FORM 8-K
COURT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934
Date of Report (Date of earliest event reported):
August 11, 2020

Prevail Therapeutics Inc.

(Exact name of registrant as specified in its charter)

Delaware
(Exact name of registrant as specified in its charter)
001-38939
(Commission
File Number)
82-2129632
(I.R.S. Employer
Identification No.)

430 East 29th Street, Suite 1520
New York, New York
(Address of principal executive offices)

Registrant’s telephone number, including area code: (917) 336-9310

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:
☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:
Common Stock, par value $0.0001 per share
PRVL
The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).
Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐
On August 11, 2020, Prevail Therapeutics Inc. (the "Company") issued a press release announcing its financial results for the quarter ended June 30, 2020 and certain other business updates. A copy of the press release is furnished herewith as Exhibit 99.1 to this Current Report on Form 8-K and incorporated herein by reference.

The Company will hold a conference call at 7:30 a.m. ET on August 11, 2020 to discuss its financial results for the quarter ended June 30, 2020 and certain other business updates. A copy of the presentation to be discussed on the conference call is furnished as Exhibit 99.2 to this Current Report on Form 8-K and incorporated herein by reference.

The information furnished pursuant to Item 2.02 and Item 7.01 of this Current Report on Form 8-K, including Exhibits 99.1 and 99.2, shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any of the Company's filings under the Securities Act of 1933, as amended, or the Exchange Act, regardless of any general incorporation language in such filing, except as otherwise expressly stated in such filing.

(d) Exhibits.

<table>
<thead>
<tr>
<th>Exhibit No.</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>99.1</td>
<td>Press Release dated August 11, 2020</td>
</tr>
<tr>
<td>99.2</td>
<td>Presentation dated August 11, 2020</td>
</tr>
</tbody>
</table>
Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

PREVAIL THERAPEUTICS INC.

By: /s/ Brett Kaplan, M.D.

Brett Kaplan, M.D.
Chief Financial Officer

Dated: August 11, 2020
Prevail Therapeutics Reports Second Quarter 2020 Financial Results and Business Highlights

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, August 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 our 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 licenses entered into 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) 996-7203 (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.
<table>
<thead>
<tr>
<th></th>
<th>Three Months Ended June 30</th>
<th>Six Months Ended June 30</th>
<th>2020</th>
<th>2019</th>
</tr>
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<tr>
<td></td>
<td>2020</td>
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</tr>
<tr>
<td></td>
<td></td>
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<tr>
<td>Operating Expenses:</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Research and development</td>
<td>$12,943</td>
<td>$11,955</td>
<td>$24,360</td>
<td>$20,366</td>
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<tr>
<td>General and administrative</td>
<td>9,208</td>
<td>3,713</td>
<td>17,070</td>
<td>5,598</td>
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<tr>
<td>Total operating loss</td>
<td>(22,151)</td>
<td>(15,668)</td>
<td>(41,430)</td>
<td>(25,964)</td>
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<tr>
<td>Other income</td>
<td>—</td>
<td>210</td>
<td>—</td>
<td>—</td>
</tr>
<tr>
<td>Interest income, net</td>
<td>51</td>
<td>565</td>
<td>545</td>
<td>916</td>
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<tr>
<td>Total other income</td>
<td>51</td>
<td>565</td>
<td>754</td>
<td>916</td>
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<tr>
<td>Net loss</td>
<td>$ (22,100)</td>
<td>$ (15,103)</td>
<td>$ (40,675)</td>
<td>$ (25,048)</td>
</tr>
<tr>
<td>Other comprehensive loss</td>
<td>(1)</td>
<td>—</td>
<td>(1)</td>
<td>—</td>
</tr>
<tr>
<td>Comprehensive loss</td>
<td>$ (22,101)</td>
<td>$ (15,103)</td>
<td>$ (40,676)</td>
<td>$ (25,048)</td>
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<td>Net loss per share, basic and diluted</td>
<td>$ (1.66)</td>
<td>$ (0.58)</td>
<td>$ (1.22)</td>
<td>$ (1.05)</td>
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<td>Weighted average shares outstanding, basic and diluted</td>
<td>33,807,346</td>
<td>28,012,306</td>
<td>33,807,344</td>
<td>23,946,198</td>
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Preval Therapeutics Inc.
Statements of Operations
(Unaudited)

(in thousands, except share and per share data)
## Assets

<table>
<thead>
<tr>
<th>Current Assets</th>
<th>June 30, 2020</th>
<th>December 31, 2019</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash and cash equivalents</td>
<td>$111,065</td>
<td>$168,051</td>
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<tr>
<td>Investments</td>
<td>$6,458</td>
<td>$2,494</td>
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<tr>
<td>Prepaid expenses and other current assets</td>
<td>$4,620</td>
<td>$2,549</td>
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<tr>
<td>Total current assets</td>
<td>$122,143</td>
<td>$174,461</td>
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<tr>
<td>Property and equipment, net</td>
<td>$2,698</td>
<td>$2,549</td>
</tr>
<tr>
<td>Investments</td>
<td>$13,674</td>
<td>$10,081</td>
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<tr>
<td>Operating lease right-of-use assets</td>
<td>$9,355</td>
<td>$10,081</td>
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<tr>
<td>Other long-term assets</td>
<td>$2,730</td>
<td>$10,081</td>
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<tr>
<td>Restricted cash</td>
<td>$91</td>
<td>$91</td>
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<tr>
<td><strong>Total Assets</strong></td>
<td><strong>$150,691</strong></td>
<td><strong>$187,102</strong></td>
</tr>
</tbody>
</table>

## Liabilities and Stockholders' Equity

### Current Liabilities

<table>
<thead>
<tr>
<th>Current Liabilities</th>
<th>June 30, 2020</th>
<th>December 31, 2019</th>
</tr>
</thead>
<tbody>
<tr>
<td>Accounts payable</td>
<td>$1,981</td>
<td>$5,162</td>
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<tr>
<td>Accrued expenses and other current liabilities</td>
<td>$9,435</td>
<td>$5,330</td>
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<tr>
<td>Operating lease liabilities</td>
<td>$1,447</td>
<td>$3,341</td>
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<tr>
<td><strong>Total current liabilities</strong></td>
<td><strong>$12,863</strong></td>
<td><strong>$13,833</strong></td>
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<tr>
<td>Long-term operating lease liabilities</td>
<td>$9,173</td>
<td>$9,927</td>
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<tr>
<td><strong>Total Liabilities</strong></td>
<td><strong>$22,036</strong></td>
<td><strong>$23,760</strong></td>
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</tbody>
</table>

### stockholders' Equity

<table>
<thead>
<tr>
<th>stockholders' Equity</th>
<th>June 30, 2020</th>
<th>December 31, 2019</th>
</tr>
</thead>
<tbody>
<tr>
<td>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</td>
<td>—</td>
<td>—</td>
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<td>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</td>
<td>$3,304,464</td>
<td>$3,304,464</td>
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<tr>
<td>Additional paid-in capital</td>
<td>$253,430</td>
<td>$249,441</td>
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<td>Accumulated deficit</td>
<td>$(124,777)</td>
<td>$(84,102)</td>
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<tr>
<td>Accumulated other comprehensive loss</td>
<td>$(1)</td>
<td>$(1)</td>
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<tr>
<td><strong>Total stockholders' equity</strong></td>
<td><strong>$128,655</strong></td>
<td><strong>$165,342</strong></td>
</tr>
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</table>

**Total Liabilities and stockholders' equity**

<table>
<thead>
<tr>
<th>Total Liabilities and stockholders' equity</th>
<th>June 30, 2020</th>
<th>December 31, 2019</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Total Liabilities and stockholders' equity</strong></td>
<td><strong>$150,691</strong></td>
<td><strong>$187,102</strong></td>
</tr>
</tbody>
</table>
Certain information contained in this presentation and statements made orally during this presentation relate to or is based on studies, publications, surveys and other data obtained from third-party sources and our own internal estimates and research. While we believe these third-party sources to be reliable as of the date of this presentation, we have not independently verified, and make no representation as to the adequacy, fairness, accuracy or completeness of, any information obtained from third-party sources. While we believe our internal research is reliable, such research has not been verified by any independent source.

This presentation contains forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our clinical results and other future conditions. All statements other than statements of historical facts contained in this presentation, including statements regarding future results of operations and financial position, business strategy, current and prospective product candidates, planned clinical trials and preclinical activities, product approvals, degree of market acceptance of approved products, research and development costs, current and prospective collaborations, timing and likelihood of success, plans and objectives of management for future operations, and future results of anticipated product candidates, are forward-looking statements. The words "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "estimate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this presentation represent our views as of the date of this presentation. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements. The forward-looking statements contained herein are subject to significant risks and uncertainties, including those described in the "Risk Factors" section of our Quarterly Report on Form 10-Q for the quarter ended March 31, 2020, filed with the Securities and Exchange Commission ("SEC") on May 14, 2020, and our other documents subsequently filed with or furnished to the SEC, including our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020. New risk factors and uncertainties may emerge from time to time, and it is not possible to predict all risk factors and uncertainties. There can be no assurance that the opportunity will meet your investment objectives, that you will receive a return of all or part of such investment. Investment results may vary significantly over any given time period. The appropriateness of a particular investment or strategy will depend on an investor's individual circumstances and objectives. We recommend that investors independently evaluate specific investments and strategies.
Call agenda

- Introduction
  - Brett Kaplan, M.D., Chief Financial Officer

- Overview
  - Asa Abelowich, M.D., Ph.D., CEO & Founder

- Clinical Update
  - Jeffrey Sevigny, M.D., Chief Medical Officer

- Financial Update
  - Brett Kaplan, M.D., Chief Financial Officer

- Q&A
Overview

Asa Abeliovich, M.D., Ph.D., CEO & Founder
Focused on our mission

- Potential disease-modifying targets identified based on human genetics
- Targeting genetically defined patient populations
- Gene delivery with AAV9 vector has a track record of efficacy and safety

- Phase 1/2 PROPEL trial for Parkinson's with GBA1 mutations (PD-GBA) underway; PD-GBA affects >90K Americans
- Potential for rapid proof-of-concept for PR001 in neuropathic Gaucher disease; PROVIDE trial planned to initiate enrollment in 2H 2020

- PR006 IND active for frontotemporal dementia with GRN mutations (FTD-GRN)
- Phase 1/2 PROCLAIM trial on track to initiate enrollment in 2H 2020

- Expertise in developing therapies for neurodegenerative diseases
- Additional genetically-validated targets in Parkinson's, Alzheimer's, ALS, FTD
- Leaders in gene therapy manufacturing and process development
Rapid advancement to clinical stage

- **2017**
  - OCTOBER 2017: Lab space secured in NYC

- **2018**
  - MARCH 2018: Seed financing and REGENXBIO license

- **2019**
  - MARCH 2019: REGENXBIO second agreement
  - MAY 2019: PR001 PD-GBA IND active
  - DECEMBER 2019: PR001 nGD IND active

- **2020**
  - MARCH 2020: PR006 FTD-GRN IND active
  - JUNE 2019: $125M IPO
  - MAY 2018: $75M Series A
  - MARCH 2019: $50M Series B
Unique pipeline of potentially disease-modifying AAV9 gene therapies for neurodegenerative diseases

<table>
<thead>
<tr>
<th>Programs</th>
<th>Indication</th>
<th>Approach</th>
<th>Stage of Development</th>
</tr>
</thead>
<tbody>
<tr>
<td>PR001</td>
<td>PD-GBA</td>
<td>GBA1 Gene Transfer</td>
<td>IND Active</td>
</tr>
<tr>
<td></td>
<td>Neuronopathic Gaucher disease</td>
<td>GBA1 Gene Transfer</td>
<td>IND Active</td>
</tr>
<tr>
<td>PR006</td>
<td>FTD-GRN</td>
<td>GRN Gene Transfer</td>
<td>IND Active</td>
</tr>
<tr>
<td>PR004</td>
<td>Synucleinopathies</td>
<td>GBA1 Gene Transfer + α-Synuclein Knockdown</td>
<td>IND Active</td>
</tr>
</tbody>
</table>

FTD granted by FDA for PR001 for the treatment of PD-GBA
ODD & RPDD granted by FDA for PR001 for the treatment of GD & nGD, respectively
ODD & FTD granted by FDA for PR006 for the treatment of FTD & FTD-GRN, respectively
Prevail owns worldwide commercial rights to all product candidates in the pipeline

FTD: Fast Track Designation; ODD: Orphan Drug Designation; RPDD: Rare Pediatric Disease Designation
Second quarter business highlights

- Clinical development of PR001 advances
  - Two nGD patients dosed via compassionate use requests
  - Two PD-GBA patients enrolled in Phase 1/2 PROPEL trial
- Encouraging early data available from one nGD patient and one PD-GBA patient
  - In both patients CSF GCase levels were undetectable at baseline; normalized at ~3-4 months after PR001 administration
- No PR001-related AEs reported in the two nGD compassionate use patients
- One PD-GBA patient dosed with PR001 experienced SAEs that markedly resolved with additional immunosuppression
- Based on early clinical data PROPEL trial protocol amended and enrollment expected to continue in 2H 2020
- Study startup activities continuing for Phase 1/2 trials of PR001 for GD2 and PR006 for FTD-GRN
- Leadership team strengthened with new General Counsel
Clinical Update
Jeffrey Sevigny, M.D., Chief Medical Officer
PR001 program update

nGD
Compassionate Use

- Two nGD patients administered PR001 following compassionate use requests
  - ~22-month-old GD2 patient dosed in January 2020 (as previously disclosed)
  - Additional nGD patient recently dosed

propel

- Two patients enrolled in PROPEL Phase 1/2 trial for PD-GBA
  - One patient received 1.4x10^{14} vg of PR001; second patient received sham procedure
  - PROPEL enrollment expected to continue in 2H 2020

provide

- PROVIDE Phase 1/2 trial for Type 2 Gaucher disease anticipated to initiate enrollment in 2H 2020

progress

- Initiation of PROGRESS Phase 1/2 trial for Type 3 Gaucher disease postponed until additional clinical data from PROPEL and PROVIDE available
Type 2 Gaucher compassionate use: early patient data

Background
- Type 2 Gaucher disease patient dosed with PR001 (1.3x10^{14} vg) in Jan 2020 following compassionate use request
- Patient was ~22 months old at dosing

Biomarkers
- CSF GCase enzyme activity increased from undetectable at baseline to normal level at Month 4

<table>
<thead>
<tr>
<th>GCase activity in CSF (μmol/L/d)</th>
<th>Day 0</th>
<th>Month 1</th>
<th>Month 4</th>
<th>Normal range (adult)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Undetectable</td>
<td></td>
<td>1.0</td>
<td>4.7</td>
<td>1.1 - 8.1</td>
</tr>
</tbody>
</table>

Clinical Safety and Efficacy
- PR001 ICM administration well-tolerated; no AEs reported
- Patient clinically stable; no apparent worsening of the patient's neurological symptoms since PR001 administration
- Follow-up clinical assessments are planned
Two PD-GBA patients enrolled in PROPEL trial: one administered PR001; one received sham procedure.

Patient who received PR001 (1.4x10^{14} vg) also diagnosed with Gaucher disease (GBA1 mutations in both chromosomal copies).

CSF GCase enzyme activity increased from undetectable at baseline to normal level at ~Month 3.

<table>
<thead>
<tr>
<th>GCase activity in CSF (μmol/L/d)</th>
<th>Day 0</th>
<th>~Month 3</th>
<th>Normal range (adult)</th>
</tr>
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<tbody>
<tr>
<td></td>
<td>Undetectable</td>
<td>3.0</td>
<td>1.1 - 8.1</td>
</tr>
</tbody>
</table>

PR001 ICM administration well-tolerated.

Patient did not complete protocol-specified immunosuppression regimen due to steroid intolerance.

Three months following PR001 administration, patient experienced SAEs presumed to be AAV9-immune mediated:

- SAEs were worsening of the patient’s underlying hallucinations and orthostasis, and encephalitis (worsening of mental status and evidence of CSF inflammation).
- MRI (with contrast) of brain and spinal cord showed no changes from baseline.

Patient’s condition responded to additional immunosuppressive treatment; SAEs have markedly resolved.
PROPEL PD-GBA Phase 1/2 trial updates

- Amendment implemented to mitigate risk of AAV9-mediated immune reactions:
  - Immunosuppression regimen modified by adding sirolimus and reducing corticosteroid dose and duration
  - Trial converted to open-label design and sample size reduced to N=12 with removal of sham procedure
- Based on updated analytical methods with increased precision, dose levels of PR001 established to be:
  - $1.4 \times 10^{14}$ vg in low dose
  - $2.8 \times 10^{14}$ vg in high dose

- Protocol amendment endorsed by IDMC, discussed with and submitted to FDA
- Patient enrollment expected to continue in 2H 2020
- Next biomarker and safety analysis on subset of patients by mid-2021
Revised PR001 PD-GBA Phase 1/2 trial
Open label, ascending dose

- Single or biallelic GBA1 mutations
- Moderate to severe Parkinson’s disease
- Stable background PD medication

- Safety and tolerability
- Key biomarkers: GCase, GluCer, GluSph (CSF and blood)
- Additional biomarkers: α-Synuclein, NFL, DAT SPECT, MRI
- Efficacy: MDS-UPDRS, cognition, ADLs

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ICM: intra-cisterna magna; MDS-UPDRS: Movement Disorders Society Unified Parkinson’s disease Rating Scale; ADLs: Activities of Daily Living; NFL: neurofilament light; DAT: Dopamine transporter; SPECT: single photon emission computed tomography
Goal of PR001 treatment is to deliver the GBA1 gene to restore GCase activity in PD-GBA patients

Source: Liu et al., Annals of Neurology 2016; Thaler et al., Parkinsonism and Related Disorders 2017

Pre-PR001 Treatment

~50% of healthy GCase

PR001

Goal of PR001 Treatment

~75% of healthy GCase

Upside of PR001 Treatment

100% of healthy GCase

PD-GBA

GCase Enzyme Activity (Illustrative)
PR001 Type 2 Gaucher disease Phase 1/2 trial
Open label

- Single ICM injection
- 2 month biomarker readout
- 12 month clinical readout
- 5-year safety and clinical follow-up

**PR001 (Open label, N=15)**

**Provide Type 2 Gaucher Patients**
- Infants 0-24 months old
- Biallelic GBA1 mutations
- Neurological signs & symptoms consistent with Type 2 Gaucher disease
- Stable SoC background medications

- Safety and tolerability
- Key biomarkers: GCase, GluCer, GluSph (CSF and blood)
- Time to clinical event (e.g., tracheostomy, PEG placement, death)
- Efficacy: behavior, cognition, gross motor, function, QoL

ICM: intra-cisterna magna; QoL: quality of life; SoC: standard of care; PEG: percutaneous endoscopic gastrostomy
PR006 FTD-GRN Phase 1/2 trial
Open label, ascending dose

- 30-80 years old
- Single pathogenic GRN mutation
- Symptomatic disease stage
- Stable background medications

- Safety and tolerability
- Key biomarkers: progranulin, NFL, volumetric MRI
- Efficacy: CDR plus NACC FTLD; measures of behavior, cognition, language, function, QoL

ICM: intra-cisterna magna; NfL: neurofilament light chain; CDR plus NACC FTLD: Clinical Dementia Rating plus National Alzheimer's Coordinating Center Frontal Temporal Lobar Dementia; QoL: quality of life
Financial Update

Brett Kaplan, M.D., Chief Financial Officer
Second quarter 2020 financial update

- Cash (inclusive investment purchases) of $131.2 million as of June 30, 2020, as compared to $149.6 million as of March 31, 2020
- Cash runway remains into first half of 2022

- R&D Expenses
  - $12.9 million for second quarter of 2020, compared to $12.0 million for second quarter of 2019

- G&A Expenses
  - $9.2 million for second quarter of 2020, compared to $3.7 million for second quarter of 2019

- Other
  - Filed prospectus supplement for $75M ATM offering
Q&A