



ZOKINVY (lonafarnib)

RATIONALE FOR INCLUSION IN PA PROGRAM

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 m² 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).



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

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.