MEDICAL POLICY

POLICY TITLE	ORPHAN DRUGS AND HUMANITARIAN USE DEVICE (HUD)
POLICY NUMBER	MP 2.383

CLINICAL BENEFIT	☒ MINIMIZE SAFETY RISK OR CONCERN. ☐ MINIMIZE HARMFUL OR INEFFECTIVE INTERVENTIONS. ☐ ASSURE APPROPRIATE LEVEL OF CARE. ☐ ASSURE APPROPRIATE DURATION OF SERVICE FOR INTERVENTIONS. ☐ ASSURE THAT RECOMMENDED MEDICAL PREREQUISITES HAVE BEEN MET. ☐ ASSURE APPROPRIATE SITE OF TREATMENT OR SERVICE.
Effective Date:	**7/1/2024**

POLICY | PRODUCT VARIATIONS | DESCRIPTION BACKGROUND
RATIONALE | DEFINITIONS | BENEFIT VARIATIONS
DISCLAIMER | CODING INFORMATION | REFERENCES
POLICY HISTORY

I. POLICY

This policy applies only when there is not another Medical Policy that delineates specific criteria for drug, biologic, device, or other intervention. If another policy does exist, then the criteria for medical necessity in that policy supersede the guidelines in this policy.

Orphan Drug Use
An orphan drug may be considered **medically necessary** when the drug:

- Has FDA Orphan Drug designation; **and**
- Has FDA marketing approval; **and**
- Is listed on the FDA website as a designated orphan product with marketing approval at https://www.accessdata.fda.gov/scripts/opdlisting/oopd/. **and**
- Is prescribed for a member who is a medically appropriate candidate **and**
- Use is consistent with FDA-approved indications **and**
- Use is consistent with peer-reviewed published medical literature or other appropriately recognized resources (e.g., NCCN, AHFS, Micromedex, ICER)

All other uses of a drug as an orphan drug product are considered **investigational**.

Humanitarian Use Device
A device may be considered **medically necessary** when:

- FDA has designated the device as a Humanitarian Use Devices (HUD); **and**
- Has FDA approved Humanitarian Device Exemption (HDE) and
- Use is consistent with FDA-approved indications; **and**
- is used at facilities that have Institutional Review Board (IRB) oversight and approval **and**
- is prescribed for a member who is a medically appropriate candidate **and**

- Use is consistent with peer-reviewed published medical literature or other appropriately recognized resources (e.g., NCCN, AHFS, Micromedex, ICER)

All other uses of a device as a HUD are considered **investigational**.

The National Comprehensive Cancer Network (NCCN) is a nonprofit alliance of cancer centers throughout the United States. NCCN develops the Clinical Practice Guidelines in Oncology which are recommendations aimed to help health care professionals diagnose, treat, and manage patients with cancer. The National Cancer Institute's PDQ (Physician Data Query) is NCI's comprehensive source of cancer information, which includes evidence-based summaries on topics that cover adult and pediatric cancer treatment. These guidelines evolve continuously as new treatments and diagnostics emerge and may be used by Capital BlueCross when determining medical necessity according to this policy.

Cross-reference:

MP 1.122 – Bronchial Valves
MP 2.010 – Clinical Trials
MP 2.103 - Off-Label Use of Medications and other Interventions
MP 2.373 - Step Therapy Treatment in Cancer, Including Treatments for Stage Four, Advanced Metastatic Cancer, and Severe Related Health Conditions

II. PRODUCT VARIATIONS TOP

This policy is only applicable to certain programs and products administered by Capital Blue Cross please see additional information below, and subject to benefit variations as discussed in Section VI below.

FEP PPO - Refer to FEP Medical Policy Manual. The FEP Medical Policy manual can be found at: https://www.fepblue.org/benefit-plans/medical-policies-and-utilization-management-guidelines/medical-policies .

III. DESCRIPTION/BACKGROUND TOP

Orphan Drugs

Orphan drug are drugs (includes biologics) for the prevention, diagnosis, or treatment of diseases or conditions affecting fewer than 200,000 persons in the United States or drugs that will not be profitable within 7 years following approval by the FDA. The term "orphan drug" can refer to either a drug or biologic intended for use in a rare disease or condition as defined by the FDA Office of Orphan Product Development Program Overview (OOPD). A drug or biologic becomes an "orphan drug" when it receives orphan-drug designation from the Office of Orphan Products Development at the FDA. Orphan drugs may be FDA approved for marketing or experimental. A list of FDA designated orphan products with marketing approval may be accessed at the following FDA website: https://www.accessdata.fda.gov/scripts/opdlisting/oopd/.

Unapproved or unlabeled uses of drugs include a variety of situations ranging from completely unstudied to thoroughly investigated drug uses where the FDA has not been asked for approval, whereas approved uses of drugs have been proved to be safe and effective by the FDA after the review of adequate and controlled clinical trials that have documented their uses.

Humanitarian Device Exemptions

As defined by the FDA, Humanitarian Use Device (HUD) is "a medical device intended to benefit patients in the treatment or diagnosis of a disease or condition that affects or is manifested in not more than 8,000 individuals in the United States per year". Humanitarian Device Exemption (HDE) is defined as "a marketing application for an HUD (Section 520(m) of the Federal Food, Drug, and Cosmetic Act (FD&C Act)). An HDE is exempt from the effectiveness requirements of Sections 514 and 515 of the FD&C Act and is subject to certain profit and use restrictions."

The FDA mentions that HDE approval is based upon the HUD will not expose patients to unreasonable or significant risk of illness or injury and the probable benefit to health from use of the device outweighs the risk of injury or illness for its use. Also, while taking into account the probable risks and benefits of currently available devices or alternate forms of treatment and other criteria. The key difference between HDE and premarket approval is HDE is exempt from demonstrating effectiveness.

IV. RATIONALE

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N/A

V. DEFINITIONS/BACKGROUND

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ORPHAN DRUG RARE DISEASE OR CONDITION refers to the FDA designation as any disease or condition which (a) affects less than 200,000 persons in the U.S. or (b) affects more than 200,000 persons in the U.S. but for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for such disease or condition will be recovered from sales in the U.S. of such drug.

Institutional Review Boards is under FDA regulations and is an appropriately constituted group that has been formally designated to review and monitor biomedical research involving human subjects. An IRB has the authority to approve, require modifications in (to secure approval), or disapprove research. IRB serves an important role in the protection of rights, safety, and welfare of human research subjects.

Humanitarian Use Device (HUD) is a "medical device intended to benefit patients in the treatment or diagnosis of a disease or condition that affects or is manifested in not more than 8,000 individuals in the United States per year."

VI. BENEFIT VARIATIONS

TOP

The existence of this medical policy does not mean that this service is a covered benefit under the member's health benefit plan. Benefit determinations should be based in all cases on the applicable health benefit plan language. Medical policies do not constitute a description of benefits. A member's health benefit plan governs which services are covered, which are excluded, which are subject to benefit limits, and which require preauthorization. There are different benefit plan designs in each product administered by Capital Blue Cross. Members and providers should consult the member's health benefit plan for information or contact Capital Blue Cross for benefit information.

VII. DISCLAIMER

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Capital Blue Cross' medical policies are developed to assist in administering a member's benefits, do not constitute medical advice and are subject to change. Treating providers are solely responsible for medical advice and treatment of members. Members should discuss any medical policy related to their coverage or condition with their provider and consult their benefit information to determine if the service is covered. If there is a discrepancy between this medical policy and a member's benefit information, the benefit information will govern. If a provider or a member has a question concerning the application of this medical policy to a specific member's plan of benefits, please contact Capital Blue Cross' Provider Services or Member Services. Capital Blue Cross considers the information contained in this medical policy to be proprietary and it may only be disseminated as permitted by law.

VIII. CODING INFORMATION

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Note: This list of codes may not be all-inclusive, and codes are subject to change at any time. The identification of a code in this section does not denote coverage as coverage is determined by the terms of member benefit information. In addition, not all covered services are eligible for separate reimbursement. The codes need to be in numerical order.

***Specific Procedure Coding does not apply to this policy**

IX. REFERENCES

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1. *U.S. Food and Drug Administration (FDA). Office of Orphan Products (OOPD) Overview. Updated 4/23/19. Accessed April 10, 2024..*
2. *U.S. Food and Drug Administration (FDA). Office of Orphan Products (OOPD) Designating an Orphan Product: Drugs and Biological Products. Accessed April 10, 2024..*
3. *U.S. Food and Drug Administration (FDA). Getting a Humanitarian Use Device to Market. Accessed April 10, 2024..*
4. *U.S. Food and Drug Administration (FDA). Humanitarian Device Exemption. Accessed April 10, 2024..*

5. *U.S. Food and Drug Administration (FDA). IDE Institutional Review Boards (IRB). Accessed April 10, 2024..*

X. POLICY HISTORY

TOP

MP 2.383	**08/26/2022 New Policy created.** Orphan drugs removed from MP 2.103 and Expanded Access removed from MP 2.010 and added to this policy.
	01/13/2023 Minor Review. Expanded Access Transferred to new policy MP 2.386. Criteria, background, and references updated
	04/10/2024 Consensus Review. No change to policy statement.

Top