CVS Caremark®

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| Reference number(s) |
| 4384-A |

# Specialty Guideline Management Zokinvy

## Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
| --- | --- |
| Zokinvy | lonafarnib |

## 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 Indications1

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 risk of mortality in Hutchinson-Gilford Progeria Syndrome (HGPS)
* For the treatment of processing-deficient Progeroid Laminopathies with heterozygous LMNA mutation with progerin-like protein accumulation
* For the treatment of processing-deficient Progeroid Laminopathies with 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.

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

## Documentation

### Hutchinson-Gilford Progeria Syndrome

Submission of the following information is necessary to initiate the prior authorization review for Hutchinson-Gilford Progeria Syndrome: genetic testing results confirming the member has a LMNA pathogenic variant.

### Processing-Deficient Progeroid Laminopathy with Progerin-Like Protein Accumulation

Submission of the following information is necessary to initiate the prior authorization review for Processing-Deficient Progeroid Laminopathy with Progerin-Like Protein Accumulation: genetic testing results confirming the member has a heterozygous LMNA pathogenic variant.

### Processing-Deficient Progeroid Laminopathy without Progerin-Like Protein Accumulation

Submission of the following information is necessary to initiate the prior authorization review for Processing-Deficient Progeroid Laminopathy without Progerin-Like Protein Accumulation: genetic testing results confirming the member has either homozygous or compound heterozygous ZMPSTE24 pathogenic variant(s).

## Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

## Coverage Criteria

### Hutchinson-Gilford Progeria Syndrome1-3

Authorization of 12 months may be granted for treatment of Hutchinson-Gilford Progeria Syndrome when all of the following criteria are met:

* The member is 12 months of age or older.
* The member has a body surface area of 0.39 m2 or above.
* The diagnosis of Hutchinson-Gilford Progeria Syndrome has been confirmed with genetic testing indicating the member has an LMNA pathogenic variant.

### Processing-Deficient Progeroid Laminopathy with Progerin-Like Protein Accumulation1-3

Authorization of 12 months may be granted for treatment of Processing-Deficient Progeroid Laminopathy with Progerin-Like Protein Accumulation when all of the following criteria are met:

* The member is 12 months of age or older.
* The member has a body surface area of 0.39 m2 or above.
* The diagnosis of Processing-Deficient Progeroid Laminopathy has been confirmed with genetic testing indicating the member has a heterozygous LMNA pathogenic variant.

### Processing-Deficient Progeroid Laminopathy without Progerin-Like Protein Accumulation1-3

Authorization of 12 months may be granted for treatment of Processing-Deficient Progeroid Laminopathy without Progerin-Like Protein Accumulation when all of the following criteria are met:

* The member is 12 months of age or older.
* The member has a body surface area of 0.39 m2 or above.
* The diagnosis of Processing-Deficient Progeroid Laminopathy has been confirmed with genetic testing indicating the member has homozygous or compound heterozygous ZMPSTE24 pathogenic variant(s).

## Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed the coverage criteria when all of the following criteria are met:

* Member meets all initial authorization criteria.
* Member is experiencing benefit from therapy.

## References

1. Zokinvy [package insert]. Palo Alto, CA: Eiger BioPharmaceuticals, Inc.; March 2024.
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 February 20, 2025.
3. Gordon LB, Brown WT, Collins FS. Hutchinson-Gilford Progeria Syndrome. 2003 Dec 12 [Updated 2023 Oct 19]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1121/.