NPLATE
(romiplostim)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Nplate (romiplostim) works as an analog to the protein thrombopoietin (TPO) and binds to the TPO receptor, similar to endogenous TPO to stimulate platelet production (1).

Regulatory Status

FDA-approved indications: Nplate is a thrombopoietin receptor agonist indicated for the treatment of thrombocytopenia in: (1)

1. Adult patients with immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy
2. Pediatric patients 1 year of age and older with ITP for at least 6 months who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.

Npate is also indicated to increase survival in adults and in pediatric patients (including term neonates) acutely exposed to myelosuppressive doses of radiation (Hematopoietic Syndrome of Acute Radiation Syndrome [HS-ARS]).

Limitations of Use: (1)

- Nplate is not indicated for the treatment of thrombocytopenia due to myelodysplastic syndrome (MDS) 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.

Nplate may increase blast cell counts and cause risk of progression to acute myelogenous leukemia. Platelet count increases may also increase the risk of thrombosis. Thrombocytopenia may occur in rare cases due to the formation of Nplate reactive antibodies (1).

Nplate must be held when platelet levels reach >400 x 10^9/L (400,000 platelets per microliter) and platelet levels monitored weekly to evaluate any decrease in levels and need for re-initiation of therapy. If platelet levels remain above 400 x 10^9/L (400,000 platelets per microliter) after two weeks, Nplate therapy must be discontinued (1).

The use of Nplate to increase survival in pediatric patients (including term neonates) acutely

exposed to myelosuppressive doses of radiation is based on efficacy studies conducted in adult animals. Efficacy studies of Nplate could not be conducted in humans with acute radiation syndrome for ethical and feasibility reasons (1).

The safety and effectiveness of Nplate in pediatric patients less than 1 year of age with ITP have not been established (1).

Summary

Nplate (romiplostim) works as an analog to the protein thrombopoietin (TPO) and binds to the TPO receptor, similar to endogenous TPO to stimulate platelet production. The use of Nplate to increase survival in pediatric patients (including term neonates) acutely exposed to myelosuppressive doses of radiation is based on efficacy studies conducted in adult animals. Efficacy studies of Nplate could not be conducted in humans with acute radiation syndrome for ethical and feasibility reasons. The safety and efficacy of Nplate in patients less than 1 year of age with ITP have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Nplate while maintaining optimal therapeutic outcomes.

References

1. Nplate [package insert]. Thousand Oaks, CA: Amgen Inc.; February 2022.