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PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Promacta (eltrombopag)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Promacta, approved by the FDA in 2008, is a small molecule thrombopoietin receptor agonist (TPO-RA) indicated for the treatment of persistent or chronic immune thrombocytopenia (ITP), for the treatment of thrombocytopenia in patients with chronic hepatitis C to allow the initiation and maintenance of interferon-based therapy, and for the treatment of severe aplastic anemia. It is important to take Promacta without a meal or with a meal low in calcium, and separated from any medication or product containing polyvalent cations. Promacta has a boxed warning for risk of hepatic decompensation in patients with chronic hepatitis C and risk of hepatotoxicity. Dose reductions are needed for patients with hepatic impairment and some patients of East-/SoutheastAsian ancestry.
ITP is a rare autoimmune disorder characterized by low levels of platelets due to platelet destruction and insufficient platelet production. Aplastic anemia (AA) is a bone marrow failure syndrome characterized by marrow hypoplasia and hematopoietic stem cell (HSC) deficiency. Most cases of AA are acquired rather than inherited. Acquired AA results from immune-mediated destruction of bone marrow. “Empty” bone marrow does not produce blood cells, causing pancytopenia.

Promacta (eltrombopag) will be considered for coverage when the following criteria are met:

Immune Thrombocytopenia (ITP)

For **initial** authorization:

1. Member is at least 1 year of age; AND
2. Medication is prescribed by or in consultation with a hematologist; AND
3. Member has a documented diagnosis of persistent or chronic ITP for at least 3 months; AND
4. Member meets one of the following:
   a) Current platelet count is $<30x10^9$/L
   b) $30x10^9$/L to $50x10^9$/L with one of the following:
      i) Active symptomatic bleeding other than minor mucocutaneous bleeding
      ii) High risk factor for bleeding (i.e., on an anticoagulant, of older age (>60 years), other clearly identified comorbidity; AND
5. Member had an inadequate response, intolerance, or contraindication to documented prior therapy with at least one of the following treatments:
   a) Corticosteroid
   b) Immunoglobulin
   c) Splenectomy; AND
6. Members 6 years of age and older requesting oral suspension must provide clinical rationale why tablets cannot be used; AND

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7. Member does NOT have any of the following:
   a) Thromboembolic condition
   b) Any cause of thrombocytopenia other than primary ITP
   c) Concurrent use with another TPO-RA or with Tavalisse.
   .
8. **Dosage allowed/Quantity limit:** Initiate at 50 mg once daily for most adult and pediatric patients 6 years and older, and at 25 mg once daily for pediatric patients aged 1 to 5 years. Adjust to maintain platelet count greater than or equal to 50 x 10^9 /L. Max dose 75 mg per day.
   QL: 30 tablets per 30 days or 30 packets per 30 days (oral suspension kit).

Note: Discontinue if the platelet count does not increase to a level sufficient to avoid clinically important bleeding after 4 weeks at the maximum dose.

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:
1. Chart notes have been provided that show improvement in platelet count from baseline; AND
2. Member's platelet count is less than 200 x 10^9/L or the dose is being reduced.

If all the above requirements are met, the medication will be approved for an additional 12 months.

Hepatitis C (HCV) Associated Thrombocytopenia

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication is prescribed by or in consultation with a hematologist, gastroenterologist, hepatologist, or infectious disease specialist; AND
3. Member has a documented diagnosis of thrombocytopenia associated with chronic hepatitis C; AND
4. Member's platelet count is less than 75 x 10^9/L; AND
5. Promacta is being prescribed for use with interferon (IFN)-based therapy for hepatitis C; AND
6. Members requesting oral suspension must provide clinical rationale why tablets cannot be used; AND
7. Member does NOT have any of the following:
   a) Decompensated liver disease (Child-Pugh score > 6, class B and C)
   b) History of ascites
   c) Hepatic encephalopathy.
8. **Dosage allowed/Quantity limit:** Initiate at a dose of 25 mg by mouth once daily. Adjust to achieve target platelet count required to start and maintain antiviral therapy. Max dose 100 mg daily. Discontinue when antiviral therapy is discontinued.
   QL: 60 tablets per 30 days.

If all the above requirements are met, the medication will be approved for 6 months.

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For **reauthorization**:
1. Chart notes have been provided that show the member has achieved and maintained a platelet count necessary to initiate and maintain antiviral therapy; AND
2. Member's platelet count is less than 200 x 10^9/L or the dose is being reduced; AND
3. Member is continuing IFN-based therapy as documented in chart notes and/or pharmacy claims.

If all the above requirements are met, the medication will be approved for an additional 6 months.

Severe Aplastic Anemia

For **initial** authorization:
1. Member is at least 2 years of age if using as part of 1st line therapy OR at least 18 years of age if refractory; AND
2. Medication is being prescribed by or in consultation with a hematologist; AND
3. Members 6 years of age and older requesting oral suspension must provide clinical rationale why tablets cannot be used; AND
4. Member has a documented diagnosis of severe aplastic anemia defined as a marrow cellularity < 25% (or 25–50% with <30% residual haematopoietic cells) plus at least 2 of the following:
   a) Neutrophils or ANC < 0.5 x 10^9/L (500/mm^3)
   b) Platelets < 20 x 10^9/L (20,000/mm^3)
   c) Reticulocyte count < 20 x 10^9/L (20,000/mm^3); AND
5. Member meets one of the following:
   a) 1st line therapy: Will be using Promacta in combination with immunosuppressive therapy, i.e., anti-thymocyte globulin (ATG) and cyclosporine
   b) Refractory disease: Member had an insufficient response to immunosuppressive therapy.
6. **Dosage allowed/Quantity limit:**
   Severe aplastic anemia first-line: Initial doses:

Age	Dose Regimen
Patients 12 years and older	150 mg once daily for 6 months
Pediatric patients 6 to 11 years	75 mg once daily for 6 months
Pediatric patients 2 to 5 years	2.5 mg/kg once daily for 6 months

   Refractory severe aplastic anemia: Initiate at a dose of 50 mg by mouth once daily, then adjust in 50 mg increment every 2 weeks as necessary to achieve target platelet count ≥ 50 x 109 /L. Max dose of 150 mg daily.
   QL: 60 tablets per 30 days or 30 packets per 30 days (oral suspension kit).

If all the above requirements are met, the medication will be approved for 6 months if using as first-line treatment; for 4 months for refractory patients. .

For **reauthorization**:
1. If continuing therapy for refractory disease, chart notes must show improvement from baseline with at least one of the following:
   a) Platelet response (increased platelet count)
   b) Neutrophil response (increased ANC)
   c) Erythroid response (increased hemoglobin)
   d) Transfusion independence; AND
2. Member's platelet count is less than 200 x 10^9/L or the dose is being reduced.

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If all the above requirements are met, the medication will be approved for an additional 6 months if the member has severe refractory aplastic anemia. Do not reauthorize if member was using as part of first-line therapy regimen.

HAP CareSource considers Promacta (eltrombopag) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/02/2018	New policy for Promacta created. Baseline liver enzymes levels requirement was removed. Four months of immunosuppressive therapy requirement for Severe Aplastic Anemia was removed. Platelets requirement threshold expanded
03/07/2019	Documented clinical reason required if request is for suspension for adult member.
02/13/2023	Transferred to new template. Updated and added references. Added quantity limits. **ITP**: Corrected "chronic immune (idiopathic) thrombocytopenic purpura (ITP)" to "persistent or chronic ITP for at least 3 months." Modified/simplified platelet count requirements. Added exclusions. Changed age for suspension to <6 years (as in clinical studies). Added note about discontinuation. Added "or dose is being reduced" to renewal criteria for not exceeding PC of >200. **Hep C**: Added GI and hepatology to specialists. Added that they must be starting IFN. For renewal, changed PC <400 to <200 or dose is being reduced, changed taking RBV or IFN to continuing IFN-based therapy, changed improved PC to PC necessary to initiate and maintain antiviral therapy. Changed auth durations from 3 mo to 6 mo. **AA**: Added criteria to accommodate 1st line use. Corrected age limit from 17 years to 2 years if using 1st line. Changed age for suspension to <6 years (as in clinical studies). Added "or 25–50% with <30% residual haematopoietic cells" to marrow cellularity criteria. Removed baseline PC of <30. Changed insufficient response to immunosuppressive therapy to insufficient response if disease is refractory or using in combination if 1st line. Added dosing info for 1st line use. Changed initial auth duration from 12 weeks to 6 months for 1st line use and 4 months for refractory. Changed renewal duration from 3 months to 6 months (for refractory disease). For renewal: changed PC <400 to <200 or dose is being reduced, changed platelet improvement to improvement in one of 3 parameters or transfusion independence.

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