



## Principia Updates PRN1008 Pemphigus Clinical Program

December 30, 2019

### Accelerated enrollment of company's Phase 3 pivotal trial anticipating final results in second half of 2021

SOUTH SAN FRANCISCO, Calif., Dec. 30, 2019 (GLOBE NEWSWIRE) -- Principia Biopharma Inc. (Nasdaq: PRNB), a late-stage biopharmaceutical company dedicated to bringing transformative oral therapies to patients with significant unmet medical needs in immune-mediated diseases, today announced updated anticipated timing for final results in its ongoing pivotal Phase 3 clinical trial of PRN1008 data in patients with pemphigus, as well as complete response rates from its Phase 2 Part B open-label trial.

"We are delighted to see the interest in our Phase 3 PEGASUS trial and through the hard work of our investigators, study coordinators and our own clinical team, we believe the accelerated enrollment timelines now project us to have final data in the second half of 2021 as opposed to the first half of 2022," said Dolca Thomas, MD, Principia's chief medical officer. "And we are pleased to see that the complete response (CR) rate in the Phase 2 Part B trial was 40 percent (six patients) despite most patients being initially treated at a sub-therapeutic dose 400 mg once daily."

Among the 15 patients dosed in Part B of the Phase 2 trial, nine (60 percent) achieved the primary endpoint of control of disease activity (CDA) by week four on low dose corticosteroids (less than or equal to 0.5 mg/kg per day). 12 of 15 (80 percent) patients achieved CDA by week 12. Nine (60 percent) of the 15 patients have achieved a PDAI score (a scoring system to indicate disease severity) of 1 or 0. Safety of PRN1008 is consistent with Phase 2 Part A trial and has been well-tolerated in Part B with no treatment related serious adverse event reported.

Final data from the Phase 2 Part B trial will be submitted for presentation at an upcoming medical conference.

#### About PEGASUS -- the Phase 3 Trial of PRN1008

Principia is currently enrolling patients in a global, randomized, double-blind, placebo-controlled, pivotal, Phase 3 clinical trial, the PEGASUS study, in approximately 120 patients to evaluate PRN1008 versus placebo using a background treatment of tapering doses of CS. The trial entry criteria include patients with moderate to severe pemphigus who are either newly diagnosed or relapsing with chronic disease. This demographic will potentially represent three quarters of the pemphigus patient population. The primary efficacy endpoint is the ability of PRN1008 to achieve durable CR on very low-dose CS (5 mg/day) at 37 weeks of treatment. Durable CR is defined as a state in which all lesions have healed, and no new lesions have appeared for a period of at least eight weeks. Key secondary endpoints include cumulative CS use and time to CR. After 37 weeks, all patients will have the option to be treated with active PRN1008 therapy in an open-label extension period of 24 weeks. PRN1008 has been granted orphan drug designation by the U.S. Food and Drug Administration for the treatment of patients with PV and by the European Commission for treatment of patients with pemphigus.

For more information on the PRN1008 pemphigus trials, please visit the [Patients](#) section of Principia's website.

#### About PRN1008

PRN1008, Principia's most advanced drug candidate, is an oral, small molecule, reversible covalent inhibitor of Bruton tyrosine kinase (BTK), which is present in the signaling pathways of most types of white blood cells except for T cells and plasma cells. PRN1008 is based on Principia's proprietary Tailored Covalency<sup>®</sup> platform to optimize PRN1008's safety and efficacy profile, resulting in prolonged and reversible action at the target site while being rapidly eliminated from the body. Principia believes this approach limits systemic exposure of PRN1008 and enables rapid clinical reversibility of effects on the immune system and is thus designed for use as a chronic therapy in immune-mediated diseases.

#### About Principia Biopharma

Principia is a late-stage biopharmaceutical company dedicated to bringing transformative oral therapies to patients with significant unmet medical needs in immune mediated diseases. Principia's proprietary Tailored Covalency<sup>®</sup> platform differentiates the company's investigational therapies from traditional small molecules and provides the potential to deliver the potency, selectivity and safety of injectable drugs while maintaining the convenience of a pill. This highly reproducible approach enables the company to pursue multiple programs efficiently. PRN1008, a reversible covalent BTK inhibitor, is being evaluated in a Phase 3 clinical trial in patients with pemphigus, an orphan autoimmune disease, and in a Phase 2 clinical trial in patients with immune thrombocytopenia (ITP), a rare hematological disease. PRN2246/SAR442168, a covalent BTK inhibitor which crosses the blood-brain barrier, is being evaluated in a Phase 2 clinical trial in patients with multiple sclerosis and is partnered with Sanofi. PRN1371, a covalent inhibitor of Fibroblast Growth Factor Receptor (FGFR) is being evaluated in a Phase 1 trial in patients with bladder cancer. For more information, please visit [www.principiabio.com](http://www.principiabio.com).

#### Forward-Looking Statements

This press release contains forward-looking statements. These forward-looking statements reflect the current beliefs and expectations of management made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to, Principia's expectations regarding the Principia pipeline of product candidates, the design of, progress of, results from, and timing of results from, its clinical trials and information regarding the timing, scope and success of additional clinical results. Such forward-looking statements involve known and unknown risks, uncertainties, and other important factors that may cause Principia's actual results, performance, or achievements to be materially different from those expressed or implied by the forward-looking statements. For a description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the Principia's business in general, see the risk factors set forth in Principia's reports filed with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Principia specifically disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

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Source: Principia Biopharma Inc.