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

November 10, 2025

- *Results from an interim analysis in the Phase 2 STARBORN-1 trial of TARA-002 in pediatric LMs patients remain on track for 4Q 2025*
- *Expect to present interim analysis from approximately 25 six-month evaluable BCG-Unresponsive NMIBC patients in the ongoing ADVANCED-2 trial in 1Q 2026*
- *Dosing of first patient in THRIVE-3 registrational trial of IV Choline Chloride in patients dependent on parenteral support now expected by year-end 2025*
- *Cash, cash equivalents and investments of approximately $134 million as of September 30, 2025, expected to support planned operations into mid-2027*

NEW YORK, Nov. 10, 2025 (GLOBE NEWSWIRE) -- Protara Therapeutics, Inc. (Nasdaq: TARA) (“Protara” or the “Company”), a clinical-stage company developing transformative therapies for the treatment of cancer and rare diseases, today announced financial results for the third quarter ended September 30, 2025, and provided a business update.

“We have made important advancements across our clinical programs this year, and with many significant milestones in the near future, we remain focused on disciplined execution across our pipeline of transformative therapies for patients with cancer and rare diseases,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We continue to make progress in our ADVANCED-2 trial of TARA-002 in non-muscle invasive bladder cancer (NMIBC) and remain on track to report interim results from approximately 25 BCG-Unresponsive patients in the first quarter of 2026. With a favorable safety profile and its differentiated ease of administration, we believe TARA-002 has the potential to become a meaningful addition to the evolving NMIBC treatment landscape.”

Mr. Shefferman added, “Across our rare disease pipeline, we expect to provide an interim update from our STARBORN-1 trial in pediatric patients with lymphatic malformations (LMs) and dose the first patient in our registrational THRIVE-3 trial of IV Choline Chloride in the fourth quarter of 2025.”

Recent Progress and Highlights

TARA-002 in NMIBC

- The Company anticipates reporting interim results from approximately 25 six-month evaluable NMIBC patients with carcinoma in situ or CIS (± Ta/T1) who are BCG-Unresponsive in the first quarter of 2026.
- The Company expects to provide an update on ongoing discussions with the U.S. Food and Drug Administration (FDA) related to the BCG-Naïve opportunity in the fourth quarter of 2025.
- Protara continues to evaluate subcutaneous dosing through priming and maintenance combined with intravesical dosing, as well as exploring combination treatments with TARA-002 in NMIBC patients with CIS.

IV Choline Chloride for Patients on Parenteral Support (PS)

- The Company now expects to initiate dosing in THRIVE-3, a registrational Phase 3 clinical trial, by the end of 2025 due to administrative and funding challenges in academic sites in the U.S.
- 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 intravenous (IV) Choline Chloride over 24 weeks in adolescents and adults on long-term parenteral support (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

- Protara continues to advance the Phase 2 STARBORN-1 trial of TARA-002 in pediatric patients with macrocystic and mixed cystic LMs, and the Company expects to provide an interim update from the trial in the fourth quarter 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 November 2025, Protara was recognized among *BioSpace's* Best Places to Work, which highlights organizations that excel in culture, career growth, leadership, and innovation.

Third Quarter 2025 Financial Results

- As of September 30, 2025, unrestricted cash and cash equivalents and investments in marketable debt securities totaled $133.6 million. The Company expects its cash, cash equivalents, and investments in marketable debt securities will be sufficient to fund operations into mid-2027.
- Research and development expenses for the third quarter of 2025 increased to $9.6 million from $8.1 million for the prior year period. The increase was primarily due to a $0.9 million increase in startup costs for our IV Choline Chloride THRIVE-3 clinical trial.
- General and administrative expenses for the third quarter of 2025 increased to $5.2 million from $4.3 million for the prior year period. This increase was primarily due to an increase of $0.7 million in personnel-related expenses.
- For the third quarter of 2025, Protara incurred a net loss of $13.3 million, or $0.31 per share, compared with a net loss of $11.2 million, or $0.50 per share, for the same period in 2024.

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 78% 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 has 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 Protara 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 the timing of any particular phases of such trials and the timing of the announcement of any data produced during such trials or phases thereof); 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	
	September 30, 2025	**December 31, 2024**
Assets		
Current assets:		
Cash and cash equivalents	$ 12,551	$ 162,798
Marketable debt securities	120,119	7,494
Prepaid expenses and other current assets	2,974	1,863
Total current assets	135,644	172,155
Restricted cash, non-current	745	745
Marketable debt securities, non-current	957	-
Property and equipment, net	828	1,027
Operating lease right-of-use asset	3,452	4,255
Other assets	3,010	3,272
Total assets	$ 144,636	$ 181,454
Liabilities and Stockholders’ Equity		
Current liabilities:		
Accounts payable	$ 4,504	$ 4,429
Accrued expenses and other current liabilities	4,185	5,408
Operating lease liability	1,220	1,124
Total current liabilities	9,909	10,961
Operating lease liability, non-current	2,436	3,359
Total liabilities	12,345	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 September 30, 2025 and December 31, 2024, 5,615 and 7,991 shares issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares:		
Common stock, 38,587,260 and 35,044,772 shares issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	39	35
Additional paid-in capital	417,221	412,077
Accumulated deficit	(285,112)	(244,980)
Accumulated other comprehensive income (loss)	143	2
Total stockholders’ equity	132,291	167,134
Total liabilities and stockholders’ equity	$ 144,636	$ 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 September 30,		For the Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Operating expenses:				
Research and development	$ 9,591	$ 8,070	$ 29,509	$ 22,205
General and administrative	5,169	4,260	15,961	12,637
Total operating expenses	14,760	12,330	45,470	34,842
Income (Loss) from operations	(14,760)	(12,330)	(45,470)	(34,842)
Other income (expense), net:				
Interest and investment income (expense)	1,502	1,111	4,857	3,015
Other income (expense)	-	-	481	-
Other income (expense), net	1,502	1,111	5,338	3,015
Net income (loss)	$ (13,258)	$ (11,219)	$ (40,132)	$ (31,827)
Other comprehensive income (loss):				
Net unrealized gain (loss) on marketable debt securities	66	29	141	60
Other comprehensive income (loss):	66	29	141	60
Comprehensive income (loss)	$ (13,192)	$ (11,190)	$ (39,991)	$ (31,767)
Net income (loss) per share attributable to common stockholders, basic and diluted	$ (0.31)	$ (0.50)	$ (0.96)	$ (1.74)
Weighted-average shares outstanding, basic and diluted	42,272,104	22,329,772	41,756,028	18,342,566

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