



## Prevail Therapeutics Reports Third Quarter 2019 Financial Results and Recent Business Highlights

November 12, 2019

*Opened Enrollment of PROPEL Phase 1/2 Trial of PR001 to Treat Parkinson's Disease Patients with GBA1 Mutations*

*PR001 for Neuronopathic Gaucher Disease and PR006 for Frontotemporal Dementia with GRN Mutations Progressing Towards Clinic*

*Ongoing Collaboration with Lonza to Support the Manufacturing of our Pipeline of Novel AAV9-Based Gene Therapy Programs*

NEW YORK, Nov. 12, 2019 (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 recent business highlights and third quarter 2019 financial results.

"Prevail made important progress throughout the third quarter in the advancement of our gene therapy programs for the treatment of neurodegenerative diseases for patients with urgent unmet medical needs. We activated the first sites for our PROPEL Phase 1/2 clinical trial of PR001 for patients with Parkinson's disease with *GBA1* mutations, and we expect to begin dosing patients in the fourth quarter. This is an exciting step forward for patients suffering from this devastating disease," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "In addition, we are working diligently to finalize the IND submissions for PR001 for patients with neuronopathic Gaucher disease and PR006 for frontotemporal dementia patients with GRN mutations. We expect to initiate those Phase 1/2 trials in the first half of next year. Finally, our collaboration with Lonza is advancing our process development and manufacturing capabilities as we prepare to supply our late-stage clinical trials."

### Recent Business Highlights

- **Actively Recruiting for PROPEL Phase 1/2 Trial of PR001 for PD-GBA:** Prevail previously [announced](#) that the U.S. Food and Drug Administration (FDA) accepted the company's Investigational New Drug (IND) application for its lead program, PR001 for the treatment of Parkinson's disease patients with *GBA1* mutations (PD-GBA). The PROPEL trial is now open for enrollment. The trial will enroll up to 16 patients to investigate the safety and tolerability of PR001 and will also measure key biomarkers and exploratory efficacy endpoints. The company expects to initiate patient dosing this year.
- **Planning to Initiate the Phase 1/2 Trial of PR001 for Neuronopathic Gaucher Disease Patients in 1H 2020:** Prevail is also developing PR001 for the potential treatment of neuronopathic Gaucher disease (nGD). In September, the company announced that, based on feedback from the FDA and findings from its preclinical studies, it is modifying the design of the Phase 1/2 clinical trial in nGD to commence at a dose higher than originally proposed. Based on this feedback, the IND was placed on clinical hold pending FDA review of an amendment to the nGD IND, which will detail this modification. To support the higher dose, Prevail recently completed a non-human primate (NHP) safety study in which no PR001-related safety events or adverse findings were observed. Prevail expects to initiate a Phase 1/2 clinical trial for patients with nGD during the first half of 2020.
- **Phase 1/2 Trial of PR006 for Frontotemporal Dementia Patients with GRN Mutations (FTD-GRN) Expected to Initiate in 1H 2020:** Prevail has completed the necessary preclinical efficacy and safety studies and is targeting submission of an IND for PR006 at the end of the year. In a mouse model of FTD-GRN, PR006 increased progranulin expression, reduced markers of neuroinflammation, and reduced measures of lysosomal pathology and no PR006-related safety events or adverse findings were observed. In a GLP NHP safety study, PR006 treatment increased progranulin levels in the brain in a dose-dependent manner. An extremely minor degree of nerve fiber degeneration in spinal cord and glial cellularity in dorsal root sensory ganglia was observed and was not considered adverse. The company expects to initiate its Phase 1/2 clinical trial for FTD-GRN during the first half of 2020. Prevail believes PR006 has the potential to be a first-in-class, disease-modifying treatment for patients with FTD-GRN.
- **Established Collaboration with Lonza to Enable Large Scale Gene Therapy Production:** In October 2019, Prevail and Lonza [announced](#) the establishment of a collaboration focused on GMP manufacturing utilizing the baculovirus/Sf9 expression system for Prevail's pipeline of novel AAV-based gene therapy programs for patients with neurodegenerative diseases. Under the terms of the agreement, Lonza will manufacture PR001 and PR006 at its gene therapy center of excellence in Houston, Texas. The collaboration also has the potential to extend to Prevail's future pipeline of AAV-based gene therapy programs.

- **Continued Expansion of Internal Expertise and Capabilities:** Prevail continued to build out its team of over 50 employees and moved into a new office space in the Alexandria Center for Life Science in New York City. The new location includes approximately 12,000 square feet of lab space to support early-stage research and in-house process and analytical development utilizing both HEK293 and baculovirus/Sf9 AAV expression systems.

### Third Quarter 2019 Financial Results

- **Cash Position:** Cash and cash equivalents were \$183.1 million as of September 30, 2019, as compared to \$202.1 million as of June 30, 2019.
- **R&D Expenses:** R&D expenses were \$16.8 million for the third quarter of 2019, compared to \$4.6 million for the third quarter of 2018. The increase was primarily related to our development programs, as a result of increased manufacturing-related spend, clinical and preclinical activities, and headcount.
- **G&A Expenses:** G&A expenses were \$4.4 million for the third quarter of 2019, compared to \$0.9 million for the third quarter of 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 \$20.3 million, or \$0.62 loss per share, for the third quarter of 2019, compared to \$5.2 million, or \$0.99 loss per share, for the third quarter of 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 genetically defined sub-populations of patients with neurodegenerative diseases. 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 of Prevail's gene therapies to modify 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; Prevail's ability to work with Lonza to supply Prevail's late stage trials and commercial production; and the applicability of the collaboration to Prevail's future pipeline of AAV-based gene therapy programs. 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; and the fact that gene therapies are novel, complex and difficult to manufacture. 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 June 30, 2019, filed with the SEC on August 14, 2019, 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 September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
<b>Operating Expenses:</b>				
Research and development	\$ 16,836	\$ 4,599	\$ 37,202	\$ 9,110
General and administrative	4,452	920	10,050	2,520
Total operating loss	(21,288)	(5,519)	(47,252)	(11,630)
Change in fair value of derivative liabilities	—	—	—	(781)
Interest income	989	320	1,905	543
Interest expense	—	—	—	(471)
Total other income (expense), net	989	320	1,905	(709)
Net loss	\$ (20,299)	\$ (5,199)	\$ (45,347)	\$ (12,339)
Net loss per share, basic and diluted	\$ (0.62)	\$ (0.99)	\$ (1.68)	\$ (2.47)

Weighted average shares outstanding, basic and diluted

32,864,156      5,244,585      26,950,854      4,989,604

**Prevail Therapeutics Inc.**

**Balance Sheets**

(Unaudited)

(in thousands, except share and per share data)

	<u>September 30, 2019</u>	<u>December 31, 2018</u>
<b>ASSETS</b>		
CURRENT ASSETS:		
Cash and cash equivalents	\$ 183,074	\$ 63,014
Prepaid expenses and other current assets	7,425	563
Total current assets	<u>190,499</u>	<u>63,577</u>
Property and equipment, net	2,527	678
Operating lease right-of-use assets	10,312	8,534
Restricted cash	91	91
<b>TOTAL ASSETS</b>	<u>\$ 203,429</u>	<u>\$ 72,880</u>
<b>LIABILITIES, REDEEMABLE CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY (DEFICIT)</b>		
CURRENT LIABILITIES:		
Accounts payable	\$ 4,952	\$ 1,241
Accrued expenses and other current liabilities	5,089	1,477
Operating lease liabilities	1,134	917
Total current liabilities	<u>11,175</u>	<u>3,635</u>
Long-term operating lease liabilities	<u>10,226</u>	<u>7,952</u>
<b>TOTAL LIABILITIES</b>	<u>21,401</u>	<u>11,587</u>
COMMITMENTS AND CONTINGENCIES (Note 13) REDEEMABLE CONVERTIBLE PREFERRED STOCK		
Series Seed preferred stock - \$0.0001 par value, 0 and 6,480,000 shares authorized, issued and outstanding as of September 30, 2019 and December 31, 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 September 30, 2019 and December 31, 2018, respectively	—	76,186
STOCKHOLDERS' EQUITY (DEFICIT)		
Common stock - \$0.0001 par value, 200,000,000 and 28,398,600 shares authorized as of September 30, 2019 and December 31, 2018, respectively, 34,098,819 and 7,209,000 shares issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	3	1
Additional paid-in capital	248,286	2,496
Accumulated deficit	<u>(66,261)</u>	<u>(20,914)</u>
Total stockholders' equity (deficit)	<u>182,028</u>	<u>(18,417)</u>
<b>TOTAL LIABILITIES, REDEEMABLE CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY (DEFICIT)</b>	<u>\$ 203,429</u>	<u>\$ 72,880</u>

**Media Contact:**

Katie Engleman  
1AB

[katie@1ABmedia.com](mailto:katie@1ABmedia.com)

**Investor Contact:**

[investors@prevailtherapeutics.com](mailto:investors@prevailtherapeutics.com)



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