Protara Therapeutics Announces Fourth Quarter and Full Year 2024 Financial Results and Provides a Business Update

March 5, 2025

- *Reported positive six-month data from ADVANCED-2 trial of TARA-002 in patients with NMIBC; Announcement of initial data from 12-month evaluable patients expected by mid-2025*
- *Dosing of first patient in THRIVE-3 registrational trial of IV Choline Chloride in patients dependent on parenteral support expected in 1H 2025*
- *On track to provide an interim update from the Phase 2 STARBORN-1 trial of TARA-002 in pediatric LMs patients by the end of 1H 2025*
- *Cash, cash equivalents and investments of $170 million as of December 31, 2024, including gross proceeds from $100 million public offering in December 2024, expected to support planned operations into 2027*

NEW YORK, March 05, 2025 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today provided a business update and announced financial results for the fourth quarter and full year ended December 31, 2024.

“Following a year of significant progress and execution across our pipeline, we remain well positioned to deliver on our mission to bring transformative therapies to patients with cancer and rare diseases,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “Notably, supported by positive six-month data from our Phase 2 ADVANCED-2 trial in non-muscle invasive bladder cancer (NMIBC), we continue to believe TARA-002 could represent a differentiated, meaningful addition to the treatment paradigm, both as a monotherapy and in potential combination with other agents. We continue to expect to report initial data from 12-month evaluable patients in our ADVANCED-2 trial by mid-year.”

Mr. Shefferman added, “Beyond our NMIBC program, we remain on track to commence our pivotal THRIVE-3 trial of intravenous (IV) Choline Chloride in the first half of the year. We also look forward to providing an interim update from our ongoing Phase 2 STARBORN-1 trial of TARA-002 in lymphatic malformations (LMs) by the end of the first half of 2025. With our cash runway extending into 2027, we look forward to achieving many critical milestones across all of our development programs as we work to deliver new treatment options to patients in need.”

Recent Progress and Highlights

- The Company is on track to report 12-month data by mid-year from its ongoing Phase 2 open-label 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. In December 2024, the Company reported positive interim results in which the complete response (CR) rate across BCG exposures was 72% (13/18) at six months and 70% (14/20) at any time, with 100% (9/9) of patients maintaining a CR from three months to six months. In addition, two of three patients maintained a CR at nine months. TARA-002 showed a favorable safety profile, with no Grade 2 or greater treatment-related adverse events and no treatment discontinuations due to adverse events.
- Interim results from approximately 25 six-month evaluable BCG-Unresponsive patients are expected to be announced by the end of 2025. 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).
- Following regulatory alignment with the FDA, the Company expects to provide an update on the design of its planned BCG-Naïve registrational trial by the end of the first half of 2025.
- Protara continues to investigate systemic subcutaneous dosing through priming and maintenance combined with intravesical dosing, as well as exploring combination treatment with TARA-002 in NMIBC patients with CIS. Given what has been observed to date with TARA-002's mechanism of action and strong safety profile, the Company believes it holds significant potential for use in both systemic dosing and combination therapy and is finalizing various opportunities.

IV Choline Chloride for Patients on Parenteral Support (PS)

- The Company plans to initiate THRIVE-3, a registrational Phase 3 clinical trial, in the first half 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=100). IV Choline Chloride was previously granted Fast Track designation by the FDA.

TARA-002 in LMs

- Protara remains on track to provide an interim update on the Phase 2 STARBORN-1 trial of TARA-002 in pediatric patients with macrocystic and mixed cystic LMs by the end of the first 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.

Fourth Quarter and Full Year 2024 Financial Results

- As of December 31, 2024, cash and cash equivalents, and marketable debt securities totaled $170.3 million. The Company expects its cash and cash equivalents, and marketable debt securities will be sufficient to fund its planned operations and data milestones into 2027.
- Research and development expenses for the fourth quarter of 2024 increased to $9.5 million from $6.4 million for the prior year period, and for the full year increased to $31.7 million compared to $25.0 million for 2023. The fourth quarter and full year increases were primarily due to an increase in expenses related to clinical trial and non-clinical activities for TARA-002 and IV Choline Chloride.
- General and administrative expenses for the fourth quarter of 2024 increased to $4.8 million from $4.7 million for the prior year period, and for the full year decreased to $17.5 million compared to $18.6 million for 2023. The full year decrease was primarily due to a decrease in personnel-related expenses.
- For the fourth quarter of 2024, Protara incurred a net loss of $12.8 million, or $0.48 per share, compared with a net loss of $10.2 million, or $0.90 per share, for the same period in 2023. Net loss for the year ended December 31, 2024 was $44.6 million, or $2.17 per share, compared with a net loss of $40.4 million, or $3.57 per share, for the year ended December 31, 2023. Net loss for the fourth quarter of 2024 included approximately $0.9 million of stock-based compensation expenses. Net loss for the year ended December 31, 2024 included approximately $4.1 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 support 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 initial data from 12-month evaluable patients in mid-2025); 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 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; the impact of government laws and regulations, including the impacts on FDA’s staffing, resources and ability to timely review and process regulatory submissions and the impacts of any executive orders or tariffs; 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.
Consolidated Balance Sheets
(in thousands, except share and per share data)

	December 31,	
	2024	**2023**
Assets		
Current assets:		
Cash and cash equivalents	$ 162,798	$ 39,586
Marketable debt securities	7,494	25,994
Prepaid expenses and other current assets	1,863	3,125
Total current assets	172,155	68,705
Restricted cash , non-current	745	745
Property and equipment, net	1,027	1,296
Operating lease right-of-use asset	4,255	5,264
Other assets	3,272	2,944
Total assets	$ 181,454	$ 78,954
Liabilities and Stockholders’ Equity		
Current liabilities:		
Accounts payable	$ 4,429	$ 2,434
Accrued expenses and other current liabilities	5,408	2,732
Operating lease liability	1,124	983
Total current liabilities	10,961	6,149
Operating lease liability, non-current	3,359	4,484
Total liabilities	14,320	10,633
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 December 31, 2024 and 2023, 7,991 shares issued and outstanding as of December 31, 2024 and 2023.	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares:		
Common stock, 35,044,772 and 11,364,903 shares issued and outstanding as of December 31, 2024 and 2023, respectively.	35	11
Additional paid in capital	412,077	268,725
Accumulated deficit	(244,980)	(200,384)

Accumulated other comprehensive income (loss)	2	(31)
Total stockholders' equity	167,134	68,321
Total liabilities and stockholders' equity	$ 181,454	$ 78,954

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

	(Unaudited) For the Three Months Ended December 31,		(Audited) For the Years Ended December 31,	
	2024	**2023**	**2024**	**2023**
Operating expenses:				
Research and development	$ 9,499	$ 6,381	$ 31,704	$ 24,989
General and administrative	4,813	4,660	17,450	18,624
Total operating expenses	14,312	11,041	49,154	43,613
Income (Loss) from operations	(14,312)	(11,041)	(49,154)	(43,613)
Other income (expense), net:				
Interest and investment income	1,156	820	4,171	3,193
Other income	387	-	387	-
Other income (expense), net	1,543	820	4,558	3,193
Net income (loss)	$ (12,769)	$ (10,221)	$ (44,596)	$ (40,420)
Other comprehensive income (loss):				
Net unrealized gain (loss) on marketable debt securities	(27)	134	33	657
Other comprehensive income (loss)	(27)	134	33	657
Comprehensive income (loss)	$ (12,796)	$ (10,087)	$ (44,563)	$ (39,763)
Net income (loss) per share attributable to common stockholders, basic and diluted	$ (0.48)	$ (0.90)	$ (2.17)	$ (3.57)
Weighted-average shares outstanding, basic and diluted	26,432,563	11,364,903	20,592,847	11,331,338

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