

Pharmacy Policy Bulletin

Title: Lonafarnib (Zokinvy™)

Policy #: Rx.01.247

Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.

This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.

Members are advised to use participating pharmacies in order to receive the highest level of benefits.

Intent:

The intent of this policy is to communicate the medical necessity criteria for Lonafarnib (Zokinvy™) as provided under the member's prescription drug benefit.

Description:

Hutchinson-Gilford Progeria Syndrome is sporadic, very rare, autosomal dominant, deadly childhood disorder. It is one of the progeroid syndromes also known as Hutchinson-Gilford progeria syndrome (HGPS). Aging is a developmental process that begins with fertilization and ends up with death involving a lot of environmental and genetic factors. The disease firstly involves premature aging and then death from complications of atherosclerosis such as myocardial infarction, stroke, atherosclerosis, or heart failure.

Progeroid laminopathies are characterized by the premature appearance of certain signs of physiological aging in a subset of tissues. They are caused by mutations in genes coding for A-type lamins or lamin-binding proteins.

Lonafarnib inhibits farnesyltransferase to prevent farnesylation and subsequent accumulation of progerin and progerin-like proteins in the inner nuclear membrane.

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

- 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

Policy:

INITIAL CRITERIA Lonafarnib (Zokinvy™) is approved when ALL of the following are met:

1. One of the following:
 - a. Diagnosis of Hutchinson-Gilford Progeria Syndrome; or
 - b. For treatment of processing-deficient Progeroid Laminopathies with one of the following:
 - i. Heterozygous LMNA mutation with progerin-like protein accumulation; or
 - ii. Homozygous or compound heterozygous ZMPSTE24 mutations; and
2. Member is 12 months of age or older; and
3. Member has a body surface area of 0.39 m² and above

Initial authorization: 2 years

REAUTHORIZATION CRITERIA Lonafarnib (Zokinvy™) is re-approved when there is documentation of positive clinical response

Reauthorization: 2 years

Black Box Warning as shown in the drug Prescribing Information:

None

Guidelines:

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

BENEFIT APPLICATION

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

References:

Ahmed MS, Ikram S, Bibi N, Mir A. Hutchinson-Gilford Progeria Syndrome: A Premature Aging Disease. Mol Neurobiol. 2018 May;55(5):4417-4427. doi: 10.1007/s12035-017-0610-7. Epub 2017 Jun 28. PMID: 28660486. Accessed July 19, 2021.

Marcelot A, Worman HJ, Zinn-Justin S. Protein structural and mechanistic basis of progeroid laminopathies. FEBS J. 2021 May;288(9):2757-2772. doi: 10.1111/febs.15526. Epub 2020 Sep 3. PMID: 32799420. Accessed July 19, 2021.

ZOKINVY (lonafarnib) [package insert]. Alto, CA; Eiger BioPharmaceuticals, Inc.; Revised November 2020. Available from: https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/213969s000lbl.pdf Accessed July 19, 2021.

Applicable Drugs:

Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name

Zokinvy™

Generic Name

Lonafarnib

Cross References:

Rx.01.33 Off Label Use

Policy Version Number:	1.00
P&T Approval Date:	June 10, 2021
Policy Effective Date:	October 01, 2021
Next Required Review Date:	June 10, 2022

The Policy Bulletins on this web site were developed to assist the Company in administering the provisions of the respective benefit programs, and do not constitute a contract. If you have coverage through the Company, please refer to your specific benefit program for the terms, conditions, limitations and exclusions of your coverage. Company does not provide health care services, medical advice or treatment, or guarantee the outcome or results of any medical services/treatments. The facility and professional providers are responsible for providing medical advice and treatment. Facility and professional providers are independent contractors and are not employees or agents of the Company. If you have a specific medical condition, please consult with your doctor. The Company reserves the right at any time to change or update its Policy Bulletins.

