MEDICAL MUTUAL®

Drug Policy

Policy:	**201521**	**Initial Effective Date: 09/30/2015**
Code(s):	**HCPCS J2796**	**Annual Review Date: 07/19/2018**
SUBJECT:	**Nplate® (romiplostim for subcutaneous injection)**	**Last Revised Date: 01/22/2019**

Prior approval is required for some or all procedure codes listed in this Corporate Drug Policy.

OVERVIEW

Nplate, a thrombopoietin receptor agonist, is indicated for the treatment of thrombocytopenia in patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. 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 administered by a healthcare provider. The dose should be adjusted weekly by increments of 1 mcg/kg to achieve and maintain a platelet count $\geq 50 \times 10^9$/L as needed to reduce the bleeding risk. Do not exceed a maximum weekly dose of 10 mcg/kg. Do not administer Nplate if the platelet count is $> 400 \times 10^9$/L. Discontinue Nplate if the platelet count does not increase after 4 weeks at the maximum dose.

Pivotal trials with Nplate involved patients who had tried at least one primary ITP therapy (e.g., corticosteroids, immunoglobulins); approximately 50% of patients had undergone splenectomy. Evidence-based practice guidelines for ITP from the American Society of Hematology, published in 2011, recommend corticosteroids or IVIG as first-line treatment for adults. Splenectomy is recommended for patients who have failed corticosteroid therapy. Thrombopoietin receptor agonists are recommended for adults at risk of bleeding who relapse following splenectomy or who have a contraindication to splenectomy and who have failed at least one other therapy. A Phase III, randomized, double-blind, placebo-controlled study investigated use of Nplate in children with immune thrombocytopenia who were aged 1 year to 17 years (n = 62) with mean platelet counts $\leq 30 \times 10^9$/L. Patients received weekly Nplate for 24 weeks with the dose adjusted from 1 mcg/kg to 10 mcg/kg SC once weekly to target platelet counts of 50 to 200×10^9/L. Durable platelet responses were observed in 52% of patients in the Nplate group (n = 22/42) compared with 10% of patients (n = 2/20) given placebo.

POLICY STATEMENT

Prior authorization is recommended for pharmacy and medical 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. **All medical benefit approvals for the Treatment of Thrombocytopenia in Patients with Chronic Immune Thrombocytopenia (ITP)** will be for up to 10 mcg/kg and are provided for 12 months in duration unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA
Coverage of Nplate is recommended in those who meet the following criteria:

Food and Drug Administration (FDA)-Approved Indication

1. **Treatment of Thrombocytopenia in Patients with Chronic Immune Thrombocytopenia (ITP).** Approve Nplate for 12 months if the patient meets the following criteria (a, b, c, d and e):
   a) The agent is prescribed by, or in consultation with, a hematologist; AND
   b) The patient is ≥ 1 years of age; AND
   c) Not using Nplate in combination with Promacta; AND
   d) The patient meets ONE of the following conditions (i or ii) AND;
      i. Current platelet count is less than $30x10^9$/L; OR
      ii. Current platelet count is less than $30x10^9$/L to $50x10^9$/L with symptomatic bleeding (eg, significant mucous membrane bleeding, gastrointestinal bleeding or trauma) or risk factors for bleeding
   e) The patient meets one of the following criteria (i or ii):
      i. The patient had an inadequate response or intolerance to prior therapy (e.g., corticosteroids, intravenous immunoglobulin, anti-D immunoglobulin, Promacta® [eltrombopag tablets and oral suspension], Tavalisse™ [fostamatinib disodium hexahydrate tablets], or Rituxan® [ritixumab injection for intravenous use]); OR
      ii. The patient has undergone splenectomy.

   Nplate is indicated for the treatment of thrombocytopenia in patients with chronic ITP who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.[1] . The pivotal trials with Nplate involved patients who had tried at least one primary ITP therapy (e.g., corticosteroids, immunoglobulins); approximately 50% of patients had undergone splenectomy.[1] Evidence-based practice guidelines for immune thrombocytopenia from ASH (published in 2011), recommend corticosteroids or IVIG as first-line treatment for adults; splenectomy is recommended for patients who have failed corticosteroid therapy. Thrombopoietin receptor agonists are recommended for adults at risk of bleeding who relapse following splenectomy or who have a contraindication to splenectomy and who have failed at least one other therapy.

   Dosing in Treatment of Thrombocytopenia in Patients with Chronic ITP (*Medical benefit only*). *FDA recommended dosing*:
   The initial dose for Nplate is 1 mcg/kg based on actual body weight; do not exceed a maximum weekly dose of 10 mcg/kg.

2. **Patient Has Been Started on Nplate.** Approve for an indication or condition addressed as an approval in the Recommended Authorization Criteria section (FDA-Approved Indications) if the patient meets the following criteria (A, B and C):
   A) Patient continues to respond to therapy with this drug (e.g. platelet count has increased); AND
   B) Patient's platelet count is at least 50 x 10^9/L and does not exceed 400 x 109/L; AND
   C) Not using Nplate in combination with Promacta.

Labs/Diagnostics.
For patients 1 years of age to 18 years of age, actual body weight should be assessed every 12 weeks

Other Uses with Supportive Evidence

1. **Thrombocytopenia in Myelodysplastic Syndrome (MDS).** Approve Nplate for 12 months 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 prescribing physician the patient has clinically significant thrombocytopenia (e.g., low platelet counts [< 30,000 mm^3 {pretreatment}]; is platelet transfusion-dependent; active bleeding; and/or a history of bleeding at low platelet counts).

Data are available that describe the use of Nplate in patients with MDS.[6-8,18] Current recommendations from the National Comprehensive Cancer Network (NCCN) regarding MDS [version 2.2018] state to consider treatment with a thrombopoietin-receptor agonist in patients with lower-risk MDS who have severe or life-threatening thrombocytopenia.[5] 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.

Dosing in treatment of MDS: Romiplostim 500 mcg or 750 mcg subQ was administered once weekly

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. Nplate should not be used in an attempt to normalize platelet counts per FDA labeling.
2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:
The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

References

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Other References Utilized

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FOR MEDICAL BENEFIT COVERAGE REQUESTS:

Prior approval is required for HCPCS Codes J2796

HCPCS Code(s):	
J2796	Injection, romiplostim, 10 micrograms