Reference number(s)
4384-A

SPECIALTY GUIDELINE MANAGEMENT

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 indicated in patients 12 months of age and older with a body surface area (BSA) of 0.39 m^2 and above:

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

II. DOCUMENTATION

A. 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* mutation.

B. 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* mutation.

C. 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* mutations.

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III. CRITERIA FOR INITIAL APPROVAL

A. Hutchinson-Gilford Progeria Syndrome
Authorization of 12 months may be granted for treatment of Hutchinson-Gilford Progeria Syndrome when all of the following criteria are met:
1. The member is 12 months of age or older.
2. The member has a body surface area of 0.39 m^2 or above.
3. The diagnosis of Hutchinson-Gilford Progeria Syndrome has been confirmed with genetic testing indicating the member has a *LMNA* mutation.

B. Processing-Deficient Progeroid Laminopathy with Progerin-Like Protein Accumulation
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:
1. The member is 12 months of age or older.
2. The member has a body surface area of 0.39 m^2 or above.
3. The diagnosis of Processing-Deficient Progeroid Laminopathy has been confirmed with genetic testing indicating the member has a heterozygous *LMNA* mutation.

C. Processing-Deficient Progeroid Laminopathy without Progerin-Like Protein Accumulation
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:
1. The member is 12 months of age or older.
2. The member has a body surface area of 0.39 m^2 or above.
3. The diagnosis of Processing-Deficient Progeroid Laminopathy has been confirmed with genetic testing indicating the member has homozygous or compound heterozygous *ZMPSTE24* mutations.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III when all of the following criteria are met:
A. Member meets all initial authorization criteria.
B. Member is experiencing benefit from therapy.

V. REFERENCES

1. Zokinvy [package insert]. Palo Alto, CA: Eiger BioPharmaceuticals, Inc.; November 2020.
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. 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/

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