MEDICAL POLICY

POLICY TITLE	EXPANDED ACCESS
POLICY NUMBER	MP 2.386

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:	**1/1/2025**

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

I. POLICY

Expanded Access for Non-Investigational Interventions

Expanded access to interventions that are approved, yet not typically preferred per medical policy may be covered when an agreement exists between a provider and Capital (or other Blue plan) and at least **ONE** of the following criteria is met:

1) The intervention meets the Capital medical policy requirements for necessity, including cost-effectiveness; **OR**
2) The agreement specifies criteria for coverage with evidence development.
   a) This may include, but does not require, enrollment by the member in a clinical trial.

Expanded Access for Investigational Interventions ("Compassionate Use")

The medical necessity and appropriateness for routine patient costs associated with an intervention authorized by the FDA as an investigational intervention, yet used outside of a clinical trial (compassionate use), will be reviewed on an individual basis, and must meet **all** the following criteria:

- The member has a serious or immediately life-threatening disease or condition **and**
- No comparable or satisfactory therapeutic alternatives are available; **and**
- The product manufacturer and the patient's doctor have made special arrangements to obtain the product for the patient; **and**
- These arrangements are documented and authorized by the FDA. **and**
- Use is consistent with FDA authorization and indications **and**
- Member benefit justifies the potential risks of treatment **and**
- Member enrollment in a clinical trial is not possible **and**
- Member is a medically appropriate candidate **and**

- Use is consistent with peer-reviewed published medical literature or other appropriately recognized resources

Routine patient costs related to expanded access to investigational interventions do not include any of the following:

- The investigational drug, biological product, device, medical treatment or procedure itself;
- Items and services that are not used in the direct clinical management of the patient;
- Any service that is clearly inconsistent with widely accepted and established standards of care for a particular diagnosis;
- Services and supplies provided by the sponsor;
- Member travel expenses.

Cross-reference:

MP 4.003 Medical Necessity
MP 2.383 Orphan Drugs, Humanitarian Use Device (HUD)
MP 2.010 Clinical Trials
MP 2.103 Off-Label Use of Medications and other Interventions

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

Expanded Access for Investigational Interventions
Per the FDA, expanded access, sometimes called "compassionate use," is the use outside of a clinical trial of an investigational medical product (i.e., one that has not been approved by Food and Drug Administration). The Food and Drug Administration (FDA) is committed to increasing awareness of and knowledge about its expanded access programs and the procedures for obtaining access to human investigational drugs (including biologics) and medical devices.

Wherever possible, use of an investigational medical product by a patient as part of a clinical trial is preferable. However, when patient enrollment in a clinical trial is not possible (e.g., that there are no ongoing trials, a patient may not have access to a clinical trial or may not be eligible for the clinical trials, distance to get to a trial prevents access), patients may be able to receive the product, when appropriate, through expanded access.

Investigational medical products have not yet been approved or cleared by FDA and FDA has not found these products to be safe and effective for their specific use. Furthermore, the investigational medical product may, or may not, be effective in the treatment of the condition, and use of the product may cause unexpected serious side effect.

Ensuring patient safety is a priority - FDA must determine that the potential patient benefit justifies the potential risk of the expanded access use of the investigational drug, and that the potential risk is not unreasonable in the context of the disease or condition to be treated. Even with safeguards, there may be significant unknowns about safety and effectiveness.

IV. RATIONALE

TOP

N/A

V. DEFINITIONS/BACKGROUND

TOP

EXPANDED ACCESS: Sometimes called “compassionate use”, expanded access is a potential pathway for a patient with an immediately life-threatening condition or serious disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

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

TOP

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

TOP

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

TOP

1. *U.S. Food and Drug Administration (FDA). IDE Institutional Review Boards (IRB). Accessed January 5, 2023*
2. *Food and drug administration. News and Events. Expanded Access (Compassionate Use). Accessed January 5, 2023*
3. *U.S. Food and Drug Administration (FDA). Expanded Access for Medical Devices. Accessed January 5, 2023*
4. *Electronic Code of Federal Regulations. Title 21 → Chapter I → Subchapter D → Part 312 → Subpart I. Expanded Access to Investigational Drugs for Treatment Use. Accessed January 5, 2023*

X. POLICY HISTORY

TOP

MP 2.386	**01/13/2023 New Policy** created. Moved Expanded access from MP 2.383 and added to policy. Updated criteria for expanded access.
	04/10/2024 Consensus Review
	11/20/2024 Administrative Update. Removed NCCN and other examples from policy statement. Intent unchanged.

Top