Nplate® (romiplostim) Injection

Policy Number: MC/PC 027
Effective Date: March 1, 2026

Instructions for Use

Table of Contents **Page**

Related Policies

- n/a

Coverage Rationale

Immune Thrombocytopenia (ITP)

For initial coverage of Nplate (romiplostim) for Immune Thrombocytopenia (ITP), the following will be required:

- Diagnosis of one of the following:
  - Immune thrombocytopenia (ITP)
  - Relapsed/refractory ITP **and**
- Baseline platelet count is less than 30,000/mcL **and**
- One of the following:
  - Patient has had a prior splenectomy
  - Trial and failure, contraindication, or intolerance to one of the following:
    - Corticosteroids (e.g., prednisone, methylprednisolone)
    - Immune globulins [e.g., Gammagard, immune globulin (human)] **and**
- Prescribed by or in consultation with a hematologist/oncologist

For reauthorization coverage of Nplate (romiplostim), the following will be required:

- Patient demonstrates positive clinical response to therapy as evidenced by an increase in platelet count to a level sufficient to avoid clinically important bleeding

Hematopoietic Syndrome of Acute Radiation Syndrome

For initial coverage of Nplate (romiplostim) for Hematopoietic Syndrome of Acute Radiation Syndrome, the following will be required:

- Diagnosis of hematopoietic syndrome of acute radiation syndrome **and**
- Patient is acutely exposed to myelosuppressive doses of radiation **and**
- Prescribed by or in consultation with a hematologist/oncologist

Applicable Codes

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Listing of a code in this policy does not imply that the service described by the code is a covered or non-covered health service. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other Policies and Guidelines may apply.

HCPCS Code	Description
J2802	Injection, romiplostim, 1 mcg

ICD-10 Code	Description
D69.3	Immune thrombocytopenic purpura

Background

Primary immune thrombocytopenia (ITP), also called idiopathic thrombocytopenic purpura or immune thrombocytopenic purpura, is an acquired thrombocytopenia caused by autoantibodies against platelet antigens (Arnold & Cuker 2025). The annual incidence of ITP is approximately 1 to 6 per 100,000 adults. ITP diagnosis is classified as primary or as secondary (to another disease) and as acute (of six months or less in duration) or chronic (12 months or greater). The presentation of symptoms varies among individuals and can include severe thrombocytopenia, with bleeding ranging from none to skin and mucosal bleeding, to intracranial, gastrointestinal (GI), and genitourinary bleeding (Bussel et al 2018). Serious bleeding is seen most often in patients with platelet counts < 20 x 10^9/L (Arnold and Cuker 2025).

Clinical Evidence

Immune Thrombocytopenia (ITP)
A pooled analysis of 13 clinical trials (N = 1111) evaluated the safety and efficacy of romiplostim in splenectomized and nonsplenectomized adults with primary ITP (Cines et al 2017). At baseline, splenectomized patients had a longer median duration of ITP and a lower median platelet count, as well as a higher proportion with > 3 prior ITP treatments vs nonsplenectomized patients.

- A platelet response (≥ 1 platelet count ≥ 50 x 10^9/L without rescue medication use in the previous 4 weeks) was attained in 82% of splenectomized patients and 91% of nonsplenectomized patients.
- A sustained platelet response (ie, platelet counts ≥ 50 x 10^9/L for 9 out of 12 weeks [75% of weekly assessments] with no use of rescue medication during the 4 weeks prior to each qualifying platelet count) was attained in 68% of splenectomized patients and 80% of nonsplenectomized patients. Response rates and sustained response rates were lower in splenectomized patients than in nonsplenectomized patients ($p < 0.001$).
- The use of rescue medication was higher in splenectomized than in nonsplenectomized patients within each treatment group. In both splenectomized and nonsplenectomized patients, the use of corticosteroids, IVIg, anti-Rh0(D) immunoglobulin infusion (anti-D), and rituximab decreased from baseline with long-term treatment and follow-up.

Acute Radiation Syndrome
Efficacy studies of romiplostim could not be conducted in humans with acute radiation syndrome for ethical and feasibility reasons. Approval for this indication was based on efficacy studies conducted in animals, romiplostim's effect on platelet count in healthy human volunteers, and on data supporting romiplostim's effect on thrombocytopenia in patients with ITP and insufficient response to corticosteroids, immunoglobulins, or splenectomy (Nplate prescribing information 2025).

Clinical Guidelines:

Clinical guidelines that provide recommendations for the treatment of ITP incl ogy (ASH) 2019 evidence-based practice guideline for ITP and the 2022 review of tl 9, Neunert et al 2024) and the international consensus report on the investigation and management of primary ITP (Provan et al 2019).

The ASH guidelines advocate a stepwise approach to the management of ITP, with treatment decisions guided by disease duration, prior therapeutic response, and individual patient preferences and values. The guidelines highlight the importance of shared decision-making, with therapy selection based on a balanced consideration of efficacy, safety, durability of response, and treatment burden. The guidelines have not yet incorporated fostamatinib as a potential second-line therapy.

- For advanced-line (second-line or later) therapy in adults with ITP, the guidelines suggest (all conditional recommendations):
    - Either eltrombopag or romiplostim in adults with ITP for ≥ 3 months who are corticosteroid-dependent or unresponsive to corticosteroids and are going to be treated with a TPO-RA
        - Based on the body of available evidence, it is likely there is no difference between eltrombopag and romiplostim.
        - Individual patient preference may place higher value on use of a daily oral medication or weekly SC injection.
        - Ongoing comparative effectiveness research of the different TPO-RA, inclusive of newer agents such as avatrombopag, is needed.
    - Either splenectomy or a TPO-RA in adults with ITP lasting ≥ 3 months who are corticosteroid-dependent or have no response to corticosteroids
        - If possible, splenectomy should be delayed for ≥ 1 year after diagnosis to allow for spontaneous remission in the first year.
    - Treatment with rituximab rather than splenectomy in adults with ITP lasting ≥ 3 months who are corticosteroid-dependent or have no response to corticosteroids
    - A TPO-RA rather than rituximab in adults with ITP lasting ≥ 3 months who are corticosteroid-dependent or have no response to corticosteroids
- The 2022 review highlighted that the 2019 guideline recommendations continue to be relevant, including recommendations related to second-line therapy for adults. The authors indicated that there was insufficient evidence to justify a revision of the entire guideline at the time of publication of the review.

The international consensus guidelines state that there is a lack of studies addressing the correct sequencing of subsequent therapies following relapse after cessation of corticosteroid treatment. The main goal of subsequent treatment is to attain a sustained increase in the platelet count that is considered hemostatic for the individual patient while minimizing AEs and allowing for the possibility of attaining a remission (defined as platelet count ≥ 30 x 10^9/L in the absence of any ITP-specific treatment).

- Guideline consensus statements indicate that:
    - Recommendations should be modified based on available resources and patient preference.
    - Some medical options may require ongoing continued treatment.
    - Splenectomy should be deferred for ≥ 1 year to allow for remission.
- Medical therapies with robust evidence include TPO-RAs, rituximab, and fostamatinib.
    - TPO-RAs have provided excellent responses (> 60%) in splenectomized and nonsplenectomized patients (Grade A recommendation). Response to continued TPO-RAs persists for up to 6 to 8 years and often allows other ITP therapy to be reduced or discontinued. Cessation of treatment will lead to the return of thrombocytopenia in most cases, but some patients (10% to 30%) may achieve a durable response after TPO-RAs are tapered and withdrawn.
    - Available evidence shows a response to rituximab in 60% of patients. Long-term durable responses occur in 20% to 25% of adult patients (Grade B recommendation).
    - Fostamatinib offers an alternative mechanism for reducing platelet destruction; it may provide response rates of 43% but stable responses of only 18%.
- Medical therapies with less robust evidence:

- Immunosuppressive agents (including mycophenolate mofetil [illegible]rine) may be used in patients failing other therapies. Danazol and d[illegible]gents that may be particularly useful in some patients (e.g., those in whom splenectomy is contraindicated or if other agents are unavailable) (Grade B recommendation).

- Adults failing multiple therapies:
  - The adequacy of prior therapies should be reassessed (e.g., reevaluate TPO-RA dose or addition of a small dose of corticosteroid to improve response).
  - Reassess the possibility of splenectomy if not already performed.
  - Other medical therapies should be considered if not already attempted (e.g., MMF, fostamatinib, rituximab, azathioprine, dapsone, danazol).
  - Switching from one TPO-RA to another and sequential therapy have been shown to have a positive effect on response and AEs.

U.S. Food and Drug Administration (FDA)

This section is to be used for informational purposes only. FDA approval alone is not a basis for coverage.

Nplate® is a thrombopoietin receptor agonist indicated for the treatment of thrombocytopenia in the following:

- Adult patients with immune thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy.
- 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.
- Nplate is 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]).

References

1. Nplate® [prescribing information]. Thousand Oaks, CA: Amgen; February 2025.
2. American Society of Hematology 2019 guidelines for immune thrombocytopenia. Available at: https://ashpublications.org/bloodadvances/article/3/23/3829/429213/American-Society-of-Hematology-2019-guidelines-for. Accessed January 21, 2025.
3. Bussel J, Arnold DM, Boxer MA, et al. Long-term fostamatinib treatment of adults with immune thrombocytopenia during the phase 3 clinical trial program. *Am J Hematol*. 2019;94(5):546-553. doi: 10.1002/ajh.25444.
4. Cines DB, Blanchette VS. Immune thrombocytopenic purpura. New England Journal of Medicine 2002; 346:995-1008.
5. Cines DB, Wasser J, Rodeghiero F, et al. Safety and efficacy of romiplostim in splenectomized and nonsplenectomized patients with primary immune thrombocytopenia**.** *Haematologica*. 2017;102(8):1342-1351. doi: 0.3324/haematol.2016.161968.
6. Arnold DM, Cuker A. Immune thrombocytopenia (ITP) in adults: Clinical manifestations and diagnosis. UpToDate Web site. 2026. Last updated October 24, 2025. http://www.uptodate.com. Accessed January 14, 2026.
7. InterQual 2020, April 2020 Release CP: Specialty Rx Non-Oncology, 2021 Change Healthcare LLC.
8. Kuter DJ, Bussel JB, Lyons RM, et al. Efficacy of romiplostim in patients with chronic immune thrombocytopenic purpura: a double-blind randomised controlled trial. Lancet. 2008; 371:395-403.
9. Neunert C, Terrell DR, Arnold DM, et al. American Society of Hematology 2019 guidelines for immune thrombocytopenia. Blood Adv. 2019;3(23):3829-3866.

10. Neunert CE, Arnold DM, Grace RF, Kuhne T, McCrae KR, Terrell DR. The 20[illegible]ty of Hematology guidelines on immune thrombocytopenia. Blood Adv. 2024;8(doi.org/10.1182/bloodadvances.2023012541.
11. Provan D, Arnold DM, Bussel JB, et al. Updated international consensus report on the investigation and management of primary immune thrombocytopenia. Blood Adv. 2019; 3(22): 3780-3817.
12. Toltl LJ, Arnold DM. Pathophysiology and management of chronic immune thrombocytopenia. British Journal of Hematology 2011; 152:52-60.

Policy History/Revision Information

Date	Summary of Changes
12/13/2023	Approved by OptumRx P&T Committee
2/15/2024	Annual Review. No changes made. Updated references.
2/20/2025	Annual Review. No changes made. Updated references.
2/19/2026	Annual Review. Updated the following: Coverage Rational for ITP, HCPCS code, Clinical Evidence, Clinical Guidelines and References.

Instructions for Use

This Medical Benefit Drug Policy provides assistance in interpreting standard benefit plans. When deciding coverage, the member specific benefit plan document must be referenced as the terms of the member specific benefit plan may differ from the standard plan. In the event of a conflict, the member specific benefit plan document governs. Before using this policy, please check the member specific benefit plan document and any applicable federal or state mandates. The insurance reserves the right to modify its Policies and Guidelines as necessary. This Medical Benefit Drug Policy is provided for informational purposes. It does not constitute medical advice.

OptumRx may also use tools developed by third parties to assist us in administering health benefits. OptumRx Medical Benefit Drug Policies are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice.

Nondiscrimination & Language Access Policy

Discrimination is Against the Law. Aspirus Health Plan, Inc. complies with applicable Federal civil rights laws and does not discriminate on the basis of race, color, national origin, age, disability, or sex, (including sex characteristics, including intersex traits; pregnancy or related conditions; sexual orientation, gender identity and sex stereotypes), consistent with the scope of sex discrimination described at 45 CFR § 92.101(a)(2). Aspirus Health Plan, Inc. does not exclude people or treat them less favorably because of race, color, national origin, age, disability, or sex.

Aspirus Health Plan, Inc.:
Provides people with disabilities reasonable modifications and free appropriate auxiliary aids and services to communicate effectively with us, such as:

- Qualified sign language interpreters.
- Written information in other formats (large print, audio, accessible electronic formats, other formats).

Provides free language assistance services to people whose primary language is not English, which may include:

- Qualified interpreters.
- Information written in other languages.

If *you* need reasonable modifications, appropriate auxiliary aids and services, or language assistance services, contact the Nondiscrimination Grievance Coordinator at the address, phone number, fax number, or email address below.

If *you* believe that Aspirus Health Plan, Inc. has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability, or sex, *you* can file a *grievance* with:

Nondiscrimination Grievance Coordinator
Aspirus Health Plan, Inc.
PO Box 1890
Southampton, PA 18966-9998
Phone: 1-866-631-5404 (TTY: 711)
Fax: 763-847-4010
Email: customerservice@aspirushealthplan.com

You can file a *grievance* in person or by mail, fax, or email. If *you* need help filing a *grievance*, the Nondiscrimination Grievance Coordinator is available to help *you*.

You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights, electronically through the Office for Civil Rights Complaint Portal, available at https://ocrportal.hhs.gov/ocr/portal/lobby.jsf, or by mail or phone at:

U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1.800.368.1019, 800.537.7697 (TDD)

Complaint forms are available at http://www.hhs.gov/ocr/office/file/index.html. This notice is available at Aspirus Health Plan, Inc.'s website: https://aspirushealthplan.com/webdocs/70021-AHP-NonDiscrim_Lang-Assist-Notice.pdf.

Language Assistance Services

Albanian: KUJDES: Nëse flitni shqip, për ju ka në dispozicion shërbime të asistencës gjuhësore, pa pagesë.Telefononi në 1-800-332-6501 (TTY: 711).

Arabic تنبيه: إذا كنت تتحدث اللغة العربية، فإن خدمات المساعدة اللغوية متاحة لك مجاناً. اتصل بن اعلى رقم الهاتف1-800-332-6501(رقم هاتف الصم والبك : 711)

French: ATTENTION: Si vous parlez français, des services d'aide linguistique vous sont proposés gratuitement. Appelez le 1-800-332-6501 (ATS: 711).

German: ACHTUNG: Wenn Sie Deutsch sprechen, stehen Ihnen kostenlos sprachliche Hilfsdienstleistungen zurVerfügung. Rufnummer: 1-800-332-6501 (TTY: 711).

Hindi: _यान द_: य_द आप ि हंदी बोलते ह_ तो आपके ि लए मु_त म_ भाषा सहायता सेवाएं उपल_ध ह_।1-800-332-6501 (TTY: 711) पर कॉल कर_।

Hmong: LUS CEEV: Yog tias koj hais lus Hmoob, cov kev pab txog lus, muaj kev pab dawb rau koj. Hu rau 1-800-332-6501 (TTY: 711).

Korean: 주의: 한국어를 사용하시는 경우, 언어 지원 서비스를 무료로 이용하실 수 있습니다.1-800-332-6501 (TTY: 711)번으로 전화해 주십시오.

Polish: UWAGA: Jeżeli mówisz po polsku, możesz skorzystać z bezpłatnej pomocy językowej. Zadzwoń pod numer1-800-332-6501 (TTY: 711).

Russian: ВНИМАНИЕ: Если вы говорите на русском языке, то вам доступны бесплатные услуги перевода.Звоните 1-800-332-6501 (телетайп: 711).

Spanish: ATENCIÓN: si habla español, tiene a su disposición servicios gratuitos de asistencia lingüística. Llame al1-800-332-6501 (TTY: 711).

Tagalog: PAUNAWA: Kung nagsasalita ka ng Tagalog, maaari kang gumamit ng mga serbisyo ng tulong sa wika nangwalang bayad. Tumawag sa 1-800-332-6501 (TTY: 711).

Traditional Chinese: 注意：如果您使用繁體中文， 您可以免費獲得語言援助服務。請 致電 1-800-332-6501 (TTY: 711)

Vietnamese: CHÚ Ý: Nếu bạn nói Tiếng Việt, có các dịch vụ hỗ trợ ngôn ngữ miễn phí dành cho bạn. Gọi số 1-800-332-6501 (TTY: 711).

Pennsylvania Dutch: Wann du Deitsch (Pennsylvania German / Dutch) schwetzscht, kannscht du mitaus Koschte ebbergricke, ass dihr helft mit die englisch Schprooch. Ruf selli Nummer uff: Call 1-800-332-6501 (TTY: 711).

Lao: ໂປດຊາບ: ຖ້າວ່າ ທ່ານເວົ້າພາສາ ລາວ, ການບໍລິການຊ່ວຍເຫຼືອດ້ານພາສາ,ໂດຍບໍ່ເສັງຄ່າ, ແມ່ນມີພ້ອມໃຫ້ທ່ານ. ໂທຣ 1-800-332-6501 (TTY: 711).