Provention Bio Reports Top Line Results from Phase 2a PRINCE Clinical Trial with PRV-6527, an Oral CSF-1R Inhibitor, in Patients with Moderate to Severe Crohn's Disease

-Study Did Not Achieve Primary Endpoint Due to High Placebo Effect-

-Excellent Safety Profile and Improvements in Clinically Relevant Secondary Endpoints (Endoscopy and Histology) Support Further Development by a Partner-

- Company to Host Conference Call at 8:30 AM Eastern Time -

OLDWICK, N.J., Oct. 22, 2019 /PRNewswire/ -- Provention Bio, Inc. (Nasdaq: PRVB), a clinical stage biopharmaceutical company dedicated to intercepting and preventing immune-mediated disease, today announced top-line results from its randomized, placebo-controlled Phase 2a PRINCE clinical trial evaluating twice-daily PRV-6527, an oral Colony Stimulating Factor-1 Receptor (CSF-1R) small molecule inhibitor.

The PRINCE study enrolled 93 moderate-to-severe Crohn's disease patients who were either naïve to biologic therapy (~70%) or who had previously failed at least one biologic drug (~30%). The primary efficacy endpoint of the study was the change in the Crohn's Disease Activity Index (CDAI) score at week 12. While PRV-6527 demonstrated a substantial improvement in this symptom driven score at week 12, it did not differentiate from placebo. This high placebo response is deemed to be related to the background medication used (~85%) in the study's predominantly biologic-naïve population.

PRV-6527 was associated with improvements in several key secondary objective endpoints in the steroid-free population (75% of study subjects), including mucosal endoscopy (as assessed by the Simple Endoscopic Score for Crohn's Disease, SES-CD) and tissue histology (as measured by the Global Histological Activity Score, GHAS). Analysis of exploratory serum and tissue biomarkers showed that patients treated with PRV-6527 had significant reductions in circulatory inflammatory monocytes, as well as macrophages, dendritic cells and the CSF1 gene signature in colonic tissue, providing proof of mechanism in the interception of inflammatory myeloid cells.

PRV-6527 was found to be generally safe and well tolerated, with no drug-related serious adverse events.

"Despite the PRINCE study's high placebo effect, single dose level, limited 12-week duration, and relatively small sample size, the initial top-line results highlight how PRV-6527's CSF-1R inhibition intercepts the migration of inflammatory cells to the gut. This rapid go/no-go, signal finding trial indicates a potential role for PRV-6527 as a well-tolerated, oral, first-line therapy in early Crohn's and, potentially, for the prevention of relapse," stated Ashleigh Palmer, CEO of Provention Bio. "Under the terms of our in- license agreement, Janssen now has 90 days to exercise its option to re-acquire this asset to support further development in inflammatory bowel disease. Meanwhile, we will continue to devote all of our efforts to advancing the rest of our portfolio, especially our lead Breakthrough Therapy program PRV-031 (teplizumab) for the prevention or delay of type 1 diabetes."

Pursuant to its licensing agreement with Provention, Janssen has the right to buy back PRV-6527 for a one-time payment of $50 million and single-digit royalties on future net sales. In the event that Janssen does not take back PRV-6527, Provention is free to sublicence the program on a worldwide basis to another partner in the field of inflammatory bowel disease.

Conference Call and Webcast Information
Provention Bio will discuss these results via conference call on Tuesday, October 22, 2019 at 8:30 am ET. A webcast presentation will also be available on the Investors page of the Company's website, www.proventionbio.com. To access the call, please dial 1-877-870-4263 (domestic) or 1-412-317-0790 (international) five minutes prior to the start time and ask to be connected to the "Provention Bio Call". A webcast replay of the call will be available beginning at 10:30 am ET on the day of the call.

About PRV-6527
PRV-6527 is a highly potent and selective small-molecule oral inhibitor of CSF-1R. It was initially developed by Janssen Pharmaceuticals and has been evaluated in over 200 subjects to date. Proof of mechanism was demonstrated based on inhibition of CSF-1R signaling and myeloid cell counts in blood. CSF-1 binds to its receptor (CSF-1R) on myeloid cells and drives the differentiation and maturation of these cells into inflammatory dendritic cells and macrophages, which then populate the gut and other tissues. In the gut, these differentiated myeloid cells present antigens from intestinal bacteria (the microbiome) to white blood cells and trigger inflammatory processes.

About Crohn's Disease
Crohn's disease is a chronic, immune-mediated inflammatory bowel disease (IBD) characterized by inflammation of the gastrointestinal (GI) tract. Myeloid cells, a species of antigen-presenting cells, are believed to play a central role in Crohn's disease by presenting microbiome antigens to white blood cells in the gut. CSF-1R drives myeloid cell differentiation in the bone marrow resulting in the maturation of inflammatory dendritic cells and macrophages, which then populate the gut and other tissues and trigger inflammatory processes.

About Provention Bio, Inc.
Provention Bio, Inc. (Nasdaq: PRVB) is a clinical-stage biopharmaceutical company leveraging a transformational drug development strategy that is focused on the prevention or interception of immune-mediated disease. Provention's mission is to in-license, transform and develop therapeutic candidates targeting the high morbidity, mortality and escalating costs of autoimmune and inflammatory diseases including: type 1 diabetes (T1D), Crohn's disease, celiac disease, and lupus. Provention's diversified portfolio includes advanced-stage product development candidates that have undergone clinical testing by other companies.

Forward Looking Statements
Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of forward-looking words such as "anticipate," "believe," "forecast," "estimate," "expect," and "intend," among others. These forward-looking statements are based on Provention's current expectations and actual results could differ materially. There are a number of factors that could cause actual events to differ materially from those indicated by such forward-looking statements. These factors include, but are not limited to, risks related to failure to obtain FDA approvals or clearances and noncompliance with FDA regulations; uncertainties of patent protection and litigation; limited research and development efforts and dependence upon third parties; substantial competition; our need for additional financing and the risks listed under "Risk factors" in our annual report on Form 10-K for the year ended December 31, 2018 and any subsequent filings with the Securities and Exchange Commission (SEC). As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. Provention does not undertake

an obligation to update or revise any forward-looking statement. The information set forth herein speaks only as of the date hereof.

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