



Prevail Therapeutics Reports First Quarter 2020 Financial Results and Business Highlights

May 14, 2020

Phase 1/2 Trial of PR001 for Parkinson's Disease with GBA1 Mutations Ongoing; Study Startup Activities Progressing for Phase 1/2 Trials of PR001 for Type 2 Neuronopathic Gaucher Disease and PR006 for Frontotemporal Dementia with GRN Mutations

Data Presentations Highlight Potential of AAV Gene Therapy Approach to Slow or Stop Neurodegenerative Disease Progression in Preclinical Models

NEW YORK, May 14, 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 business highlights and reported financial results for the first quarter ended March 31, 2020.

"We are excited to continue the clinical development of PR001 and are on track to report interim data for a subset of patients from our Phase 1/2 clinical trial of PR001 for Parkinson's disease with *GBA1* mutations (PD-GBA) later this year. In addition, we are advancing our AAV gene therapy-based pipeline, with the planned mid-year initiation of Phase 1/2 clinical trials of PR001 for Type 2 neuronopathic Gaucher disease (nGD) and PR006 for frontotemporal dementia with *GRN* mutations (FTD-GRN)," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "In addition, at ASGCT and AAT-AD/PD, we presented or will present data that validate the potential of these products for neurodegenerative disease patients with urgent unmet needs, and detailed our ongoing and planned clinical trials."

Recent Business Highlights and Updates:

- **Data and Clinical Trial Designs Presented or to be Presented for PR001 in nGD and PR006 in FTD-GRN at the 2020 American Society of Gene & Cell Therapy (ASGCT) Annual Meeting:** In separate presentations, Prevail described the design of two planned clinical trials: the PROVIDE Phase 1/2 clinical trial of PR001 for the treatment of patients with Type 2 nGD, and the PROCLAIM Phase 1/2 clinical trial of PR006 for the treatment of FTD-GRN. Preclinical data serving as the basis for the company's clinical programs, including PR001 for nGD and PD-GBA and PR006 for FTD-GRN, will also be presented.
- **Data and Clinical Trial Design Presented for PR001 in PD-GBA and PR006 in FTD-GRN at the AAT-AD/PD Focus Meeting 2020: Advances in Alzheimer's and Parkinson's Therapies:** Presentations at the AAT-AD/PD Focus Meeting included details on the design of Prevail's PROPEL Phase 1/2 clinical trial to evaluate the safety and efficacy of PR001 in patients with PD-GBA, and preclinical data related to PR001 for PD-GBA and PR006 for FTD-GRN.
- **Clinical Development of PR001:** As announced in March 2020, Prevail initiated dosing for the PROPEL Phase 1/2 trial of PR001 for patients with PD-GBA. As previously reported, in response to the COVID-19 pandemic, trial sites had temporarily suspended patient screening and enrollment activity. Prevail now expects patient screening to resume by the end of the second quarter of 2020 at certain clinical sites. The Company remains on track to report interim data on a subset of patients in the second half of 2020.

In addition, study startup activities are continuing for the PROVIDE Phase 1/2 clinical trial of PR001 for Type 2 nGD, and the Company intends to initiate dosing in mid-2020. Prevail also continues to expect to initiate the PROGRESS Phase 1/2 clinical trial of PR001 for Type 3 nGD in the second half of 2020. The timelines for PR001 are subject to any delays related to the COVID-19 pandemic.

Clinical Development of PR006: Study startup activities are also underway for the PROCLAIM Phase 1/2 clinical trial of PR006 for FTD-GRN patients, which is planned to initiate in mid-2020, subject to any delays related to the COVID-19 pandemic.

First Quarter 2020 Financial Results

- **Cash Position:** Cash and cash equivalents were \$149.6 million as of March 31, 2020, as compared to \$168.1 million as of December 31, 2019. As previously guided, the Company expects its current cash position to be sufficient to fund operating expenses into the first half of 2022.
- **R&D Expenses:** R&D expenses were \$11.4 million for the three months ended March 31, 2020, compared to \$8.4 million

for the three months ended March 31, 2019. The increase was primarily related to the Company's development programs, specifically as a result of increased clinical trial activities, and headcount.

- **G&A Expenses:** G&A expenses were \$7.9 million for the three months ended March 31, 2020, compared to \$1.9 million for the three months ended March 31, 2019. The increase was primarily due to an increase in professional services fees associated with the ongoing arbitration matter, personnel costs resulting from increased headcount, and other corporate and public company-related expenses.
- **Net Loss:** Net loss was \$18.6 million, or \$0.56 loss per share, for the three months ended March 31, 2020, compared to \$9.9 million, or \$1.73 loss per share, for the three months ended March 31, 2019.

About Prevail Therapeutics

Prevail is a 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, including resuming of delayed trials and initiation of new trials; the expected timing of reporting of interim data for a subset of patients from Prevail's Phase 1/2 clinical trial of PR001; 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 or about May 14, 2020, the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, filed with the SEC on March 26, 2020, and its other documents subsequently filed with or furnished to the SEC. 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 March 31,	
	2020	2019
Operating Expenses:		
Research and development	\$ 11,417	\$ 8,411
General and administrative	7,862	1,885
Total operating loss	(19,279)	(10,296)
Other income	210	—
Interest income	494	351
Total other income	704	351
Net loss	\$ (18,575)	\$ (9,945)
Net loss per share, basic and diluted	\$ (0.56)	\$ (1.73)
Weighted average shares outstanding, basic and diluted	33,267,342	5,740,874

Balance Sheets

(in thousands, except share and per share data)

	March 31, 2020	December 31, 2019
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(unaudited)

ASSETS

CURRENT ASSETS:

Cash and cash equivalents	149,580	168,051
Prepaid expenses and other current assets	4,909	6,410
Total current assets	154,489	174,461
Property and equipment, net	2,725	2,549
Operating lease right-of-use assets	9,682	10,001
Other long-term assets	1,817	—
Restricted cash	91	91
TOTAL ASSETS	\$ 168,804	\$ 187,102

LIABILITIES AND STOCKHOLDERS' EQUITY

CURRENT LIABILITIES:

Accounts payable	3,943	\$ 5,162
Accrued expenses and other current liabilities	5,452	5,330
Operating lease liabilities	1,395	1,341
Total current liabilities	10,790	11,833
Long-term operating lease liabilities	9,553	9,927
TOTAL LIABILITIES	20,343	21,760

COMMITMENTS AND CONTINGENCIES (Note 12)

STOCKHOLDERS' EQUITY

Preferred stock - \$0.0001 par value, 10,000,000 shares authorized as of March 31, 2020 and December 31, 2019, respectively; no shares issued as of March 31, 2020 and December 31, 2019, respectively	—	—
Common stock - \$0.0001 par value, 200,000,000 shares authorized as of March 31, 2020 and December 31, 2019, respectively, 34,196,456 and 34,138,750 shares issued and outstanding as of March 31, 2020 and December 31, 2019, respectively	3	3
Additional paid-in capital	251,135	249,441
Accumulated deficit	(102,677)	(84,102)
Total stockholders' equity	148,461	165,342
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	\$ 168,804	\$ 187,102

Media Contact:

Mary Carmichael
Ten Bridge Communications
mary@tenbridgecommunications.com
617-413-3543

Investor Contact:

investors@prevailtherapeutics.com



Source: Prevail Therapeutics