Nplate® (romiplostim) – Expanded indication

- On December 14, 2018, Amgen announced the FDA approval of Nplate (romiplostim), for the treatment of pediatric patients 1 year of age and older with immune thrombocytopenia (ITP) for at least 6 months who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
    - Nplate was previously approved for the treatment of adult patients with chronic ITP who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
    - Nplate is not indicated for the treatment of thrombocytopenia due to myelodysplastic syndrome or any cause of thrombocytopenia other than chronic ITP.
    - Nplate should be used only in patients with ITP whose degree of thrombocytopenia and clinical condition increases the risk for bleeding.
    - Nplate should not be used in an attempt to normalize platelet counts.

- ITP is a rare, serious autoimmune disease characterized by low platelet counts in the blood and impaired platelet production. In the U.S., the estimated prevalence of ITP in children is 5.3 per 100,000 children annually. The treatment goal for children with ITP is to achieve and maintain a platelet count that reduces the risk of bleeding.

- The expanded indication for Nplate was based on data from two placebo-controlled studies in pediatric patients. In the first study (phase 3), efficacy was measured by the proportion of patients achieving a durable platelet response and the proportion of patients achieving an overall platelet response (defined as a durable or transient platelet response). In the second study (phase 1/2), efficacy was measured by the proportion of patients who achieved a platelet count of $\geq 50 \times 10^9$/L for 2 consecutive weeks and by the proportion of patients who achieved an increase in platelet count of $\geq 20 \times 10^9$/L above baseline for 2 consecutive weeks.
    - In the first study, durable platelet response was achieved in 52% and 10% of patients in the Nplate and placebo arms, respectively. Overall platelet response was achieved in 71% and 20% of patients in the Nplate and placebo arms, respectively ($p < 0.05$ for both).
    - Of the 17 patients who received Nplate in the second study, 15 achieved a platelet count of $\geq 5 \times 10^9$/L for 2 consecutive weeks (88.2%, 95% CI: 63.6%, 98.5%). The same 15 patients also achieved an increase in platelet count of $\geq 20 \times 10^9$/L above baseline for 2 consecutive weeks during the treatment period. None of the patients treated with placebo achieved either endpoint.

- The recommended initial dose of Nplate is 1 mcg/kg in pediatric patients with ITP. Actual body weight at initiation of treatment should always be used when calculating initial dose.
    - Future dose adjustments are based on changes in platelet counts and changes in body weight. Reassessment of body weight is recommended every 12 weeks.
    - Refer to the Nplate drug label for additional dosing information for pediatric and adult patients.