**POLICY:** Thrombocytopenia – Nplate® (romiplostim injection for subcutaneous use – Amgen)

**APPROVAL DATE:** 07/03/2019

**OVERVIEW**

Nplate, a thrombopoietin receptor agonist, is indicated for the treatment of thrombocytopenia in adults with chronic immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Also, Nplate is indicated for use in patients ≥ 1 year of age with ITP for at least 6 months who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Nplate should only be utilized in patients with ITP whose degree of thrombocytopenia and clinical condition increase the risk for bleeding. Nplate should not be used in an attempt to normalize platelet counts. The initial Nplate dose is 1 mcg/kg once weekly as a subcutaneous (SC) injection by a healthcare provider. The dose should be adjusted weekly by increments of 1 mcg/kg to achieve and maintain a platelet count ≥ 50 x 10^9/L as needed to reduce the bleeding risk. Do not exceed a maximum weekly dose of 10 mcg/kg. Discontinue Nplate if the platelet count does not increase after 4 weeks at the maximum dose.

Guidelines

**ITP**

In 2011 the American Society of Hematology published an evidence-based practice guideline for immune thrombocytopenia. First-line treatment for adults includes corticosteroids or intravenous immunoglobulin (IVIG). For patients who are unresponsive or relapse after initial corticosteroid therapy splenectomy is recommended. Thrombopoietin receptor agonists are recommended for patients with a bleeding risk who relapse following splenectomy, or have a contraindication to splenectomy and who have failed at least one other therapy. The guidelines also suggest that thrombopoietin receptor agonists be considered for those at risk of bleeding who have failed one line of therapy, such as corticosteroids or IVIG, and who have not undergone splenectomy.

**Myelodysplastic Syndrome (MDS)**

National Comprehensive Cancer Network recommendations regarding MDS (version 2.2019 – October 18, 2018) state to consider treatment with a thrombopoietin receptor agonist in patients with lower-risk MDS who have severe or life-threatening thrombocytopenia. Data are available that describe the use of Nplate in patients with MDS. The data with Nplate are discussed noting an increased rate of platelet response and decreased overall bleeding events among patients with low to intermediate risk MDS.

**POLICY STATEMENT**

Prior authorization is recommended for medical benefit coverage of Nplate. Approval is recommended for those who meet the Criteria and Dosing for the listed indication(s). Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Nplate as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Nplate to be prescribed by or in consultation with a physician who specializes in the condition being treated.

**RECOMMENDED AUTHORIZATION CRITERIA**

Coverage of Nplate is recommended in those who meet one of the following criteria:
FDA-Approved Indications

1. **Chronic Immune Thrombocytopenia.** Approve for 1 year if the patient meets the following criteria (A and B):
   
   A) The agent is prescribed by or in consultation with a hematologist; AND
   
   B) The patient meets one of the following criteria (i or ii):
      
      i. The patient has tried at least one other therapy. Note: Examples of therapies are corticosteroids, intravenous immunoglobulin, anti-D immunoglobulin, Promacta® (eltrombopag tablets and oral suspension), Tavalisse™ (fostamatinib tablets), Doptelet® (avatrombopag tablets) and rituxumab; OR
      
      ii. The patient has undergone splenectomy.

   **Dosing.** Approve up to 10 mcg/kg SC no more frequently than once weekly.

Other Uses with Supportive Evidence

2. **Thrombocytopenia in Myelodysplastic Syndrome.** Approve for 1 year if the patient meets the following criteria (A, B, and C):

   A) The agent is prescribed by or in consultation with a hematologist or an oncologist; AND
   
   B) The patient has low- to intermediate-risk MDS; AND
   
   C) According to the prescriber the patient has clinically significant thrombocytopenia (e.g., low platelet counts [< 30 x 10⁹/L] [< 30,000/µL] [pretreatment]; is platelet transfusion-dependent; active bleeding; and/or a history of bleeding at low platelet counts).

   **Dosing.** Approve up to 1,500 mcg SC no more frequently than twice weekly.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

1. Coverage is not recommended for circumstances not listed in the Authorization Criteria (FDA-approved indications and Other Uses with Supportive Evidence). Criteria will be updated as new published data are available.

REFERENCES


**HISTORY**

<table>
<thead>
<tr>
<th>Type of Revision</th>
<th>Summary of Changes</th>
<th>Approval Date</th>
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</thead>
<tbody>
<tr>
<td>New policy</td>
<td></td>
<td>01/30/2019</td>
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<tr>
<td>Early annual revision</td>
<td>The following criteria changes were made.</td>
<td></td>
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<tr>
<td></td>
<td>1. <strong>Chronic Immune Thrombocytopenia:</strong> The approval duration was changed from 3 year to 1 year. Doptelet was added to the list of alternatives that count towards the criteria that requires a trial of one other therapy. The dosing was changed to approve up to 10 mcg/kg SC no more frequently than once weekly.</td>
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<td>2. <strong>Myelodysplastic Syndrome:</strong> Dosing was changed to approve up to 1,500 mcg SC no more frequently than twice weekly.</td>
<td>07/03/2019</td>
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