



Prevail Therapeutics Reports Full Year 2019 Financial Results and Business Highlights

March 26, 2020

Dosing Initiated in PROPEL Phase 1/2 Trial of PR001 for Treatment of Parkinson's Disease with GBA1 Mutations

Preparations Underway to Initiate PROVIDE Phase 1/2 Trial of PR001 for Treatment of Type 2 Neuronopathic Gaucher Disease in Mid-2020

IND for PR006 for Treatment of Patients with Frontotemporal Dementia with GRN Mutation Active; Initiation of PROCLAIM Phase 1/2 Trial Planned for Mid-2020

Cash Runway into First Half of 2022

NEW YORK, March 26, 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 reported financial results for the full year ended December 31, 2019, and reviewed recent business highlights.

"2019 was an important year for Prevail as we continued to make important clinical and regulatory progress across our portfolio of novel AAV9-based gene therapy candidates for neurodegenerative diseases and brought the company public," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "Dosing has begun in our PROPEL clinical trial of PR001 for patients with Parkinson's disease with GBA1 mutations, or PD-GBA. Additionally, we are working to initiate three more Phase 1/2 clinical trials this year: the PROVIDE and PROGRESS trials of PR001 for the treatment of Type 2 and Type 3 neuronopathic Gaucher disease, or nGD, respectively, and the PROCLAIM trial of PR006 for the treatment of frontotemporal dementia patients with GRN mutation, or FTD-GRN — potentially the first gene therapy for this condition to enter clinical trials. Our ability to advance multiple candidates towards the clinic over the last year reflects the dedication of our team, the potential reach of our platform, and the urgent need for novel potentially disease-modifying therapies for patients with these genetically-defined neurodegenerative diseases."

Recent Business Highlights and Updates:

- **PROPEL trial for PD-GBA underway:** Prevail has initiated dosing in its PROPEL Phase 1/2 trial of PR001 for patients with PD-GBA. In response to the COVID-19 pandemic, trial sites have temporarily suspended patient screening and enrollment activity. The Company still intends to report interim data on a subset of patients in the second half of 2020, however, this may be impacted if there is a prolonged suspension of enrollment. This randomized, double-blind trial is enrolling up to 16 moderate-to-severe PD-GBA patients to evaluate the safety and tolerability of two escalating doses of PR001, and will measure key biomarkers and exploratory efficacy endpoints. Currently, there are no treatments available to slow or stop disease progression for PD-GBA patients.
- **Clinical Development of PR001 for nGD Advancing:** Prevail announced in December 2019 that its Investigational New Drug (IND) application for PR001 for the treatment of nGD has been accepted by the U.S. Food and Drug Administration (FDA) and is now active. Study startup activities are ongoing for the PROVIDE Phase 1/2 clinical trial for Type 2 nGD. The Company intends to initiate dosing in the PROVIDE trial in mid-2020 and in the PROGRESS Phase 1/2 clinical trial for Type 3 nGD in the second half of 2020, in each case, subject to any delays related to the COVID-19 pandemic. There are no effective treatments available to address the neurological manifestations associated with either type of neuronopathic Gaucher disease.
- **PR001 Granted Orphan Drug and Rare Pediatric Disease Designations:** The FDA has granted Orphan Drug Designation for PR001 for the treatment of patients with Gaucher disease, and Rare Pediatric Disease Designation for the treatment of nGD, the most severe form of the condition. Programs with Orphan Drug status receive partial tax credit for clinical trial expenditures, waived user fees and eligibility for seven years of marketing exclusivity. Programs with Rare Pediatric Disease status may, upon marketing approval, qualify for a fully transferable priority review voucher applicable to a subsequent marketing application.
- **IND Active for PR006 for the Treatment of Patients with FTD-GRN:** Prevail announced in March 2020 that the FDA accepted the Company's IND application for PR006 for the treatment of FTD-GRN. FTD-GRN is a fatal, rapidly progressive, early-onset form of dementia for which there are no FDA approved treatments. The Company believes that PR006, which is being developed as a potential one-time gene therapy, has the ability to slow or stop progression of FTD-GRN. Prevail plans to initiate the PROCLAIM Phase 1/2 clinical trial for FTD-GRN patients in mid-2020, subject to any delays related to the COVID-19 pandemic.

- **PR006 Granted Orphan Drug and Fast Track Designations:** The FDA has granted Orphan Drug Designation for PR006 for the treatment of FTD and Fast Track Designation for FTD-GRN. Programs with Fast Track status receive accelerated approval or rolling review of a company's Biologics License Application (BLA). In addition, such a product candidate could be eligible for Priority Review if supported by clinical data at the time of BLA submission.
- **Preclinical Data Presented for PR001 in nGD and PR006 in FTD-GRN at WORLDSymposium™:** Prevail presented preclinical data at the 16th Annual WORLDSymposium in February 2020 demonstrating proof of concept in both nGD and FTD-GRN. Prevail also hosted a presentation and roundtable discussion during the WORLDSymposium featuring Professor Ari Zimran, M.D., a key opinion leader in the field of nGD. A recording of this event, including Dr. Zimran's presentation "Unmet Needs and Emerging Therapeutic Options for Neuronopathic Gaucher Disease," can be found under "Events and Presentations" in the Investors & Media section of the company's website, www.prevailtherapeutics.com.
- **Strengthened Leadership with Board Appointment:** Prevail appointed Morgan Sheng, Ph.D., to its Board of Directors. Dr. Sheng, who brings a wealth of expertise in neurodegenerative disease drug development, is a Core Institute Member and Co-Director of the Stanley Center for Psychiatric Research at the Broad Institute of MIT and Harvard, and a Professor in the Department of Brain and Cognitive Science at MIT. Previously, he was Vice President of Neuroscience at Genentech, where he was head of neuroscience research and drug discovery.

Full Year 2019 Financial Results

- **Cash Position:** Cash and cash equivalents were \$168.1 million as of December 31, 2019, as compared to \$63.0 million as of December 31, 2018. Following a strategic review and prioritization of programs, the Company expects its current cash position to be sufficient to fund operating expenses into the first half of 2022, versus mid-2021 as previously guided.
- **R&D Expenses:** R&D expenses were \$48.8 million for the year ended December 31, 2019, compared to \$14.1 million for the year ended December 31, 2018. The increase was primarily related to the Company's development programs, as a result of increased manufacturing-related spend, clinical and preclinical activities, and headcount.
- **G&A Expenses:** G&A expenses were \$17.0 million for the year ended December 31, 2019, compared to \$4.7 million for the year ended December 31, 2018. The increase was primarily due to an increase in personnel costs resulting from increased headcount, professional services fees, and other corporate-related expenses.
- **Net Loss:** Net loss was \$63.2 million, or \$2.22 loss per share, for the year ended December 31, 2019, compared to \$19.1 million, or \$3.71 loss per share, for the year December 31, 2018.

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 a GBA1 mutation (PD-GBA) and neuronopathic Gaucher disease; PR006 for patients with frontotemporal dementia with a GRN mutation (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 Phase 1/2 clinical trials of PR001 in PD-GBA and in nGD and Prevail's clinical trial of PR006; and the potential impacts of Orphan Drug and Rare Pediatric Disease Designations. 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 September 30, 2019, filed with the SEC on November 12, 2019, the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, to be filed with the SEC on or about 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.

(in thousands, except share and per share data)

	Year ended December 31, 2019	Year ended December 31, 2018
Operating Expenses:		
Research and development	\$ 48,798	\$ 14,127
General and administrative	17,005	4,682
Operating loss	(65,803)	(18,809)
Change in fair value of derivative liabilities	—	(781)
Other income	—	87
Interest income	2,615	887
Interest expense	—	(471)
Total other income (expense), net	2,615	(278)
Net loss	\$ (63,188)	\$ (19,087)
Net loss per share		
Basic and diluted	\$ (2.22)	\$ (3.71)
Weighted average shares outstanding:		
Basic and diluted	28,494,950	5,145,469

**Prevail Therapeutics Inc.
Balance Sheets**

(in thousands, except share and per share data)

	December 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 168,051	\$ 63,014
Prepaid expenses and other current assets	6,410	563
Total current assets	174,461	63,577
Property and equipment, net	2,549	678
Operating lease right-of-use assets	10,001	8,534
Restricted cash	91	91
Total assets	\$ 187,102	\$ 72,880
Liabilities, redeemable convertible preferred stock, and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	\$ 5,162	\$ 1,241
Accrued expenses and other current liabilities	5,330	1,477
Operating lease liabilities	1,341	917
Total current liabilities	11,833	3,635
Long-term operating lease liabilities	9,927	7,952
Total liabilities	21,760	11,587
Commitments and contingencies (see Note 15)		
redeemable convertible preferred stock		
Series Seed Preferred Stock - \$0.0001 par value, 0 and 6,480,000 shares authorized, issued and outstanding as of December 31, 2019 and 2018, respectively	—	3,524
Series A Preferred Stock - \$0.0001 par value, 0 and 9,072,000 shares authorized, 0 and 8,997,085 shares issued and outstanding as of December 31, 2019 and 2018, respectively	—	76,186
Stockholders' equity (deficit)		

Common stock - \$0.0001 par value, 200,000,000 and 28,398,600 shares authorized as of December 31, 2019 and 2018, respectively, 34,098,819 and 7,209,000 shares issued and outstanding as of December 31, 2019 and 2018, respectively	3	1
Additional paid-in capital	249,441	2,496
Accumulated deficit	(84,102)	(20,914)
Total stockholders' equity (deficit)	<u>165,342</u>	<u>(18,417)</u>
Total liabilities, redeemable convertible preferred stock, and stockholders' equity (deficit)	<u>187,102</u>	<u>\$ 72,880</u>

Media Contact:

Mary Carmichael
Ten Bridge Communications
mary@tenbridgecommunications.com

Investor Contact:

investors@prevailtherapeutics.com



Source: Prevail Therapeutics