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

August 6, 2024

- *On track to report preliminary data from six-month evaluable patients in ADVANCED-2 trial of TARA-002 in NMIBC in Q4 2024*
- *Expect to dose first patient in pivotal trial for IV Choline Chloride in Q1 2025*
- *Current cash resources are approximately $90 million, including net proceeds from April 2024 private placement, expected to fund operations into 2026*

NEW YORK, Aug. 06, 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 second quarter ended June 30, 2024.

"The first half of the year has been marked by significant progress as we advance our mission to deliver transformative therapies to patients with cancer and rare diseases," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Notably, we were pleased with our recent announcement of positive three-month data from our TARA-002 program in patients with non-muscle invasive bladder cancer (NMIBC). We believe our growing clinical dataset continues to support the potential of TARA-002 to serve as a meaningful addition to the NMIBC treatment landscape and look forward to sharing interim data from our ADVANCED-2 trial in the fourth quarter of this year. We also continue to focus on identifying the best agent for combination as we believe TARA-002's mechanism of action and safety profile give it strong potential for use in combination therapy in NMIBC."

Mr. Shefferman continued, "Beyond the NMIBC program, we announced alignment with the FDA on a path forward for intravenous (IV) Choline Chloride for patients on long-term parenteral nutrition (PN), and we expect to start dosing patients in our pivotal trial in the first quarter of 2025. Finally, enrollment continues in our Phase 2 STARBORN-1 trial of TARA-002 in pediatric patients with lymphatic malformations (LMs), an underserved population with no U.S. Food and Drug Administration (FDA)-approved therapies."

Recent Progress and Highlights

TARA-002 in NMIBC

- The Company expects to share preliminary results from a pre-planned risk-benefit analysis from approximately ten 6-month evaluable patients in the ongoing Phase 2 open-label ADVANCED-2 trial in the fourth quarter of 2024. 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 aligned with the FDA's 2018 BCG-Unresponsive Non-muscle Invasive Bladder Cancer: Developing Drugs and Biologics for Treatment Guidance for Industry.
- In addition to the ADVANCED-2 trial, the Company is assessing higher dosing at an 80KE[1] dose and has completed enrollment of a three-patient exploratory dosing cohort with data expected by the end of this year. To date, no differences in safety profile have been observed between the 40KE and the 80KE doses.
- 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 the best possible options for the clinical program.

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 who are or may become unable to synthesize choline from oral or enteral nutrition sources. The Company expects to begin dosing patients in a pivotal trial in the first quarter of 2025.

TARA-002 in LMs

- Dosing continues to progress in the ongoing STARBORN-1 trial. The Company has completed enrollment of the first safety cohort and will soon begin enrolling patients in the second safety cohort. STARBORN-1 is a Phase 2 clinical trial of TARA-002 in pediatric patients with macrocystic and mixedcystic 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 complete response (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.

- In addition to LMs, the Company believes that TARA-002 has the potential to be used to treat other maxillofacial cysts. While completing the STARBORN-1 trial in LMs remains a priority, there may be potential in the future to explore the use of TARA-002 to treat different types of maxillofacial cysts based on the historical literature from the TARA-002 predecessor, OK-432, as well as data in a pediatric patient with a maxillofacial cyst called a Ranula, which resolved nearly 100% after a single 1KE dose of TARA-002.

Corporate Update

- In June 2024, the Company appointed Lisa Schlesinger as Vice President, Head of New Product Development and Market Access.

Second Quarter 2024 Financial Results

- As of June 30, 2024, cash, cash equivalents and investments in marketable debt securities totaled approximately $89.6 million, which includes approximately $42.0 million in net proceeds from the April 2024 private placement. 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 second quarter of 2024 decreased to $6.4 million from $7.2 million for the prior year period. This decrease was primarily due to a decrease in non-clinical and clinical trial expenses related to TARA-002.

- General and administrative expenses for the second quarter of 2024 decreased to $4.3 million from $4.9 million for the prior year period. This decrease was primarily due to a reduction in personnel-related expenses associated with stock-based compensation as well as a decrease in market development activities.

- For the second quarter of 2024, Protara incurred a net loss of $9.5 million, or $0.45 per share, compared with a net loss of $11.3 million, or $1.00 per share, for the same period in 2023. Net loss for the second quarter of 2024 included approximately $1.1 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 and also approved in Taiwan. 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 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	
	June 30, 2024	**December 31, 2023**
Assets		
Current assets:		
Cash and cash equivalents	$ 89,581	$ 39,586
Marketable debt securities	-	25,994
Prepaid expenses and other current assets	2,633	3,125
Total current assets	92,214	68,705
Restricted cash, non-current	745	745
Property and equipment, net	1,185	1,296
Operating lease right-of-use asset	4,768	5,264
Other assets	2,735	2,944
Total assets	$ 101,647	$ 78,954
Liabilities and Stockholders’ Equity		
Current liabilities:		
Accounts payable	$ 1,385	$ 2,434
Accrued expenses and other current liabilities	3,228	2,732
Operating lease liability	1,035	983
Total current liabilities	5,648	6,149
Operating lease liability, non-current	3,949	4,484

Total liabilities	9,597	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 June 30, 2024 and December 31, 2023, 7,991 shares issued and outstanding as of June 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 June 30, 2024 and December 31, 2023, respectively.	21	11
Additional paid-in capital	313,021	268,725
Accumulated deficit	(220,992)	(200,384)
Accumulated other comprehensive income (loss)	-	(31)
Total stockholders' equity	92,050	68,321
Total liabilities and stockholders' equity	$ 101,647	$ 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 June 30,		For the Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
Operating expenses:				
Research and development	$ 6,387	$ 7,247	$ 14,135	$ 12,390
General and administrative	4,274	4,893	8,377	9,482
Total operating expenses	10,661	12,140	22,512	21,872
Loss from operations	(10,661)	(12,140)	(22,512)	(21,872)
Other income (expense), net:				
Interest and investment income	1,148	846	1,904	1,533
Other income (expense), net	1,148	846	1,904	1,533
Net income (loss)	$ (9,513)	$ (11,294)	$ (20,608)	$ (20,339)
Net income (loss) per share attributable to common stockholders, basic and diluted	$ (0.45)	$ (1.00)	$ (1.26)	$ (1.80)
Weighted-average shares outstanding, basic and diluted	21,233,163	11,307,842	16,327,056	11,305,867
Other comprehensive income (loss):				
Net unrealized gain (loss) on marketable debt securities	1	133	31	352
Other comprehensive income (loss)	1	133	31	352
Comprehensive loss	$ (9,512)	$ (11,161)	$ (20,577)	$ (19,987)

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