Protara Therapeutics Announces Fourth Quarter and Full Year 2021 Financial Results and Business Overview

March 9, 2022

- Phase 1 Study of TARA-002 in Patients with Non-Muscle Invasive Bladder Cancer Under Way -

- Strong Cash Position of $130.7M as of December 31, 2021 Expected to Fund Operations into Mid-2024 -

NEW YORK, March 09, 2022 (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 fourth quarter and year ended December 31, 2021 and provided a business update.

"Following a productive 2021, we are well poised to advance our pipeline in 2022, in particular, we are excited to have commenced our Phase 1 study of TARA-002 in non-muscle invasive bladder cancer (NMIBC), a significant step forward in our mission to bring a new immunotherapy to this patient population," said Jesse Shefferman, Chief Executive Officer of Protara Therapeutics. "Discussions with the U.S. Food and Drug Administration (FDA) remain ongoing on the design of a clinical trial of TARA-002 in patients with lymphatic malformations (LMs), a rare pediatric indication for which there are currently no U.S. FDA-approved therapies. In addition, we continue to assess potential future indications and combinations for TARA-002."

Mr. Shefferman added, "Supported by a strong balance sheet, which includes ample runway to support our planned operations into mid-2024, we remain steadfast in our commitment to bringing meaningful new therapeutic options to pressing areas of high unmet need."

Recent Highlights

TARA-002 in NMIBC

- In October 2021, the Company announced that the FDA cleared its Investigational New Drug (IND) application for TARA-002, an investigational cell-based therapy in development for the treatment of NMIBC. A Phase 1 clinical trial has commenced to assess the safety, tolerability, and preliminary signs of anti-tumor activity of TARA-002 in adults with high-grade NMIBC.

TARA-002 in LMs

- In October 2021, the Company updated its IND submission for TARA-002 for the treatment of LMs with completed confirmatory, current Good Manufacturing Practices (cGMP) comparability data. The Company is engaged with the FDA to align on a development plan for TARA-002 in LMs.

IV Choline Chloride in Intestinal Failure Associated Liver Disease (IFALD)

- The Company's prospective prevalence study to enhance understanding of the incidence of IFALD and choline deficiency in patients dependent on parenteral nutrition remains ongoing. The Company plans to use results from the prospective study, as well as its previously completed retrospective study, to inform next steps for the IV Choline Chloride development program.

Corporate Updates

- In January 2022, Protara announced the appointment of Jathin Bandari, M.D., as Chief Medical Officer. Dr. Bandari is a practicing urologic oncologist, recently serving at the University of Rochester where he specializes in both minimally invasive urologic oncology and advanced open pelvic retroperitoneal cancer surgery, and where he maintains a faculty appointment. Dr. Bandari joined Protara in April 2020 and most recently was Vice President, Head of Clinical Development, and Interim Chief Medical Officer.

Fourth Quarter and Full Year 2021 Financial Results

- As of December 31, 2021, cash, cash equivalents and marketable debt securities totaled $130.7 million. The Company expects its cash, cash equivalents, and marketable debt securities will be sufficient to fund its planned operations into mid-2024.

- Research and development expenses for the fourth quarter of 2021 increased to $4.1 million from $3.7 million for the prior year period, and for the full year increased to $21.1 million compared to $12.0 million for 2020. The fourth quarter and full year increases were primarily due to increases in non-clinical, clinical and regulatory expenses associated with TARA-002, headcount and stock-based compensation, and other employee-related expenses.

- General and administrative expenses for the fourth quarter of 2021 increased to $6.2 million from $5.3 million for the prior year period, and for the full year increased to $26.4 million compared to $22.5 million for 2020. The fourth quarter and full year increases were due to increases in headcount and employee-related expenses, market development capabilities, and costs associated with the new office in New York.

- For the fourth quarter of 2021, Protara reported a net loss of $10.2 million, or $0.91 per share, compared with a net loss of $8.8 million, or $0.79 per share, for the same period in 2020. Net loss for the year ended December 31, 2021 was $47.3 million, or $4.21 per share, compared with a net loss of $34.0 million, or $4.70 per share, for the year ended December 31, 2020. Net loss for the fourth quarter included approximately $2.0 million of stock-based compensation expenses. Net loss for the year ended December 31, 2021 included $10.4 million of stock-based compensation expenses.

About TARA-002

TARA-002 is an investigational cell therapy in development for the treatment of NMIBC and 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 Taiwan by Chugai Pharmaceutical Co., Ltd. Protara has successfully demonstrated 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-6, IL-8, IL-12, interferon (IFN)-gamma, tumor necrosis factor (TNF)-alpha, and vascular endothelial growth factor (VEGF) are secreted by immune cells to induce a strong local 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 and Intestinal Failure-associated Liver Disease (IFALD)

IV Choline Chloride is an investigational, intravenous (IV) phospholipid substrate replacement therapy initially in development for patients receiving parenteral nutrition (PN) who have IFALD. 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. If approved, IV Choline Chloride would be the first approved therapy for IFALD. It has been granted Orphan Drug Designations (ODDs) by the FDA for the treatment of IFALD and the prevention of choline deficiency in PN patients.

About Protara Therapeutics, Inc.

Protara is committed to identifying and advancing transformative therapies for people with cancer and rare diseases with limited treatment options. 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 therapy for the treatment of intestinal failure-associated liver disease. 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; statements related to expectations regarding interactions with the FDA, including potential alignment with the FDA on clinical trial design for TARA-002 in pediatric LM patients; Protara's financial footing; 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; the impact of the COVID-19 pandemic on Protara's business and the global economy as well as the impact on Protara's contract research organizations, study sites or other clinical partners; 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 or political 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.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)

	As of	
	December 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 35,724	$ 168,598
Restricted cash	-	50
Marketable debt securities	55,505	-
Prepaid expenses and other current assets	1,883	787
Total current assets	93,112	169,435
Restricted cash, non-current	745	745
Marketable debt securities, non-current	39,467	-
Property and equipment, net	1,719	1,240
Operating lease right-of-use asset	7,171	1,060
Goodwill	29,517	29,517
Other assets	865	1,160
Total assets	$ 172,596	$ 203,157
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 954	$ 914
Accrued expenses	2,489	1,913
Operating lease liability, current	855	88
Total current liabilities	4,298	2,915
Operating lease liability, non-current	6,384	999
Total liabilities	10,682	3,914
Commitments and Contingencies (Note 11)		
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, 2021 and 2020, respectively 8,027 shares issued and outstanding as of December 31, 2021 and 2020, respectively.	-	-
Common stock, $0.001 par value, authorized 100,000,000 shares:		
Common stock, 11,235,731 and 11,211,840 shares issued and outstanding as of December 31, 2021 and 2020, respectively.	11	11
Additional paid in capital	256,126	245,992
Accumulated deficit	(94,012)	(46,760)
Accumulated other comprehensive income (loss)	(211)	-
Total stockholders' equity	161,914	199,243
Total Liabilities and Stockholders' Equity	$ 172,596	$ 203,157

PROTARA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)

	For the Years Ended December 31,

	2021	2020
Operating expenses:		
Research and development	$ 21,088	$ 11,982
General and administrative	26,401	22,462
Total operating expenses	47,489	34,444
Loss from operations	(47,489)	(34,444)
Other income (expense), net		
Interest and investment income	237	500
Interest expense	-	(34)
Other income (expense), net	237	466
Net loss	(47,252)	(33,978)
Net loss per share attributable to common stockholders, basic and diluted	$ (4.21)	$ (4.70)
Weighted average shares outstanding, basic and diluted	11,232,576	7,233,913
Other comprehensive income (loss):		
Net unrealized (loss) gain on marketable debt securities	(211)	-
Other comprehensive income (loss)	(211)	-
Comprehensive Loss	$ (47,463)	$ (33,978)

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