Generic Name:
Romiplostim
Trade Name:
Nplate
Company:
Amgen
Notes:

FDA approved romiplostim for pediatric patients aged 1 year and older who have had immune thrombocytopenia (ITP) for at least 6 months and have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.

Approval was based on two double-blind, placebo-controlled clinical trials in pediatric patients aged 1 year and older with ITP for at least 6 months' duration. In one study (NCT01444417), patients whose disease was refractory or relapsed after at least one prior ITP therapy were randomized (2:1) to receive romiplostim (n = 42) or placebo (n = 20). Durable platelet response (at least 6 weekly platelet counts ? 50 × 109/L during weeks 18 through 25 of treatment) was achieved in 22 patients (52%) who received romiplostim and 2 (10%) in the placebo arm.

Overall platelet response, defined as a durable or a transient platelet response, was achieved in 30 (71%) and 4 (20%) patients, respectively. Patients who received romiplostim had platelet counts ? 50 x 109/L for a median of 12 weeks, compared with 1 week in patients who received placebo. The results for all three endpoints were statistically significant, with *P* values all less than 0.05.

In the other study (NCT00515203), patients diagnosed with ITP at least 6 months prior to enrollment were randomized (3:1) to receive romiplostim (n = 17) or placebo (n = 5). Fifteen patients who received romiplostim achieved a platelet count ? 50 x 109/L for 2 consecutive weeks and an increase in platelet count of ? 20 × 109/L above baseline for 2 consecutive weeks during the treatment period (88%, 95% CI 64?99). No patient receiving placebo achieved either endpoint.

In pediatric patients, the most common adverse reactions (?25%) included contusion, upper respiratory tract infection, and oropharyngeal pain.

The recommended initial romiplostim dose for pediatric patients is 1 mcg/kg on the basis of actual body weight and administered as a weekly S.C. injection. Dose should be adjusted in increments of 1 mcg/kg until the patient achieves a platelet count of ? 50 x 109/L. Reassessment of body weight is recommended every 12 weeks.

Medication Monitor Categories:
Supplemental Approvals