Protara Therapeutics Announces Third Quarter 2024 Financial Results and Provides a Business Update

November 12, 2024

- *On track to report interim data in six-month evaluable patients in ADVANCED-2 trial of TARA-002 in NMIBC in 4Q 2024*
- *Expect to report interim 12-month data in ADVANCED-2 trial of TARA-002 in NMIBC in mid-2025*
- *In pediatric LMs patients, TARA-002 demonstrated encouraging results and was generally well-tolerated in the first cohort of Phase 2 STARBORN-1 trial; initial results from next cohort expected in 1H 2025*
- *Received Fast Track designation from the U.S. FDA for IV Choline Chloride; expect to dose first patient in THRIVE-3 registrational trial in 1Q 2025*

NEW YORK, Nov. 12, 2024 (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 third quarter ended September 30, 2024.

"We continue to make significant progress advancing all of our ongoing programs as we strive toward our goal of bringing life-changing therapies to patients impacted by cancer and rare diseases," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Notably, we remain on track to report interim data in the fourth quarter of 2024 from our ADVANCED-2 study of TARA-002 in patients with non-muscle invasive bladder cancer (NMIBC). Given its ease of use, favorable safety profile, and encouraging early clinical data, we believe TARA-002 could be an impactful addition to the NMIBC treatment landscape."

Mr. Shefferman added, "In addition to our NMIBC program, we remain on track to commence the pivotal THRIVE-3 study of intravenous (IV) Choline Chloride, for which we recently received Fast Track designation from the U.S. Food and Drug Administration (FDA), in the first quarter of 2025. Finally, we continue to expect interim data in the first half of 2025 from our ongoing Phase 2 STARBORN-1 trial of TARA-002 in lymphatic malformations (LMs), a rare condition primarily impacting children for which there are no FDA approved therapies."

Recent Progress and Highlights

TARA-002 in NMIBC

- The Company remains on track to report results from a pre-planned interim analysis of six-month evaluable patients in the ongoing Phase 2 open-label ADVANCED-2 trial later this quarter. The ADVANCED-2 trial is assessing intravesical TARA-002 in NMIBC patients with carcinoma in situ or CIS (± Ta/T1) who are Bacillus Calmette-Guérin (BCG)-unresponsive (n≈100) and BCG-Naïve (n=27). The BCG-Unresponsive cohort has been designed to be registrational in alignment with the FDA's 2024 BCG-Unresponsive Non-muscle Invasive Bladder Cancer: Developing Drugs and Biological Products for Treatment Draft Guidance for Industry.
- The Company expects to report preliminary results in mid-2025 from 12-month evaluable patients in the ongoing Phase 2 open-label ADVANCED-2 trial.
- The Company continues to explore systemic priming dosing prior to initiation of intravesical administration, as well as combination therapy with TARA-002 in NMIBC patients with CIS. Given TARA-002's mechanism of action and safety profile, the Company believes it has strong potential for use in combination therapy and is working to determine various opportunities for the clinical program.

IV Choline Chloride for Patients on Parenteral Support (PS)

- In October 2024, Protara announced that the FDA granted Fast Track designation to IV Choline Chloride, the Company's investigational IV phospholipid substrate replacement therapy, in patients for whom oral or enteral nutrition is not possible, insufficient, or contraindicated. Protara is currently developing IV Choline Chloride as a source of choline for adult and adolescent patients on PS.
- In September 2024, the Company announced results from THRIVE-1, a prospective, observational study evaluating the prevalence of choline deficiency and liver injury in patients dependent on PS. The study found that 78% of patients who are dependent on PS were choline deficient, with 63% of these patients demonstrating liver dysfunction, including steatosis, cholestasis, and hepatobiliary injury.
- In September 2024, the U.S. Patent and Trademark Office issued the Company a second patent in this program, claiming a method for treating choline deficiency with a choline composition with a term until 2041.
- The Company expects to begin dosing patients in the THRIVE-3 registrational trial in the first quarter of 2025.

TARA-002 in LMs

- In September 2024, Protara announced completion of the first safety cohort of the ongoing Phase 2 STARBORN-1 clinical trial of TARA-002 in pediatric patients with macrocystic and mixed cystic LMs. Of the three patients treated in the first cohort, which enrolled individuals six years to less than 18 years of age, two patients treated with TARA-002, including one with a macrocystic LM and one with a ranula (a type of maxillofacial cyst) achieved a complete response after receiving one dose of TARA-002. TARA-002 was generally well-tolerated. Enrollment is underway in additional cohorts, with initial results expected in the first half of 2025.

Third Quarter 2024 Financial Results

- As of September 30, 2024, cash, cash equivalents, and investments in marketable debt securities totaled approximately $81.5 million. The Company expects its cash, cash equivalents, and investments in marketable debt securities will be sufficient to fund its planned operations and data milestones into 2026.
- Research and development expenses for the third quarter of 2024 increased to $8.1 million from $6.2 million for the prior year period. This increase was primarily due to an increase in expenses related to TARA-002 of $1.5 million as well as an increase in expenses related to IV Choline Chloride of $0.4 million.
- General and administrative expenses for the third quarter of 2024 decreased to $4.3 million from $4.5 million for the prior year period. This decrease was primarily due to a reduction in personnel-related expenses of $0.6 million, offset partially by increases in market development, business development, and investor relations activities of $0.3 million.
- For the third quarter of 2024, Protara incurred a net loss of $11.2 million, or $0.50 per share, compared with a net loss of $9.9 million, or $0.87 per share, for the same period in 2023. Net loss for the third quarter of 2024 included approximately $0.9 million of stock-based compensation expenses.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of NMIBC and 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 have some degree of liver damage, 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 and has been granted Orphan Drug Designation by the FDA for the prevention of choline deficiency in PS patients. The U.S. Patent and Trademark Office has issued the Company 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.

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	
	September 30, 2024	**December 31, 2023**
Assets		
Current assets:		
Cash and cash equivalents	$ 51,757	$ 39,586
Marketable debt securities	29,742	25,994
Prepaid expenses and other current assets	3,583	3,125
Total current assets	85,082	68,705
Restricted cash, non-current	745	745
Property and equipment, net	1,109	1,296
Operating lease right-of-use asset	4,514	5,264
Other assets	2,640	2,944
Total assets	$ 94,090	$ 78,954
Liabilities and Stockholders’ Equity		
Current liabilities:		
Accounts payable	$ 2,295	$ 2,434
Accrued expenses and other current liabilities	5,268	2,732
Operating lease liability	1,079	983
Total current liabilities	8,642	6,149
Operating lease liability, non-current	3,657	4,484
Total liabilities	12,299	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 September 30, 2024 and December 31, 2023, 7,991 shares issued and outstanding as of September 30, 2024 and December 31, 2023.	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares: Common stock, 20,629,772 and 11,364,903 shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively.	21	11
Additional paid-in capital	313,952	268,725
Accumulated deficit	(232,211)	(200,384)
Accumulated other comprehensive income (loss)	29	(31)

Total stockholders' equity	81,791	68,321
Total liabilities and stockholders' equity	$ 94,090	$ 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 September 30,		For the Nine Months Ended September 30,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 8,070	$ 6,218	$ 22,205	$ 18,608
General and administrative	4,260	4,482	12,637	13,964
Total operating expenses	12,330	10,700	34,842	32,572
Loss from operations	(12,330)	(10,700)	(34,842)	(32,572)
Other income (expense), net:				
Interest and investment income	1,111	840	3,015	2,373
Other income (expense), net	1,111	840	3,015	2,373
Net income (loss)	$ (11,219)	$ (9,860)	$ (31,827)	$ (30,199)
Net income (loss) per share attributable to common stockholders, basic and diluted	$ (0.50)	$ (0.87)	$ (1.74)	$ (2.67)
Weighted-average shares outstanding, basic and diluted	22,329,772	11,347,887	18,342,566	11,320,027
Other comprehensive income (loss):				
Net unrealized gain (loss) on marketable debt securities	29	171	60	523
Other comprehensive income (loss)	29	171	60	523
Comprehensive income (loss)	$ (11,190)	$ (9,689)	$ (31,767)	$ (29,676)

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