Clinical Policy: Global Biopharm Criteria

Reference Number: PA.CP.PHAR.57
Effective Date: 1/18
Last Review Date: 08/17 Revision Log
Line of Business: Medicaid

Description
This policy is to be used to determine medical necessity of existing or newly approved specialty drug therapy where no custom coverage criteria are available, including requests for an indication, treatment regimen, or patient population not approved by the U.S. FDA (Food and Drug Administration).

Policy/Criteria
Provider must submit documentation (which may include office chart notes and lab results) supporting that member has met all approval criteria

It is the policy of **Pennsylvania Health and Wellness**that all medical necessity determinations for drug therapy without Centene® custom coverage criteria be considered on a case-by-case basis by a physician, pharmacist or ad hoc committee, using the guidance provided within this policy.

I. Approval Criteria

A. Labeled Use without Custom Criteria (must meet all):

1. The drug is prescribed for an FDA (Food and Drug Administration)- approved indication;
2. Requested dosage regimen and duration is within dosing guidelines recommended for the specific indication according to the product information label of the drug;
3. Failure of adequate trials of two formulary agents considered standard of care for the relevant diagnosis, when such agents exist, unless member experiences clinically significant adverse effect or has contraindication(s);
4. Member has no contraindications to prescribed agent per the product information label;
5. If applicable, prescriber has taken necessary measures to minimize any risk associated with a boxed warning in the product information label.

Approval duration: duration of request or 3 months (whichever is less)

B. Off-label Use (must meet all)**:**

1. Request meets one of the following (a or b):
   a. Use is supported by the National Comprehensive Cancer Network Drug Information and Biologics Compendium level of evidence 1 & 2a;
   b. The use is supported by evidence from two high quality published studies in peer-reviewed journals or evidence-based clinical practice guidelines that provide all of the following:
      i. Adequate representation of the member's clinical characteristics, age, and diagnosis;
      ii. Adequate representation of the prescribed drug regimen;
      iii. Clinically meaningful outcomes as a result of the drug therapy in question;

iv. Appropriate experimental design method to address research questions (see Appendix C for additional information);

2. Treatment is not for a cosmetic purpose;
3. Prescribed by or in consultation with an appropriate specialist for the diagnosis;
4. Failure of adequate trials of two FDA approved drug(s) that are considered the standard of care, when such agent exists, at maximum indicated doses, unless member experiences clinically significant adverse effect or has contraindication(s);
5. Requested dosage regimen and duration is within dosing guidelines recommended by clinical practice guidelines and/or medical literature.

Approval duration: duration of request or 3 months (whichever is less)

II. Continued Therapy

A. All requests (must meet all)**:**

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 applies (PA.LTSS.PHAR.02) ;
2. Documentation supports positive response to therapy (examples: sign/symptom reduction, no disease progression, slowed disease progression, no significant toxicity);
3. If request is for a dose increase, new dose does not exceed dosing guidelines recommended by clinical practice guidelines and/or medical literature.

Approval duration: duration of request or 6 months *(whichever is shorter)*

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Indications or diagnoses in which the drug has been shown to be unsafe or ineffective.

IV. Appendices/ General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration

Appendix B: General Information

- The U.S. FDA approves drugs for specific indications included in the drug's product information label. The approval by the FDA means that the company can include the information in their package insert. Omission of uses for a specific age group or a specific disorder from the approved label means that the evidence required by law to allow their inclusion in the label has not been submitted to the FDA. Off-label, or "unlabeled," drug use is the utilization of an FDA-approved drug for indications, treatment regimens, or populations other than those listed in the FDA-approved labeling. Many off-label uses are effective and well-documented in the peer-reviewed literature, and they are widely used even though the manufacturer has not pursued the additional

indications. Refer to the drug's FDA approved indication(s) and labeling (varies among drug products).

Appendix C: Appropriate Experimental Design Methods
Randomized, controlled trials are generally considered the gold standard; however:

- In some clinical studies, it may be unnecessary or not feasible to use randomization, double-blind trials, placebos, or crossover.
- Case reports are generally considered uncontrolled and anecdotal information and do not provide adequate supportive clinical evidence for determining accepted uses of drugs.
- Non-randomized clinical trials with a significant number of subjects may be a basis for supportive clinical evidence for determining accepted uses of drugs.

V. Dosage and Administration

Not applicable

VI. Product Availability

Not applicable

Reviews, Revisions, and Approvals	Date	Approval Date

VII. References

A. Food and Drug Administration. Good Reprint Practices for the Distribution of Medical Journal Articles and Medical or Scientific Reference Publications on Unapproved New Uses of Approved Drugs and Approved or Cleared Medical Devices. January 2009. Available at: http://www.fda.gov/RegulatoryInformation/Guidances/ucm125126.htm. Accessed July 14, 2017.