Nplate

Description

Nplate (romiplostim)

Background
Nplate is used to treat adult patients with immune thrombocytopenia (ITP) who have not responded adequately to corticosteroids, immunoglobulins, or to the removal of their spleen (splenectomy). Patients with ITP experience low numbers of platelets and have a higher risk of serious bleeding. Nplate works as an analog to the protein thrombopoietin (TPO), and binds to the TPO receptor similar to 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.

Limitations of Use:
- 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 (1).
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 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 after two weeks, Nplate therapy must be discontinued (1).

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

**Related policies**

Cablivi, Promacta

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**Policy**

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Nplate may be considered **medically necessary** in patients 1 year of age or older for the treatment of immune thrombocytopenia (ITP) and if the conditions indicated below are met.

Nplate may be considered **investigational** for patients that are less than 1 year of age and for all other indications.

**Prior-Approval Requirements**

**Age**

1 year of age or older

**Diagnosis**

Patient must have the following:

Immune thrombocytopenia (ITP)

AND ALL of the following:

1. Inadequate response or intolerant to corticosteroids, immunoglobulins, or splenectomy
2. Platelet count at time of diagnosis less than 50,000 platelets per microliter
3. **Age 1-17 only:** Patient has had ITP for at least 6 months
4. **NOT** used in combination with another thrombopoietin receptor agonist or with Tavalisse (fostamatinib disodium hexahydrate)

**Prior – Approval Renewal Requirements**

**Age**

1 year of age or older

**Diagnosis**

Patient must have the following:

- Immune thrombocytopenia (ITP)

**AND ONE** of the following:

1. Platelet count 50,000 - 200,000 platelets per microliter
2. Platelet count greater than or equal to 200,000 platelets per microliter or less than or equal to 400,000 platelets per microliter with agreement that therapy will be adjusted to the minimum platelet count needed to reduce the bleeding risk

**AND** the following:

1. **NOT** used in combination with another thrombopoietin receptor agonist or with Tavalisse (fostamatinib disodium hexahydrate)

**Policy Guidelines**

**Pre - PA Allowance**

None

**Prior - Approval Limits**

**Duration**

12 months

**Prior – Approval Renewal Limits**

Same as above

**Rationale**
Summary
Nplate works as an analog to the protein thrombopoietin (TPO), and binds to the TPO receptor similar to TPO to stimulate platelet production. Nplate is used to treat patients with immune thrombocytopenia (ITP) who have not responded adequately to corticosteroids, immunoglobulins, or to the removal of their spleen (splenectomy). Nplate should not be used in patients with myelodysplastic syndrome (MDS). The safety and efficacy of Nplate in patients less than 1 year of age 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

Policy History

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<td>December 2014</td>
<td>Addition to PA</td>
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<tr>
<td>March 2015</td>
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<td>March 2016</td>
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<td>Policy number change from 5.10.20 to 5.85.20</td>
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<td>December 2016</td>
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<td>Added age limit to renewal section</td>
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<td>September 2017</td>
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<td>Verbiage for platelet count changed from $10^9$/L to number of platelets per microliter</td>
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<td>September 2018</td>
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<td>Addition of “no dual therapy with Tavalisse” to initiation and renewal criteria</td>
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<td>December 2018</td>
<td>Addition of new indication: pediatric patients 1 year of age and older with ITP for at least 6 months who have had an inadequate response to corticosteroids, immunoglobulins, or splenectomy</td>
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Keywords
This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective January 1, 2020.