Clinical Policy: Off-Label Use

Reference Number: PA.CP.PMN.53
Effective Date: 01/18
Last Review Date: 11/16
Line of Business: Medicaid

Coding Implications
Revision Log

Description
Off-label drug use is the utilization of an FDA-approved drug for uses other than those listed in the FDA-approved labeling or in treatment regimens or populations that are not included in approved labeling.

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

It is the policy of Pennsylvania Health and Wellness® that the off-label use of a non-specialty drug is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

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

1. Submitted clinical practice guidelines and/or medical literature (Phase 3 or high quality, large, controlled research study published in reputable drug reference books or peer-reviewed journals) provide all of the following:
   a. Adequate representation of the member's clinical characteristics and diagnosis;
   b. Adequate representation of the prescribed drug regimen;
   c. Clinically meaningful outcomes as a result of the drug therapy in question;
   d. Appropriate experimental design method to address research questions (see Appendix C for additional information);
2. Failure of an adequate trial of an FDA-approved drug considered the standard of care or a PDL drug for the same indication at maximum indicated doses, unless no such drugs exist or member experiences clinically significant adverse effect or has contraindication(s) to all relevant FDA-approved drugs and PDL drugs;
3. Requested dosage regimen and duration is in within dosing guidelines recommended by clinical practice guidelines and/or medical literature.

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

II. Continued Therapy

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

1. Currently receiving medication via Pennsylvania Health and Wellness benefit, or member has previously met initial approval criteria or the Continuity of Care policy applies (PA.LTSS.PHAR.02) and documentation supports positive response to therapy;
2. 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 12 months (whichever is shorter)

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

N/A

IV. Appendices/General Information

Appendix A: Abbreviation Key
FDA: Food and Drug Administration

Appendix B: 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.
- Non-randomized clinical trials with a significant number of subjects may be a basis for supportive clinical evidence for determining accepted uses of drugs.

Case reports are generally considered uncontrolled and anecdotal information and do not provide adequate supportive clinical evidence for determining accepted uses of drugs.

V. Dosage and Administration

N/A

VI. Product Availability

N/A

VII. References

N/A

Reviews, Revisions, and Approvals	Date	Approval Date