Principia Announces Positive Preliminary Data of PRN1008 from its Ongoing Phase 2 Part B Trial in Pemphigus

October 10, 2019

Consistent efficacy and safety profile for pemphigus patients observed

Confirms 400mg twice daily dose in Phase 3 trial

Principia to host investor conference call at 8:00 am Eastern time

SOUTH SAN FRANCISCO, Calif., Oct. 10, 2019 (GLOBE NEWSWIRE) -- Principia Biopharma Inc. (Nasdaq: PRNB), a late-stage biopharmaceutical company focused on developing novel therapies for immune mediated diseases, today announced positive preliminary data from its Phase 2 pemphigus, open-label, trial.

“As we are actively recruiting patients for the Phase 3 PEGASUS trial, I believe it is important to recognize that, between the Phase 2 Parts A and B of the pemphigus trial, we now have been able to observe consistent safety and efficacy data in approximately 40 pemphigus patients,” said Dr. Murrell, professor and Head of the Department of Dermatology at The St. George Hospital Clinic School, University of New South Wales in Sydney, Australia, who is the Principal Investigator of the PEGASUS Phase 3 trial. “While these results are not final, I am very encouraged by the observed positive risk benefit of an oral BTK inhibitor in a debilitating disease where patients have limited treatment options.” Part B of the Phase 2 trial tested six months of PRN1008 therapy compared to three months of PRN1008 therapy in Part A.

Among all 15 patients in Part B of the Phase 2 trial, six patients (40 percent) have reached a complete response (CR) thus far, with four patients remaining on treatment who have the potential to still reach CR. There were five patients who were unable to achieve a CR (CR non-responders). To date, nine (60 percent) of the 15 patients enrolled in Part B have achieved a PDAI score (a scoring system to indicate disease severity) of 1 or 0. The primary efficacy endpoint (control of disease activity) results have been similar in Part A and Part B. In Part B, a starting dose of 400mg once daily was tested and it was determined to be less effective. At the recommendation of the Safety Monitoring Committee, all patients were escalated to twice daily dosing. Regarding safety, the preliminary data are consistent with Part A and no additional serious adverse events have been reported at doses up to 600mg twice daily. Additional data from this trial will be submitted for presentation at an upcoming medical conference and, with the last patient enrolled in June, Principia anticipates providing a further update by the end of the year.

Principia earlier this year announced positive Phase 2 Part A trial data that included 27 patients with pemphigus (including both pemphigus vulgaris and pemphigus foliaceus) in an open-label trial to evaluate PRN1008’s potential to induce rapid onset of clinical response, enable tapering and/or avoidance of corticosteroids (CS) use, and lower autoantibody levels. In Part A, the observed CR rate was 25 percent for those patients who had been on therapy for 12 weeks.

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 corticosteroids (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 complete remission on very low-dose CS (≤5 mg/day) after 36 weeks of treatment. Durable complete remission 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 complete remission. After 36 weeks, all patients will 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.

About PRN1008

PRN1008, Principia’s most advanced drug candidate, is an oral, small molecule, reversible covalent inhibitor of Bruton’s 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® 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.

Conference call information:

The call may be accessed by dialing 866-354-2347 (domestic) or 602-563-8496 (international) with the conference ID number of 3077829. Please dial in at least ten minutes in advance and inform the operator that you would like to join the “Principia Conference Call.” A replay of the call will be available approximately one hour after completion of the call and will be archived on Principia’s website.

About Principia Biopharma

Principia is a late-stage biopharmaceutical company focused on developing novel therapies for immune mediated diseases. Principia’s proprietary Tailored Covalency® 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 1/2 clinical trial in patients with immune thrombocytopenia (ITP), a rare hematological disease. PRN2246/SAR442168, a covalent BTK inhibitor that crosses the blood-brain barrier, has commenced a Phase 2 clinical trial in patients with multiple sclerosis, and has been 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.
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 potential of PRN1008 to rapidly and effectively treat pemphigus while significantly reducing the exposure to moderate to high CS doses, the safety and efficacy of PRN1008, the planned patient enrollment for the Phase 3 PEGASUS trial, and the timing, scope and success of additional clinical results (including, without limitation, the final results from Part B of the Phase 2 trial). 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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