Clinical Policy: Thalidomide (Thalomid)
Reference Number: PA.CP.PHAR.78
Effective Date: 09/11
Last Review Date: 04/19

Description
Thalomid, α-(N-phthalimido) glutarimide, is an immunomodulatory agent.

FDA Approved Indication(s)
Thalomid is indicated:

- For the treatment of patients with newly diagnosed multiple myeloma (MM) in combination with dexamethasone
- For the acute treatment of the cutaneous manifestations of moderate to severe erythema nodosum leprosum (ENL)
- As maintenance therapy for prevention and suppression of the cutaneous manifestations of ENL recurrence

Limitation of use: Thalomid is not indicated as monotherapy for such ENL treatment in the presence of moderate to severe neuritis.

Policy/Criteria
It is the policy of Pennsylvania Health and Wellness® that Thalomid is medically necessary when the following criteria are met:

I. Initial Approval Criteria
   A. Multiple Myeloma (must meet all):
      1. Diagnosis of multiple myeloma (MM);
      2. Prescribed by or in consultation with an oncologist or hematologist;
      3. Age ≥12 years;
      4. Prescribed in combination with dexamethasone;
      5. Request meets one of the following (a or b):
         a. Dose does not exceed 200 mg/day;
         b. Dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (prescriber must submit supporting evidence).
   Approval duration: 6 months

   B. Erythema Nodosum Leprosum (must meet all):
      1. Diagnosis of erythema nodosum leprosum (ENL);
      2. Prescribed by or in consultation with an infectious disease specialist, immunologist, or dermatologist;
      3. Age ≥12 years;
      4. Dose does not exceed 400 mg/day.
   Approval duration: 6 months

   C. Myeloproliferative Neoplasms (off-label) (must meet all):
Thalidomide

1. Diagnosis of myeloproliferative neoplasms (myelofibrosis) with associated anemia;
2. Prescribed by or in consultation with an oncologist or hematologist;
3. Age ≥12 years;
4. Member meets one of the following (a or b):
   a. Serum EPO ≥ 500 mU/mL;
   b. Serum EPO < 500 mU/mL, and no response or loss of response to erythropoietic stimulating agents;
5. Request meets one of the following (a or b):
   a. Dose does not exceed 400 mg/day;
   b. Dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (prescriber must submit supporting evidence).

Approval duration: 6 months

D. Castleman’s Disease (off-label) (must meet all):
1. Diagnosis of multicentric Castleman’s disease;
2. Prescribed by or in consultation with an oncologist or hematologist;
3. Age ≥12 years;
4. Prescribed as subsequent therapy with or without rituximab for disease that has progressed following treatment of relapsed/refractory or progressive disease;
5. Request meets one of the following (a or b):
   a. Dose does not exceed 400 mg/day;
   b. Dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (prescriber must submit supporting evidence).

Approval duration: 6 months

E. Kaposi Sarcoma (off-label) (must meet all):
1. Diagnosis of AIDS-related Kaposi Sarcoma;
2. Prescribed by or in consultation with an oncologist or immunologist;
3. Age ≥12 years;
4. Prescribed in combination with antiretroviral therapy;
5. Disease has progressed or not responded to doxorubicin and paclitaxel;
6. Request meets one of the following (a or b):
   a. Dose does not exceed 400 mg/day;
   b. Dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (prescriber must submit supporting evidence).

Approval duration: 6 months

F. Other diagnoses/indications (must meet all):
1. Refer to PA.CP.PMN.53

II. Continued Approval
A. All Indications in Section I (must meet all):
CLINICAL POLICY

Thalidomide

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care Policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy;
3. If request is for a dose increase, request meets one of the following (a or b):
   a. Dose does not exceed 400 mg/day;
   b. Dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (prescriber must submit supporting evidence).

Approval duration: 12 months

B. Other diagnoses/indications (1 or 2):
   1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy or the Continuity of Care Policy (PA.LTSS.PHAR.01) applies.
   Approval duration: Duration of request or 6 months (whichever is less); or
   2. Refer to PA.CP.PMN.53

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
ENL: erythema nodosum leprosum
FDA: Food and Drug Administration
MM: multiple myeloma
NCCN: National Comprehensive Cancer Network

Appendix B: Therapeutic Alternatives
This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Dosing Regimen</th>
<th>Dose Limit/Maximum Dose</th>
</tr>
</thead>
<tbody>
<tr>
<td>Doxorubicin</td>
<td>Kaposi Sarcoma: 20 mg/m² IV every 3 weeks</td>
<td>20 mg/m²/dose</td>
</tr>
<tr>
<td>Paclitaxel</td>
<td>Kaposi Sarcoma: 100 mg/m² IV every 2 weeks</td>
<td>100 mg/m²/dose</td>
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</tbody>
</table>

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.

Appendix C: Contraindications/Boxed Warnings
- Contraindication(s): pregnancy; hypersensitivity
- Boxed warning(s): embryo-fetal toxicity and venous thromboembolism

Appendix D: General Information
- Thalomid is only available under a restricted distribution program called the Thalomid REMS program due to a black box warning for embryo-fetal toxicity. Patient and physician enrollment in the manufacturer's REMS program is required.

IV. Dosage and Administration
**Indication**  | **Dosing Regimen**  | **Maximum Dose**
--- | --- | ---
MM | 200 mg PO QD | 200 mg/day
ENL | 100 to 300 mg PO QD | 400 mg/day

V. **Product Availability**
Capsules: 50 mg, 100 mg, 150 mg, 200 mg

VI. **References**

**Reviews, Revisions, and Approvals**

<table>
<thead>
<tr>
<th>Event Description</th>
<th>Date</th>
<th>Approval Date</th>
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<tbody>
<tr>
<td>2Q 2018 annual review: added prescriber and age requirements; removed off label indication for systemic light chain amyloidosis that is no longer included in NCCN Compendium; added off-label use for Kaposi Sarcoma; summarized NCCN and FDA approved uses for improved clarity; added specialist involvement in care; references reviewed and updated.</td>
<td>01.22 .18</td>
<td>04.18</td>
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<tr>
<td>2Q 2019 annual review: myeloproliferative neoplasms – removed requirement for use in combination with prednisone to align with NCCN compendium; removed Waldenstrom macroglobulinemia/lymphoplasmacytic lymphoma criteria set as this indication is no longer supported by NCCN compendium; expanded prescriber requirements for MM, MPN, Castleman’s disease; references reviewed and updated.</td>
<td>04/19</td>
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