**PRIOR AUTHORIZATION POLICY**

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

**TAC 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 prescription benefit coverage of Nplate. 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 efficacy, approval requires Nplate to be prescribed by or in consultation with a physician who specializes in the condition being treated.

**Automation:** None.
RECOMMENDED AUTHORIZATION CRITERIA
Coverage of Nplate is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Chronic Immune Thrombocytopenia (ITP).** 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 disodium hexahydrate tablets), Doptelet® (avatrombopag tablets), or rituximab; OR
   ii. The patient has undergone splenectomy.

Other Uses with Supportive Evidence

2. **Thrombocytopenia in Myelodysplastic Syndrome (MDS).** 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 10^9/L {< 30,000/µL} (pretreatment); is platelet transfusion-dependent; active bleeding; and/or a history of bleeding at low platelet counts).

CONDITIONS NOT RECOMMENDED FOR APPROVAL
Nplate has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. Rationale for non-coverage for these specific conditions is provided below. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval.)

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. 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>TAC Approval Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Annual revision</td>
<td>The name of the policy was changed to add the header “Thrombopoietin Receptor Agonists”. The diagnosis of thrombocytopenia in MDS was removed from the “Conditions Not Recommended for Approval” section and added to the “Other Uses with Supportive Evidence”. Criteria are to approve for 12 months if the agent is prescribed by, or in consultation with, a hematologist or an oncologist; if the patient has low- to intermediate-risk MDS; and if, according to the prescribing physician, the patient has clinically significant thrombocytopenia (examples listed).</td>
<td>06/14/2017</td>
</tr>
<tr>
<td>Annual revision</td>
<td>The wording of the diagnosis of ITP was changed from “Treatment of Thrombocytopenia in Patients with Chronic Immune (Idiopathic) Thrombocytopenia Purpura” to “Chronic Immune Thrombocytopenia”. Also, for chronic ITP, the wording regarding medication trials was changed. Previously, patients were required to meeting one of the following conditions: 1) the patient has tried corticosteroids; 2) the patient has tried IVIG; OR 3) the patient has undergone splenectomy. This criteria wording was changed to state that the patient meets one of the following criteria: 1) the patient has tried one other therapy (e.g., corticosteroids, IVIG, anti-D immunoglobulin, Promacta, Tavalisse, or Rituxan) OR 2) the patient has undergone splenectomy.</td>
<td>06/27/2018</td>
</tr>
<tr>
<td>Annual revision</td>
<td>The following criteria changes were made. 1. <strong>Chronic Immune Thrombocytopenia</strong>: The approval duration was changed from 3 years to 1 year. Doptelet was added to the list of alternatives that count towards the criteria that requires a trial of one other therapy.</td>
<td>07/03/2019</td>
</tr>
</tbody>
</table>

*TAC – Therapeutic Assessment Committee; * For a further summary of criteria changes, refer to respective TAC minutes available at: [http://esidepartments/sites/Dep043/Committees/TAC/Forms/AllItems.aspx](http://esidepartments/sites/Dep043/Committees/TAC/Forms/AllItems.aspx); MDS – Myelodysplastic syndrome; ITP – Immune cytopenia purpura; IVIG – Intravenous immunoglobulin.*