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5.99.020

Section:	Prescription Drugs	**Effective Date:**	April 1, 2025
Subsection:	Miscellaneous Products	**Original Policy Date:**	December 18, 2020
Subject:	Zokinvy	**Page:**	1 of 4

Last Review Date: March 7, 2025

Zokinvy

Description

Zokinvy (lonafarnib)

Background
Zokinvy (lonafarnib) inhibits farnesyltransferase to prevent farnesylation and subsequent accumulation of progerin and progerin-like proteins in the inner nuclear membrane (1).

Regulatory Status
FDA-approved indications: Zokinvy is indicated in patients 12 months of age and older with a body surface area (BSA) of 0.39 m^2 and above: (1)

- To reduce the risk of mortality in Hutchinson-Gilford Progeria Syndrome (HGPS)
- For the treatment of processing-deficient Progeroid Laminopathies with either:
  - Heterozygous *LMNA* mutation with progerin-like protein accumulation
  - Homozygous or compound heterozygous *ZMPSTE24* mutations

Limitations of Use: Zokinvy is 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 (1).

Zokinvy is contraindicated in patients taking strong or moderate CYP3A inhibitors or inducers, midazolam, lovastatin, simvastatin, or atorvastatin (1).

Some patients treated with Zokinvy developed laboratory abnormalities such as: hyperkalemia, hypokalemia, hyponatremia, hypercalcemia, myelosuppression, or increased liver enzymes. Electrolytes, complete blood counts, and liver enzymes should be monitored periodically, and any abnormalities managed accordingly (1).

Zokinvy may also cause nephrotoxicity or retinal toxicity. Renal function should be monitored at regular intervals during therapy. Ophthalmological evaluations should be performed at regular intervals and at the onset of any new visual changes during therapy (1).

Zokinvy may cause embryo-fetal harm when administered to a pregnant woman. Pregnant women should be advised of the risk to a fetus. Females of reproductive potential should be advised to use appropriate effective contraception during treatment with Zokinvy (1).

The safety and effectiveness of Zokinvy in pediatric patients less than 12 months of age have not been established (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Zokinvy may be considered **medically necessary** if the conditions indicated below are met.

Zokinvy may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 12 months of age or older

Diagnoses

Patient must have **ONE** of the following:

1. Hutchinson-Gilford Progeria Syndrome (HGPS)
2. Processing-deficient Progeroid Laminopathies with **ONE** of the following:
    a. Heterozygous *LMNA* mutation with progerin-like protein accumulation
    b. Homozygous or compound heterozygous *ZMPSTE24* mutations

AND ALL of the following:

a. Body surface area (BSA) ≥ 0.39 m^2
b. Prescriber agrees to monitor **ALL** of the following:
   - Electrolytes
   - Complete blood counts (CBC)
   - Liver function tests (LFTs)
   - Renal function
   - Ophthalmological evaluations
c. Females of reproductive potential **only:** patient will be advised to use appropriate effective contraception during treatment with Zokinvy

Prior – Approval *Renewal* Requirements

Same as above

Policy Guidelines

Pre – PA Allowance

None

Prior - Approval Limits

Quantity 360 capsules per 90 days

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

Zokinvy (lonafarnib) inhibits farnesyltransferase to prevent farnesylation and subsequent accumulation of progerin and progerin-like proteins in the inner nuclear membrane. The safety and effectiveness of Zokinvy in pediatric patients less than 12 months of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Zokinvy while maintaining optimal therapeutic outcomes.

References

1. Zokinvy [package insert]. Palo Alto, CA: Eiger BioPharmaceuticals, Inc.; March 2024.

Policy History

Date	Action
December 2020	Addition to PA
March 2021	Annual review
December 2022	Annual review. Changed policy number to 5.99.020
December 2023	Annual review
March 2024	Annual review
March 2025	Annual review and reference update

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.