

ZOKINVY (lonafarnib)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met, and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Zokinvy is a farnesyltransferase inhibitor indicated in patients 12 months of age and older with a body surface area of 0.39 m² and above:

1. To reduce risk of mortality in Hutchinson-Gilford Progeria Syndrome
2. For treatment of processing deficient Progeroid Laminopathies with heterozygous *LMNA* mutation with progerin-like protein accumulation
3. For treatment of processing deficient Progeroid Laminopathies with homozygous or compound heterozygous *ZMPSTE24* mutations

Limitations of Use:

Not indicated for other Progeroid Syndromes or processing-proficient Progeroid Laminopathies. Based upon its mechanism of action, Zokinvy would not be expected to be effective in these populations.

All other indications are considered experimental/investigational and not medically necessary.

II. CRITERIA FOR INITIAL APPROVAL

1. The member is 12 months of age or older
2. The member has a body surface area of 0.39 m² or above
3. Zokinvy is prescribed by or in consultation with a specialist in progeria, genetics, and/or metabolic disorders
4. One of the following have been confirmed by genetic testing and the genetic testing is provided with the request
 - a) The diagnosis of Hutchinson-Gilford Progeria Syndrome has been confirmed with genetic testing indicating the patient has *LMNA* mutation.
 - b) The diagnosis of Processing Deficient Progeroid Laminopathy with Progerin-Like Protein Accumulation has been confirmed with genetic testing indicating the patient has heterozygous *LMNA* mutation.
 - c) The diagnosis of Processing Deficient Progeroid Laminopathy without Progerin-Like Protein Accumulation has been confirmed with genetic testing indicating the patient has homozygous or compound heterozygous *ZMPSTE24* mutations.
5. The member does not have overt renal, hepatic, or pulmonary disease or immune dysfunction
6. The requested dose does not exceed the FDA's recommended dosing regimen (115mg/m² twice daily and then increased to 150mg/m² twice a day after 4 months of therapy).
7. The member is not taking strong or moderate CYP3A inhibitors or inducers (examples: midazolam, lovastatin, simvastatin, atorvastatin, etc.)

III. CONTINUATION OF THERAPY

1. All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria
2. All members (including new members) are tolerating therapy
3. All members (including new members) have experienced a positive response to therapy as determined by the prescribing physician. Examples of positive response to therapy include but are not limited to no new or worsening heart failure, no stroke incidence, or reduction in seizures

IV. APPROVAL DURATION

Initial: 6 months

Renewal: 6 months

V. QUANTITY LIMITS

- i. Zokinvy 50mg 120 capsules per 30 days
- ii. Zokinvy 75mg 120 capsules per 30 days

VI. REFERENCES

1. Zokinvy [package insert]. Palo Alto, CA: Eiger BioPharmaceuticals, Inc.; July 2025. Accessed May 2026.
2. Progeria Research Foundation (PRF). The Progeria Handbook: A Guide for Families & Health Care Providers of Children with Progeria. Second Edition. PRF. https://www.progeriaresearch.org/wp-content/uploads/2019/03/PRF_Handbook_2019_eFile.pdf. Accessed November 27, 2020.
3. Gordon LB, Brown WT, Collins FS. Hutchinson-Gilford Progeria Syndrome. *GeneReviews*. University of Washington, Seattle; 2019.
4. AHFS DI (Adult and Pediatric) [database online]. Hudson, OH: Lexi-Comp, Inc.; http://online.lexi.com/lco/action/index/dataset/complete_ashp [available with subscription]. Accessed March 15, 2021.
5. Micromedex Solutions [database online]. Ann Arbor, MI: Truven Health Analytics Inc. Updated periodically. www.micromedexsolutions.com [available with subscription]. Accessed March 15, 2021.