Oscar Clinical Guideline: Accelerated Approval Products (PG289, Ver. 1)

Accelerated Approval Products

Disclaimer

Clinical guidelines are developed and adopted to establish evidence-based clinical criteria for utilization management decisions. Clinical guidelines are applicable according to policy and plan type. The Plan may delegate utilization management decisions of certain services to third parties who may develop and adopt their own clinical criteria.

Coverage of services is subject to the terms, conditions, and limitations of a member's policy, as well as applicable state and federal law. Clinical guidelines are also subject to in-force criteria such as the Centers for Medicare & Medicaid Services (CMS) national coverage determination (NCD) or local coverage determination (LCD) for Medicare Advantage plans. Please refer to the member's policy documents (e.g., Certificate/Evidence of Coverage, Schedule of Benefits, Plan Formulary) or contact the Plan to confirm coverage.

Summary

The United States Food and Drug Administration (FDA) instituted its Accelerated Approval Program to allow for earlier approval of drugs, biologics, or gene therapies that treat a serious condition, and fill an unmet medical need based on a surrogate endpoint. The FDA Accelerated Approval Program permits earlier approval of drugs and biologics that treat serious or life-threatening conditions and address unmet medical needs, based on surrogate or intermediate clinical endpoints that are reasonably likely to predict clinical benefit.

A surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, or physical sign, that is thought to predict clinical benefit but is not itself a measure of clinical benefit. An intermediate clinical endpoint, such as measuring tumor shrinkage or physical milestones, is a therapeutic effect measure considered reasonably likely to predict the clinical benefit. However, these endpoints remain indirect indicators of clinical benefit, have not been validated in the real world, and as such, there is uncertainty regarding how well they predict clinically meaningful health outcomes.

Studies using surrogate or intermediate endpoints prioritize objective data points that may not reliably predict the true clinical outcome. The FDA requires the manufacturers of products that receive accelerated approval to conduct adequate and well-controlled studies to confirm the anticipated clinical benefit. If the confirmatory trial shows the product actually provides a clinical benefit, the FDA grants traditional approval. If the confirmatory trial does not show that the product provides clinical benefit, the FDA may remove the drug from the market.

This policy targets drugs, biologics, or gene therapies under Oscar Health's medical and pharmacy benefits that have been approved solely via the FDA-accelerated approval pathway without demonstrating clinical data that show effectiveness. The list of drugs, biologics, or gene therapies that are accelerated approvals can be found on the FDA website available at: fda.gov > Drugs Development & Approval Process | Drugs > How Drugs are Developed and Approved > Drug and Biologic Approval and IND Activity Reports > NDA and BLA Approvals > Accelerated Approvals. This is updated quarterly in January, April, July, and October of each year. Alternatively, products with accelerated approvals are noted in the package insert (package inserts can be found at: fda.gov > Drug Databases > Drugs@FDA) under the "Indications and Usage".

Definitions

"Accelerated Approval Product" refers to any drug or biologic granted FDA approval under the Accelerated Approval Program (e.g., 21 CFR 314.510), based on surrogate or intermediate clinical endpoints reasonably likely to predict clinical benefit.

"Compendia" are summaries of drug information and medical evidence to support decision-making about the appropriate use of drugs and medical procedures. Examples include, but are not limited to:

1. American Hospital Formulary Service Drug Information
2. Elsevier Clinical Pharmacology

3. National Comprehensive Cancer Network Drugs and Biologics Compendium
4. Thomson Micromedex DrugDex
5. United States Pharmacopeia-National Formulary (USP-NF)

"Documentation" refers to written information, including but not limited to:

- Up-to-date chart notes, relevant test results, and/or relevant imaging reports to support diagnoses; or
- Prescription claims records, and/or prescription receipts to support prior trials of formulary alternatives.

"FDA," or the Food and Drug Administration, is an agency of the United States federal government responsible for protecting and promoting public health through the regulation and supervision of food safety, tobacco products, dietary supplements, prescription and over-the-counter medications, vaccines, biopharmaceuticals, blood transfusions, medical devices, electromagnetic radiation emitting devices, cosmetics, and veterinary products. The FDA's main goal is to ensure that these products are safe and effective for their intended use, and that their labeling and marketing are truthful and not misleading.

"Intermediate Clinical Endpoint" refers to a clinical measure (often closer to irreversible morbidity or mortality than a surrogate marker) that is reasonably likely to predict the ultimate clinical outcome, but is not itself the final patient-centered outcome.

"No evidence of" indicates that the reviewer has not identified any records of the specified item or condition within the submitted materials or claims history. In the absence of such evidence, the member is considered eligible. If any evidence of the item or condition is present upon review of the request, the member does not qualify.

"Objective Endpoint"is a measurement that does not require subjective interpretation such as overall survival.

"Primary endpoint" is the main, predefined endpoint used to measure a product's effectiveness.

"Randomized, Controlled Trial or RCT" is a type of study design where patients are randomly assigned to either the experimental treatment or a control group to eliminate bias.

"Semi-objective" is a measurement that involves some subjective interpretation or observation, but relies on a standardized or structured scale such as tumor response rate.

"Statistically significant" is a mathematical finding indicating that the difference in results between study groups is unlikely to have occurred by random chance.

"Surrogate Endpoint" is a marker such as a laboratory measurement, radiographic image, physical sign, or other test result that is used in clinical trials as a substitute for a direct measure of how a patient feels, functions, or survives; the surrogate is reasonably likely to predict clinical benefit but does not itself measure that benefit.

"Validated endpoint" is an outcome measure that has been scientifically proven in prior research to accurately reflect a patient's true clinical state or prediction of a health outcome.

"[s]" indicates state mandates may apply.

Clinical Indications

Medical Necessity Criteria for Initial Clinical Review

Accelerated Approval Products

The Plan considers accelerated approval products medically necessary when ALL of the following criteria are met:

1. The indication is supported, or at least reasonably acknowledged, by authoritative compendia or guidelines (e.g., NCCN Category 1–2B for oncology, or equivalent endorsement for non-oncology conditions), with clear specification of population and line of therapy*; AND*
2. The member meets all components of the FDA-labeled indication including age, diagnosis, stage, severity, biomarker or genetic testing requirements, and/or treatment setting as applicable, unless the Plan explicitly defines more restrictive or broader criteria based on evidence and clinical consensus*; AND*
3. There is no clinically appropriate alternative(s)[‡] with demonstrated patient-centered benefit that should reasonably be used before the accelerated approval product[s]*; AND*
   [‡]*Clinically appropriate alternatives may include products being used off-label when such use is supported by high-quality peer-reviewed evidence or compendia recommendations.*
4. The request must include the following, as applicable:
   a. Diagnosis and staging information, including relevant clinical details*; and/or*
   b. Documentation of biomarker or diagnostic testing results required by the FDA label or guideline recommendations*; and/or*
   c. History of prior therapies, responses, intolerance, and rationale for using the accelerated approval product*; and/or*
   d. Proposed treatment plan, including dose, schedule, route of administration, and anticipated duration, align with labeling and/or guidelines.

If the above prior authorization criteria are met, the requested product will be authorized for up to 6-months.[s]

Coverage Limitation[s]:
While the product has received FDA accelerated approval, accelerated approval alone does not establish medical necessity under this guideline. If the member does NOT meet the above criteria, the available clinical evidence is insufficient to demonstrate clinically meaningful benefit for the requested indication.

Continued Care

Medical Necessity Criteria for Subsequent Clinical Review

Accelerated Approval Products

The Plan considers accelerated approval products medically necessary when ALL of the following criteria are met:

1. The member meets the above applicable Initial Clinical Review; *AND*
2. The member has documentation experiencing ONE of the following with the requested product:
    a. Disease improvement*; or*
    b. Disease stability*; or*
    c. Delayed disease progression; *AND*
3. There is no evidence of unacceptable toxicity or adverse reactions to the requested product*; AND*
4. The requested product's proposed treatment plan, including dose, schedule, route of administration, and anticipated duration, align with labeling and/or guidelines.

If the above reauthorization criteria are met, the requested product will be authorized for up to 6-months.[s]

Experimental / Investigational, or unproven[s]

Accelerated Approval Products for any other use beyond the FDA-approved indication is considered experimental, investigational, or unproven. The Plan will continue to monitor emerging evidence, including results from ongoing clinical trials, and will reassess its position as new data become available.

Not Medically Necessary[s]

The Plan may determine that certain accelerated approval products or indications are not medically necessary when:

- The surrogate or intermediate endpoint has weak or uncertain correlation with meaningful clinical outcomes.
- Confirmatory trials have failed to demonstrate clinically relevant benefit, have identified new safety concerns, or have been significantly delayed beyond reasonable timelines, especially when the FDA has signaled concern or initiated actions regarding the indication.

References

1. Christensen R, Ciani O, Manyara AM, Taylor RS. Surrogate endpoints: a key concept in clinical epidemiology. J Clin Epidemiol. 2024 Mar;167:111242.

2. Code of Federal Regulations. Title 21, Chapter 1. Subchapter D Part 314 Subpart H--Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses. 10/6/2016. Available at: eCFR :: 21 CFR Part 314 Subpart H -- Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses. Accessed April 7, 2026.
3. Mittal A, Kim MS, Dunn S, Wright K, Gyawali B. Frequently asked questions on surrogate endpoints in oncology-opportunities, pitfalls, and the way forward. EClinicalMedicine. 2024 Sep 13;76:102824.
4. US Food & Drug Administration. Accelerated Approvals. Available at: https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approvals. Accessed April 7, 2026.
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6. US Food & Drug Administration. Development & Approval Process | Drugs. Available at: https://www.fda.gov/drugs/development-approval-process-drugs. Accessed April 7, 2026.
7. US Food & Drug Administration. Food and drug administration safety and innovation act. 03/28/2018. Available at: Food and Drug Administration Safety and Innovation Act (FDASIA) | FDA. Accessed April 7, 2026.

Clinical Guideline Revision / History Information

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