Prevail Therapeutics Reports Second Quarter 2020 Financial Results and Business Highlights

August 11, 2020

Preliminary Data Demonstrates Normalization of CSF GBA1 Enzyme Activity in Parkinson's Disease with GBA1 Mutations and Neuronopathic Gaucher Disease Patients

Company Modifies Protocol for PROPEL Trial of PR001 for Parkinson's Disease Patients with GBA1 Mutations; Expects to Continue Enrollment in Second Half of 2020

PROVIDE Trial of PR001 for Type 2 Gaucher Disease and PROCLAIM Trial of PR006 for Frontotemporal Dementia Patients with GRN Mutations Expected to Initiate Enrollment in Second Half of 2020

$75.0 Million At-The-Market Equity Program

Conference call and live webcast today at 7:30 a.m. ET

NEW YORK, Aug. 11, 2020 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (Nasdaq: PRVL), a biotechnology company developing potentially disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases, today reviewed recent clinical and business updates and reported financial results for the second quarter ended June 30, 2020.

"We are making strong progress advancing our lead program, PR001, for both Parkinson's disease patients with *GBA1* mutations (PD-GBA) and neuronopathic Gaucher disease (nGD) patients — two devastating neurodegenerative disorders with no disease-modifying treatments available," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "The first patients dosed demonstrated normalization of CSF enzyme activity in response to PR001 administration, which is very encouraging and a critical first step in establishing PR001 as a potential new therapeutic approach for patients in need of effective treatment options."

Dr. Abeliovich added, "We are incredibly proud of the many achievements made by our team as we continue to advance our gene therapy programs into the clinic, including activation of the IND and clinical preparations for PR006 for the treatment of frontotemporal dementia patients with *GRN* mutations (FTD-GRN). Their ongoing dedication to Prevail's goal of developing groundbreaking gene therapies to help as many patients as quickly as possible is evident in our many accomplishments this year."

"There are no effective treatment options available for patients with neuronopathic Gaucher disease," said Ari Zimran, M.D., founder, Gaucher Clinic, Shaare Zedek Medical Center, Jerusalem. "The fact that PR001 was able to increase GCase enzyme activity to normal levels in these two patients is incredibly encouraging, and we look forward to future updates."

Recent Business Updates:

- **Clinical Administration of PR001 Yields Early Data from Two Patients:** To date, two patients have been enrolled in the Phase 1/2 PROPEL trial of PR001 for PD-GBA — one who received PR001 and another who received a sham procedure. Additionally, the company has received initial data from a Type 2 Gaucher disease patient treated with PR001 under a previously disclosed compassionate use request.

  In both treated patients, administration of PR001 resulted in normalization of glucocerebrosidase (GCase) enzyme activity levels measured in the cerebrospinal fluid (CSF) at 3 to 4 months after administration.

  - The Type 2 Gaucher disease patient demonstrated an increase in CSF GCase enzyme activity from an undetectable level at baseline to 1.0 µmol/L/d at month 1 and 4.7 µmol/L/d at month 4 following PR001 administration (adult normal range: 1.1 – 8.1 µmol/L/d).

  - The PD-GBA patient is also diagnosed with Gaucher disease and thus has *GBA1* mutations in both chromosomal copies. This patient demonstrated an increase in CSF GCase enzyme activity from an undetectable level at baseline to 3.0 µmol/L/d at month 3 following PR001 administration.

PR001 was observed to be well tolerated in the Type 2 Gaucher disease patient, and no adverse events related to PR001 treatment have been reported. The patient is clinically stable and no apparent worsening of the patient's neurological symptoms has been observed since PR001 administration. Follow-up clinical assessments are planned.

In the case of the PD-GBA patient, approximately three months following PR001 administration, the patient experienced severe adverse events (SAEs) that are presumed to have been caused by an immune-mediated response to the AAV9 viral vector. The patient received additional immunosuppressive treatment and the SAEs have markedly resolved.

Based on these initial efficacy and safety findings, Prevail has elected to modify the clinical protocol for the PROPEL trial in order to optimize the immunosuppression regimen, and has adapted the trial design to be open-label. The modifications have been endorsed by the independent data monitoring committee and discussed with and submitted to the FDA.

Taking into account the prior impact of COVID-19 on trial enrollment as well as this protocol amendment, the Company expects to continue enrollment in PROPEL in the second half of 2020, and to provide the next biomarker and safety analysis on a subset of patients enrolled in the PROPEL trial by mid-2021.

- **Planning Continues for Phase 1/2 Clinical Trial for nGD:** Study startup activities are continuing for the PROVIDE Phase 1/2 clinical trial of PR001 for Type 2 Gaucher disease patients, and the Company expects to initiate enrollment in the second half of 2020. The optimized immunosuppression regimen to be used in the amended PROPEL trial will also be implemented in the PROVIDE trial. In addition, the initiation of the PROGRESS Phase 1/2 clinical trial of PR001 for Type 3 Gaucher disease will be postponed until additional clinical data from the PROPEL and PROVIDE trials is available to inform the clinical development strategy for this indication.

- **Second Compassionate Use Patient Dosed:** The Company has granted a second compassionate use request for the administration of PR001 to a child with nGD, following approval by an international regulatory authority. The second patient was recently dosed, and the procedure was well tolerated.

- **PROCLAIM Trial of PR006 for FTD-GRN Scheduled to Initiate Enrollment in Second Half of 2020:** Study startup activities are also continuing for the PROCLAIM Phase 1/2 clinical trial of PR006 for FTD-GRN patients. The optimized immunosuppression regimen to be used in the amended PROPEL trial will also be implemented in the PROCLAIM trial.

- **Composition of Matter Patent Granted:** On June 23, the United States Patent and Trademark Office (USPTO) issued a composition of matter patent, U.S. Patent No. 10,689,625, with claims directed to the AAV vector used in PR006, Prevail's experimental gene therapy program for the treatment of FTD-GRN. The base patent term extends until October 2038, excluding patent term extensions or coverage in additional related patent filings.

- **Data Presented at Annual Alzheimer's Association International Conference (AAIC):** Prevail presented three poster presentations at the 2020 AAIC meeting in July. The data underscored the robust preclinical evidence in support of Prevail's AAV-based gene therapy approach, and highlighted the Company's strategy to validate these data in the planned PROCLAIM clinical trial evaluating PR006 for FTD-GRN.

- **Leadership Team Strengthened with Addition of General Counsel:** Kira Schwartz, J.D., joined Prevail on June 1 as the Company's General Counsel. In this new role, she leads all aspects of the Company's legal organization. Prior to joining Prevail, Ms. Schwartz served as Senior Vice President, Associate General Counsel and Assistant Secretary at Allergan plc (formerly Actavis plc), where she led a legal group supporting business development, corporate governance, finance, human resources, supply chain and real estate functions.

- **$75 Million At-The-Market Equity Program:** The Company has established an at-the-market equity program under which it may offer and sell up to $75.0 million of shares of its common stock.

Second Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and investments were $131.2 million as of June 30, 2020, as compared to $149.6 million and $168.1 million as of March 31, 2020 and December 31, 2019, respectively. The Company continues to anticipate that its cash runway will extend into the first half of 2022.

- **R&D Expenses:** R&D expenses were $12.9 million for the second quarter of 2020, compared to $12.0 million for the second quarter of 2019. The increase was primarily due to an increase of $2.6 million in direct clinical trial costs related to the PROPEL, PROVIDE, and PROCLAIM clinical trials and other trial startup costs and a $1.8 million increase in employee-related costs. These increases were partially offset by decreases of $2.6 million in direct manufacturing and process development cost due to the timing of production of clinical and preclinical supply and $1.7 million in license fees related to the options exercised under a license agreement with REGENXBIO during the three months ended June 30, 2019.

- **G&A Expenses:** G&A expenses were $9.2 million for the second quarter of 2020, compared to $3.7 million for the second quarter of 2019. The increase was primarily due to a $4.4 million increase in legal fees, primarily related to costs associated with the ongoing arbitration matter, intellectual property patent costs and costs to operate as a public company.

- **Net Loss:** Net loss was $22.1 million, or $0.66 loss per share, for the second quarter of 2020, compared to $15.1 million,

or $0.58 loss per share, for the second quarter of 2019.

Conference Call and Webcast Information

Prevail will host a conference call and webcast today at 7:30 a.m. ET to discuss its second quarter 2020 financial results and other clinical and business updates.

The webcast will be available under "Events and Presentations" in the Investors and Media section of the Company's website at ir.prevailtherapeutics.com. The conference call can be accessed by dialing 1 (866) 996-7201 (U.S. domestic) or +1 (270) 215-9495 (international) and referring to conference ID 7058186. A replay of the webcast will be archived on the Prevail Therapeutics website following the presentation.

About Prevail Therapeutics

Prevail is a clinical stage gene therapy company leveraging breakthroughs in human genetics with the goal of developing and commercializing disease-modifying AAV-based gene therapies for patients with neurodegenerative diseases. The company is developing PR001 for patients with Parkinson's disease with *GBA1* mutations (PD-GBA) and neuronopathic Gaucher disease; PR006 for patients with frontotemporal dementia with GRN mutations (FTD-GRN); and PR004 for patients with certain synucleinopathies.

Prevail was founded by Dr. Asa Abeliovich in 2017, through a collaborative effort with The Silverstein Foundation for Parkinson's with GBA and OrbiMed, and is headquartered in New York, NY.

Forward-Looking Statements Related to Prevail

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, as amended. Examples of these forward-looking statements include statements concerning: the potential impact of COVID-19 on Prevail's ongoing and planned clinical trials, business and operations; the potential of Prevail's gene therapies to modify the course of neurodegenerative diseases; the anticipated timing of Prevail's clinical trials of PR001 in PD-GBA and in nGD and Prevail's clinical trial of PR006; the expected timing of reporting of additional interim data for a subset of patients from the PROPEL trial; the modifications to the clinical trial protocols for PR001, PR004 and PR006 and the FDA's feedback thereon; and expectations regarding Prevail's cash runway. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These risks and uncertainties include, among others: Prevail's novel approach to gene therapy makes it difficult to predict the time, cost and potential success of product candidate development or regulatory approval; Prevail's gene therapy programs may not meet safety and efficacy levels needed to support ongoing clinical development or regulatory approval; the regulatory landscape for gene therapy is rigorous, complex, uncertain and subject to change; the fact that gene therapies are novel, complex and difficult to manufacture; and risks relating to the impact on our business of the COVID-19 pandemic or similar public health crises.

These and other risks are described more fully in Prevail's filings with the Securities and Exchange Commission (SEC), including the "Risk Factors" section of the Company's Quarterly Report on Form 10-Q for the period ended March 31, 2020, filed with the SEC on May 14, 2020 and its other documents subsequently filed with or furnished to the SEC, including its Quarterly Report on Form 10-Q for the period ended June 30, 2020. All forward-looking statements contained in this press release speak only as of the date on which they were made. Except to the extent required by law, Prevail undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

Prevail Therapeutics Inc.
Statements of Operations
(Unaudited)
(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Operating Expenses:				
Research and development	$ 12,943	$ 11,955	$ 24,360	$ 20,366
General and administrative	9,208	3,713	17,070	5,598
Total operating loss	(22,151)	(15,668)	(41,430)	(25,964)
Other income	—	—	210	—
Interest income, net	51	565	545	916
Total other income	51	565	754	916
Net loss	$ (22,100)	$ (15,103)	$ (40,675)	$ (25,048)
Other comprehensive loss	(1)	—	(1)	—
Comprehensive loss	$ (22,101)	$ (15,103)	$ (40,676)	$ (25,048)
Net loss per share, basic and diluted	$ (0.66)	$ (0.58)	$ (1.22)	$ (1.05)
Weighted average shares outstanding, basic and diluted	33,467,346	26,212,356	33,367,344	23,945,198

Prevail Therapeutics Inc.
Balance Sheets
(Unaudited)

(in thousands, except share and per share data)

	June 30, 2020	December 31, 2019
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 111,065	$ 168,051
Investments	6,458	—
Prepaid expenses and other current assets	4,620	6,410
Total current assets	122,143	174,461
Property and equipment, net	2,698	2,549
Investments	13,674	—
Operating lease right-of-use assets	9,355	10,001
Other long-term assets	2,730	—
Restricted cash	91	91
TOTAL ASSETS	$ 150,691	$ 187,102
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 1,981	$ 5,162
Accrued expenses and other current liabilities	9,435	5,330
Operating lease liabilities	1,447	1,341
Total current liabilities	12,863	11,833
Long-term operating lease liabilities	9,173	9,927
TOTAL LIABILITIES	22,036	21,760
COMMITMENTS AND CONTINGENCIES (Note 13)		
STOCKHOLDERS' EQUITY		
Preferred stock - $0.0001 par value, 10,000,000 shares authorized as of June 30, 2020 and December 31, 2019, respectively; no shares issued as of June 30, 2020 and December 31, 2019, respectively	—	—
Common stock - $0.0001 par value, 200,000,000 shares authorized as of June 30, 2020 and December 31, 2019, respectively, 34,214,851 and 34,138,750 shares issued and outstanding as of June 30, 2020 and December 31, 2019, respectively	3	3
Additional paid-in capital	253,430	249,441
Accumulated deficit	(124,777)	(84,102)
Accumulated other comprehensive loss	(1)	—
Total stockholders' equity	128,655	165,342
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 150,691	$ 187,102

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