Prevail Therapeutics Reports Second Quarter 2019 Financial Results and Recent Business Highlights

August 14, 2019

IND Active for Phase 1/2 Trial of PR001 to Treat Parkinson's Disease Patients with GBA1 *Mutations*

Successful $125.0 Million Initial Public Offering Completed to Support Program Advancement and Expansion

NEW YORK, Aug. 14, 2019 (GLOBE NEWSWIRE) -- Prevail Therapeutics Inc. (NASDAQ: PRVL), a biotechnology company developing potentially disease-modifying AAV-based gene therapies for patients with neurodegenerative disorders, today reported financial results for the second quarter ended June 30, 2019 and provided an update on recent business highlights.

"Since Prevail's inception in 2017, we have made rapid progress toward our goal of bringing urgently needed therapies to patients with Parkinson's disease and other neurodegenerative disorders. With the first IND active for our lead gene therapy program, PR001, in Parkinson's disease with a *GBA1* mutation, we are one step closer to our goal, and remain focused on initiating a Phase 1/2 clinical trial of PR001 before the end of this year," said Asa Abeliovich, M.D., Ph.D., Founder and Chief Executive Officer of Prevail. "Our strong cash balance of $202 million following our $125 million IPO in June enables us to continue advancing PR001 and to initiate additional clinical trials, including for our second gene therapy candidate, PR006, for the treatment of frontotemporal dementia patients with a progranulin mutation."

Recent Business Highlights

- **IND Active for PR001**: In May 2019, Prevail's Investigational New Drug Application (IND) for its lead gene therapy candidate, PR001, for the treatment of Parkinson's disease patients with a *GBA1* mutation (PD-GBA) was accepted by the U.S. Food and Drug Administration (FDA) and is now active. Clinical site activation is ongoing, with patient dosing on track for the fourth quarter of 2019.

- **Fast Track Designation Received for PR001**: In July 2019, Prevail announced that the FDA granted Fast Track Designation for PR001 for the treatment of PD-GBA.

- **Preclinical Development of PR006 for Frontotemporal Dementia Patients with a *GRN* Mutation (FTD-GRN) advanced**: Based on pre-IND regulatory interaction with the FDA in June 2019, Prevail intends to initiate its Phase 1/2 clinical trial in FTD-GRN patients in 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.

- **Leadership Team Strengthened and Board of Directors Expanded to Support Growth:** In May 2019, Prevail announced the promotion of Yong Dai, Ph.D., to Chief Technology Officer. Dr. Dai will lead the Company's process and analytical development and establish advanced manufacturing. In addition, the Company appointed Tim Adams, Chief Financial Officer of ObsEva, to its Board of Directors and as Audit Committee Chair, and expanded the role of Board member Francois Nader, M.D. to Non-Executive Chairman.

- **Successful Completion of Initial Public Offering:** In July 2019, Prevail announced the closing of its initial public offering at a price to the public of $17.00 per share. The Company raised $125.0 million in aggregate gross proceeds to support the advancement of its three initial gene therapy programs and business operations.

Year to Date 2019 Financial Results

- **Cash Position:** Cash and cash equivalents were $202.1 million as of June 30, 2019, as compared to $63.0 million as of December 31, 2018.

- **R&D Expenses:** Research and development (R&D) expenses were $20.4 million for the six months ended June 30, 2019, compared to $4.5 million for the six months ended June 30, 2018. The increase was primarily due to a $11.2 million increase in external research and development expenses, a $3.2 million increase in personnel costs resulting from increased headcount, and a $1.5 million increase in REGENXBIO license fees.

- **G&A Expenses:** General and administrative (G&A) expenses were $5.6 million for the six months ended June 30, 2019, compared to $1.6 million for the six months ended June 30, 2018. The increase was primarily due to a $1.8 million increase in personnel costs resulting from increased headcount, a $1.7 million increase in consulting and professional service fees and other expenses, and a $0.5 million increase in facility rent expense.

- **Net Loss:** Net loss was $25.0 million, or $1.05 loss per share, for the six months ended June 30, 2019, compared to $7.1 million, or $0.42 loss per share, for the six months ended June 30, 2018.

Second Quarter 2019 Financial Results

- **Cash Position:** Cash and cash equivalents were $202.1 million as of June 30, 2019, as compared to $100.3 million as of March 31, 2019.

- **R&D Expenses:** R&D expenses were $12.0 million for the second quarter of 2019, compared to $3.2 million for the second quarter of 2018. The increase is primarily due to a $6.3 million increase in external research and development expenses, a $1.3 million increase in personnel costs resulting from increased headcount, and a $1.2 million increase in REGENXBIO license fee.

- **G&A Expenses:** G&A expenses were $3.7 million for the second quarter of 2019, compared to $1.0 million for the second quarter of 2018. The increase is primarily due to a $1.4 million increase in personnel costs resulting from increased headcount, a $0.9 million increase in consulting and professional services fees and other expenses, and a $0.4 million increase in facility rent expense.

- **Net Loss:** Net loss was $15.1 million, or $0.58 loss per share, for the second quarter of 2019, compared to $3.9 million, or $0.19 loss per share, for the second quarter of 2018.

About Prevail Therapeutics

Prevail Therapeutics 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. Prevail was founded by Dr. 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

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 ability of Prevail's balance sheet to enable continued development of its product candidates; the anticipated use of proceeds from Prevail's initial public offering; the likelihood of its interactions with the FDA to support Prevail's clinical development plans; and the timing of initiation of Prevail's Phase 1/2 clinical trials of PR001 and PR006. 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; PR001 or Prevail's other gene therapy programs may not meet safety and efficacy levels needed to support ongoing clinical development or regulatory approval; and the regulatory landscape for gene therapy is rigorous, complex, uncertain and subject to change. 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 final prospectus for its initial public offering, filed with the SEC on June 20, 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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Operating Expenses:				
Research and development	$ 11,955	$ 3,207	$ 20,366	$ 4,511
General and administrative	3,713	959	5,598	1,600
Total operating loss	(15,668)	(4,166)	(25,964)	(6,111)
Change in fair value of derivative liabilities	—	—	—	(781)
Interest income	565	222	916	222
Interest expense	—	—	—	(471)
Total other income (expense), net	565	222	916	(1,030)
Net loss	$ (15,103)	$ (3,944)	$ (25,048)	$ (7,141)
Net loss per share, basic and diluted	$ (0.58)	$ (0.19)	$ (1.05)	$ (0.42)
Weighted average shares outstanding, basic and diluted	26,212,356	20,326,716	23,945,198	16,970,543

Prevail Therapeutics Inc.
Balance Sheets
(Unaudited)
(in thousands, except share and per share data)

	June 30, 2019	December 31, 2018
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 202,095	$ 63,014
Prepaid expenses and other current assets	4,253	563
Total current assets	206,348	63,577
Property and equipment, net	2,123	678
Operating lease right-of-use assets	7,516	8,534
Restricted cash	91	91
TOTAL ASSETS	$ 216,078	$ 72,880
LIABILITIES, REDEEMABLE CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY (DEFICIT)		
CURRENT LIABILITIES:		
Accounts payable	$ 2,003	$ 1,241
Accrued expenses and other current liabilities	4,555	1,477
Operating lease liabilities	1,101	917
Total current liabilities	7,659	3,635
Long-term operating lease liabilities	7,380	7,952
TOTAL LIABILITIES	15,039	11,587
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 June 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 June 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 June 30, 2019 and December 31, 2018, respectively, 34,021,194 and 7,209,000 shares issued and outstanding as of June 30, 2019 and December 31, 2018	3	1
Additional paid-in capital	246,998	2,496
Accumulated deficit	(45,962)	(20,914)
Total stockholders' equity (deficit)	201,039	(18,417)
TOTAL LIABILITIES, REDEEMABLE CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY (DEFICIT)	$ 216,078	$ 72,880

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Source: Prevail Therapeutics