Protara Therapeutics Announces Second Quarter 2023 Financial Results and Business Update

August 3, 2023

- *Dosing is progressing on schedule in ADVANCED-1EXP trial evaluating TARA-002 in NMIBC patients with CIS; preliminary results expected in 1H24*
- *Company plans to commence dosing in ADVANCED-2 trial evaluating TARA-002 in NMIBC patients with BCG-unresponsive CIS and BCG-naïve CIS in 4Q23*
- *Company plans to initiate Phase 2 trial of TARA-002 in Lymphatic Malformations in 4Q23*
- *Cash, cash equivalents and investments of $80M as of June 30, 2023 expected to fund operations into 2025*

NEW YORK, Aug. 03, 2023 (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 second quarter ended June 30, 2023 and provided a business update.

“Following the positive preliminary data from the dose escalation portion of the ADVANCED-1 trial of TARA-002 in patients with high-grade non-muscle invasive bladder cancer (NMIBC), we have seen rapid enrollment in the expansion portion of the trial and anticipate sharing preliminary results in the first half of 2024,” said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. “We are building on this momentum, and plan to initiate two additional trials for TARA-002 later this year while continuing to maintain a disciplined approach to investments. We are in a solid financial position with cash runway into 2025.”

Recent Highlights

TARA-002 in NMIBC

- In April 2023 at the American Urological Association Annual Meeting, the Company announced positive preliminary results from the Phase 1a dose-escalation component of the ADVANCED-1 clinical trial of TARA-002, its investigational cell-based therapy, for the treatment of patients with high-grade NMIBC. The clinical data indicate that TARA-002 was generally well tolerated and anti-tumor activity was observed, including tumor regression in all three evaluable patients with carcinoma in situ (CIS), including one heavily pre-treated Bacillus Calmette-Guérin (BCG)-unresponsive patient who achieved a complete response (CR).

- In May 2023, the Company dosed the first patients in its Phase 1b ADVANCED-1EXP study, an open-label expansion trial evaluating intravesical TARA-002 at the 40KE[1] dose in 12 CIS patients, including BCG-naïve, BCG-unresponsive, and BCG-inadequately treated patients. Dosing continues to progress in the trial, with preliminary results expected in the first half of 2024. The primary endpoint of the trial is the CR rate at three months.

- In the fourth quarter of 2023, the Company plans to initiate ADVANCED-2, a Phase 1b/2 open-label trial evaluating intravesical TARA-002 in up to 102 patients with high-grade CIS, including 27 patients with CIS (± Ta/T1), BCG-Naïve, or BCG-experienced, who have not received intravesical BCG for at least 24 months prior to CIS diagnosis, and 75 patients with BCG-unresponsive CIS (± Ta/T1).

TARA-002 in Lymphatic Malformations (LMs)

- In April 2023, the Company received regulatory clearance from the U.S. Food and Drug Administration (FDA) to commence STARBORN-1, a Phase 2 clinical trial of TARA-002 in pediatric patients with macrocystic and mixed-cystic LMs. Trial start-up activities are well underway at the ten pediatric centers of excellence participating in the trial, and initiation is expected in the fourth quarter of 2023.

IV Choline Chloride Program

- Protara is concluding its prospective prevalence study to enhance understanding of the incidence of choline deficiency in patients dependent on parenteral nutrition. The Company continues to engage with the FDA and plans to use both regulatory feedback and results from the prospective study to inform next steps for the IV Choline Chloride development program.

Second Quarter 2023 Financial Results

- As of June 30, 2023, cash, cash equivalents and restricted cash were $80.4 million. The Company expects its current cash

and cash equivalents will be sufficient to fund its planned operations into 2025.

- Research and development expenses for the second quarter of 2023 increased to $7.2 million from $3.1 million during the second quarter of 2022, primarily reflecting an increase in expenses related to clinical and non-clinical trial activities for TARA-002.
- General and administrative expenses for the second quarter of 2023 decreased to $4.9 million from $5.6 million for the prior year period, primarily due to lower employee related expenses.
- For the second quarter of 2023, Protara reported a net loss of $11.3 million, or $1.00 per share, compared with a net loss of $8.5 million, or $0.76 per share, for the same period in 2022. Net loss for the second quarter of 2023 included approximately $1.6 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 and approved in Taiwan 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 strong immune cascade. Neutrophils, monocytes, and lymphocytes infiltrate the abnormal cells and various cytokines, including interleukins IL-2, IL-6, IL-8, IL-10, IL-12, interferon (IFN)-gamma, and tumor necrosis factor (TNF)-alpha are secreted by immune cells to induce a strong inflammatory reaction and destroy the abnormal cells.

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 3 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 (IV) phospholipid substrate replacement therapy initially in development for patients receiving parenteral nutrition (PN). Choline is a known important substrate for phospholipids that are critical for healthy liver function. Because PN patients cannot sufficiently absorb adequate levels of choline and no available PN formulations contain sufficient amounts of choline to correct this deficiency, PN patients often experience a prolonged progression to hepatic failure and death, with the only known intervention being a dual small bowel/liver transplant. IV Choline Chloride has been granted Orphan Drug Designation by the FDA for the prevention of choline deficiency in PN patients.

About Protara Therapeutics, Inc.

Protara is committed to advancing transformative therapies for people with cancer and rare diseases. Protara's portfolio includes its lead program, TARA-002, an investigational cell-based therapy being developed for the treatment of non-muscle invasive bladder cancer and lymphatic malformations, and IV Choline Chloride, an investigational phospholipid substrate replacement for patients dependent on parenteral nutrition. 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; the loss of key members of management; 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
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)

	As of	
	June 30, 2023	**December 31, 2022**
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 33,978	$ 24,127
Marketable debt securities	46,382	60,243
Prepaid expenses and other current assets	3,905	1,776
Total current assets	84,265	86,146
Restricted cash, non-current	745	745
Marketable debt securities, non-current	-	17,886
Property and equipment, net	1,443	1,592
Operating lease right-of-use asset	5,807	6,277
Other assets	2,881	644
Total assets	$ 95,141	$ 113,290
Liabilities and Stockholders’ Equity		
Current liabilities:		
Accounts payable	$ 1,852	$ 1,586
Accrued expenses	2,131	3,237
Operating lease liability	949	917
Total current liabilities	4,932	5,740
Operating lease liability, non-current	4,984	5,467
Total liabilities	9,916	11,207
Commitments and contingencies (Note 8)		
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, 2023 and December 31, 2022, 8,027 shares issued and outstanding as of June 30, 2023 and December 31, 2022.	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares: Common stock, 11,307,962 and 11,267,389 shares issued and outstanding as of June 30, 2023 and December 31, 2022, respectively.	11	11
Additional paid-in capital	265,853	262,724
Accumulated deficit	(180,303)	(159,964)
Accumulated other comprehensive income (loss)	(336)	(688)
Total stockholders’ equity	85,225	102,083
Total liabilities and stockholders’ equity	$ 95,141	$ 113,290

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,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				

Research and development	$ 7,247	$ 3,084	$ 12,390	$ 8,353
General and administrative	4,893	5,621	9,482	11,226
Total operating expenses	12,140	8,705	21,872	19,579
Loss from operations	(12,140)	(8,705)	(21,872)	(19,579)
Other income (expense), net:				
Interest and investment income	846	166	1,533	285
Other income (expense), net	846	166	1,533	285
Net loss	$ (11,294)	$ (8,539)	$ (20,339)	$ (19,294)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.00)	$ (0.76)	$ (1.80)	$ (1.71)
Weighted-average shares outstanding, basic and diluted	11,307,842	11,255,215	11,305,867	11,252,686
Other comprehensive income (loss):				
Net unrealized (loss) gain on marketable debt securities	133	(180)	352	(911)
Other comprehensive income (loss)	133	(180)	352	(911)
Comprehensive Loss	$ (11,161)	$ (8,719)	$ (19,987)	$ (20,205)

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