

## Principia Announces Positive Preliminary Data of PRN1008 for Immune Thrombocytopenia in Ongoing Phase 1/2 Trial

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**Preliminary results from trial have been accepted as an oral presentation at upcoming American Society of Hematology Annual Meeting**

**SOUTH SAN FRANCISCO, Calif., October 15, 2019** – 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 an ongoing Phase 1/2 trial of its investigational treatment, PRN1008, in a highly treatment-resistant and refractory patient population (median of five prior therapies) with immune thrombocytopenia (ITP). Further updated data from the trial will be presented at an oral scientific session at the 61<sup>st</sup> American Society of Hematology Annual Meeting in December (ASH).

“We are very encouraged by the data so far and are pleased to see the scientific community view this as important new clinical data of our oral BTK inhibitor. We look forward to sharing updated analyses with the worldwide hematology community at an oral presentation at ASH,” said Dolca Thomas, MD, chief medical officer at Principia.

To date, the Phase 1/2 trial of PRN1008 has enrolled 26 adult patients who have had two platelet counts < 30,000/ $\mu$ L within 15 days prior to treatment. Oral PRN1008 starting doses were 200mg once daily, 400mg once daily, 300mg twice daily, and 400mg twice daily, with intra-patient dose escalation allowed every four weeks, with the trial having a current median treatment duration of 12.7 weeks (range 0.14 to 39.71).

Of the 26 patients enrolled to date, 39 percent (80% confidence interval (CI) 27.3, 51.0) achieved the trial’s primary endpoint of  $\geq 2$  consecutive platelet counts of  $\geq 50,000/\mu$ L, separated by at least five days, and increased by  $\geq 20,000/\mu$ L from baseline without requiring rescue medication. In addition, 46 percent (80% CI 34.2, 58.5) of enrolled patients achieved any 2 platelet counts  $\geq 50,000/\mu$ L. These results were observed despite the limited duration of therapy and patients at multiple escalating dose levels. In the preliminary data on 15 patients across all doses who had completed at least 12 weeks of therapy, the response rate was greater than 50 percent for both endpoints. To date PRN1008 has been well-tolerated at all doses studied, whether given as a monotherapy or with allowed concomitant ITP therapy, with no reported treatment related bleeding or thrombotic events.

These results are preliminary in nature and may change as additional patients are enrolled and progress in the trial. The company will present a further updated data set at the upcoming American Society of Hematology Annual Meeting in December.

### About ITP and PRN1008

Immune thrombocytopenia (ITP) is characterized by immune-mediated platelet destruction and impairment of platelet production, leading to downstream thrombocytopenia, a predisposition to bleeding, and adverse impact on patient quality of life. Unmet needs in relapsed or refractory ITP are to improve remission rates and durability by targeting underlying disease mechanisms. PRN1008 is an oral, reversible, covalent inhibitor of Bruton tyrosine kinase (BTK) that modulates immune-mediated processes in ITP. Preclinical PRN1008 data showed inhibition of B cell receptor-mediated activation of human B cells, Fc receptor (Fc-gamma and Fc-epsilon)-mediated activation of immune cells, and dose-dependent reduction in platelet loss in a mouse ITP model. In platelets from both normal healthy volunteer and ITP patients, clinically relevant concentrations of PRN1008 showed no effect on platelet aggregation or interference with other platelet agonists, in contrast to ibrutinib (Langrish et al. *ASH* 2017:1052).

### About Principia Biopharma

Principia is a late-stage biopharmaceutical company focused on developing novel therapies for 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 1/2 clinical trial in patients with ITP. 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 safety and efficacy of PRN1008, trial design, enrollment and progress, and the timing, scope and success of additional clinical results (including, without limitation, the Phase 1/2 trial of PRN1008 for ITP). 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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