assets	126,532	172,155
Restricted cash, non-current	745	745
Marketable debt securities, non-current	33,154	-
Property and equipment, net	988	1,027
Operating lease right-of-use asset	3,992	4,255
Other assets	3,148	3,272
Total assets	$ 168,559	$ 181,454
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,310	$ 4,429
Accrued expenses and other current liabilities	3,538	5,408
Operating lease liability	1,170	1,124
Total current liabilities	7,018	10,961

Operating lease liability, non-current	3,057	3,359
Total liabilities	10,075	14,320
Commitments and contingencies		
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, 2025 and December 31, 2024, 5,615 and 7,991 shares issued and outstanding as of March 31, 2025 and December 31, 2024, respectively	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares:		
Common stock, 38,577,813 and 35,044,772 shares issued and outstanding as of March 31, 2025 and December 31, 2024, respectively	39	35
Additional paid-in capital	415,250	412,077
Accumulated deficit	(256,894)	(244,980)
Accumulated other comprehensive income (loss)	89	2
Total stockholders' equity	158,484	167,134
Total liabilities and stockholders' equity	$ 168,559	$ 181,454

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,	
	2025	**2024**
Operating expenses:		
Research and development	$ 9,148	$ 7,748
General and administrative	4,976	4,103
Total operating expenses	14,124	11,851
Income (Loss) from operations	(14,124)	(11,851)
Other income (expense), net:		
Interest and investment income (expenses)	1,729	756
Other income (expense)	481	-
Other income (expense), net	2,210	756
Net income (loss)	$ (11,914)	$ (11,095)
Other comprehensive income (loss):		
Net unrealized gain (loss) on marketable debt securities	87	30
Other comprehensive income (loss)	87	30
Comprehensive income (loss)	$ (11,827)	$ (11,065)
Net income (loss) per share attributable to common stockholders, basic and diluted	$ (0.29)	$ (0.97)
Weighted-average shares outstanding, basic and diluted	40,707,937	11,420,948

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Source: Protara Therapeutics