

Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy



Clinical Policy: Vatiquinone (PTC743)

Reference Number: CP.PHAR.729

Effective Date: **FDA Approval Date**

Last Review Date: 05.25

Line of Business: Commercial, HIM, Medicaid

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

Vatiquinone (PTC743^{®/™}) is a selective inhibitor of 15-lipoxygenase (15-LO).

FDA Approved Indication(s) [Pending]

PTC is indicated for the treatment of Friedreich's ataxia (FA).

Limitation(s) of use: [XXX]

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 health plans affiliated with Centene Corporation[®] that PTC743 is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

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 7 years;*
5. Recent (within the last 30 days) baseline modified Functional Assessment Rating Scale (mFARS) score (see *Appendix D*); *
6. Recent (within the last 30 days) baseline ventricular ejection fraction \geq 50%;*
7. PTC743 is not prescribed concurrently with Skylarys[®];*
8. Dose does not exceed 1,200 mg per day.*

Approval duration: 6 months

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

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):

- a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Friedreich's Ataxia (must meet all):

1. Member meets one of the following (a or b):
 - a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
 - b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B*);
2. Member is responding positively to therapy as evidenced by, including but not limited to, improvement in any of the following parameters: FA symptoms (see *Appendix D*) or mFARS score; *
3. If request is for a dose increase, new dose does not exceed 1,200 mg per day.*

Approval duration: 12 months

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

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

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 – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

15-LO: 15-lipoxygenase

FA: Friedreich’s ataxia

FDA: Food and Drug Administration

FXN: frataxin

mFARs; modified functional assessment rating scale

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

- Contraindication(s): **pending**
- Boxed warning(s): **pending**

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) 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)

Neurologic assessment type (maximum points)	Description (points)
	Tandem walk (3) Gait (5)

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
FA	<ul style="list-style-type: none"> Age < 12 years and weight < 25 kg: 200 mg PO TID* Age ≥ 12 years and/or weight ≥ 25 kg: 400 mg PO TID* 	1,200 mg/day*

VI. Product Availability [Pending]

Capsule: **Pending**

VII. References

1. Clinicaltrials.gov. A study to assess the efficacy and safety of Vatiquinone for the treatment of participants with Friedreich ataxia (MOVE-FA). Available at: <https://clinicaltrials.gov/study/NCT04577352> . Assessed March 19, 2025.
2. Corben LA, Collins V, Milne S, et al. Clinical management guidelines of Friedreich ataxia: Best practices in rare diseases. Orphanet J Rare Dis. 2022;17(1):415.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	04.08.25	05.25

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy,

contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:

For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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