

## Clinical Policy: Omaveloxolone (Skyclarys)

Reference Number: PA.CP.PHAR.590

Effective Date: 06/2023

Last Review Date: 04/2024

### Description

Omaveloxolone (Skyclarys™) is a nuclear factor erythroid 2–related factor 2 (Nrf2) pathway activator.

### FDA Approved Indication(s)

Skyclarys is indicated for the treatment of Friedreich’s ataxia (FA) in adults and adolescents aged 16 years and older.

### Policy/Criteria

*Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.*

It is the policy of PA Health & Wellness® that Skyclarys is **medically necessary** when the following criteria are met:

#### I. Initial Approval Criteria

##### A. Friedreich’s Ataxia (must meet all):

1. Diagnosis of FA;
2. Documentation of genetic testing that shows a GAA triplet-repeat expansion in the frataxin (FXN) gene;;
3. Prescribed by or in consultation with a neurologist;
4. Age  $\geq$  16 years;
5. Dose does not exceed both of the following (a and b):
  - a. 150 mg per day;
  - b. 3 capsules per day.

**Approval duration: 6 months**

##### B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

#### II. Continued Therapy

##### A. Friedreich’s Ataxia (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy as evidenced by, including but not limited to, improvement or stabilization in any of the following parameters: FA symptoms (see Appendix D) or mFARS score;
3. Dose does not exceed both of the following (a and b):
  - a. 150 mg per day;

- b. 3 capsules per day.

**Approval duration: 12 months**

**B. Other diagnoses/indications (must meet 1 or 2):**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.LTSS.PHAR.01) applies.

**Approval duration: Duration of request or 12 months (whichever is less); or**

2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

**III. Diagnoses/Indications for which coverage is NOT authorized:**

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

**IV. Appendices/General Information**

*Appendix A: Abbreviation/Acronym Key*

FA: Friedreich’s ataxia

FDA: Food and Drug Administration

FXN: frataxin

mFARS: modified functional assessment rating scale

Nrf2: nuclear factor erythroid 2–related factor 2

*Appendix B: Therapeutic Alternatives*

Not applicable

*Appendix C: Contraindications/Boxed Warnings*

None reported

*Appendix D: General Information*

- FA is a progressive, life-shortening ataxia which has cardinal symptoms of progressive gait and limb ataxia, lower limb areflexia, extensor plantar responses and dysarthria. In addition to ataxia, FA may cause fatigue, cardiomyopathy, and metabolic disturbances.
- The mFARS is a disease specific, exam-based neurological rating scale which includes assessment of bulbar function, upper limb coordination, lower limb coordination, and upright stability. The mFARS has a maximum cumulative value of 93 points, where higher cumulative scores signify greater degree of disability. The rating scale is provided below:

Neurologic assessment type (maximum points)	Description (points)
Bulbar (5)	Cough (2) Speech (3)
Upper limb coordination (36)	Finger-finger (3+3)

Neurologic assessment type (maximum points)	Description (points)
	Nose-finger (4+4) Dysmetria (4+4) Rapid movement (3+3) Finger taps (4+4)
Lower limb coordination (16)	Heel-shin slide (4+4) Heel-shin tap (4+4)
Upright stability (36)	Sitting position (4) Stance feet apart (4) Stance feet apart with eyes closed (4) Stance feet together (4) Stance feet together with eyes closed (4) Tandem stance (4) Stance dominant foot (4) Tandem walk (3) Gait (5)

**V. Dosage and Administration**

Indication	Dosing Regimen	Maximum Dose
FA	150 mg PO QD	150 mg/day

**VI. Product Availability**

Capsule: 50 mg

**VII. References**

1. Skyclarys Prescribing Information. Plano, TX: Reata Pharmaceuticals, Inc.; January 2024. Available at: <https://hcp.skyclarys.com>. Accessed February 5, 2024.
2. Lynch DR, Chin MP, Delatycki MB, et al. Safety and efficacy of omaveloxolone in Friedreich ataxia (MOXIe Study). *Ann Neurol*. 2021;89(2):212-225. <https://doi.org/10.1002/ana.25934>
3. Lynch DR, Farmer J, Hauser L, et al. Safety, pharmacodynamics, and potential benefit of omaveloxolone in Friedreich ataxia. *Ann Clin Transl Neurol*. 2018;6(1):15-26. Published 2018 Nov 10. <https://doi.org/10.1002/acn3.660>
4. Corben LA, Lynch D, Pandolfo M, et al. Consensus clinical management guidelines for Friedreich ataxia. *Orphanet J Rare Dis*. 2014; 9: 184. <https://doi.org/10.1186/s13023-014-0184-7>
5. Corben LA, Collins V, Milne S, et al. Clinical management guidelines for Friedreich ataxia: best practice in rare diseases. *Orphanet J Rare Dis*. 2022;17(1):415. doi: 10.1186/s13023-022-02568-3
6. Rummey C, Corben LA, Delatycki MB, et al. Psychometric properties of the Friedreich ataxia rating scale. *Neurol Genet* 2019;5e371. <https://doi.org/10.1212/NXG.0000000000000371>

Reviews, Revisions, and Approvals	Date
Policy created	05/2023

Reviews, Revisions, and Approvals	Date
4Q 2023 annual review: no significant changes; references reviewed and updated.	10/2023
For genetic testing, added that it shows a GAA triplet-repeat expansion in the FXN gene; removed requirement that member has ability to swallow capsules; for continued therapy, revised language from “improvement in any of the following parameters” to “improvement or stabilization in any of the following parameters” and changed approval duration from 6 months to 12 months.	01/2024
2Q 2024 annual review: no significant changes; references reviewed and updated.	04/2024