

Clinical Policy: Deferiprone (Ferriprox)

Reference Number: IL.ERX.SPA.92

Effective Date: 06.01.21

Last Review Date: 08.21

Line of Business: Illinois Medicaid

[Revision Log](#)

See **Important Reminder** at the end of this policy for important regulatory and legal information.

Description

Deferiprone (Ferriprox[®]) is an iron chelator.

FDA Approved Indication(s)

Ferriprox is indicated for the treatment of transfusional iron overload in adult and pediatric patients (tablets: 8 years of age and older; oral solution: 3 years of age and older) with thalassemia syndromes, sickle cell disease, or other anemias.

Limitation(s) of use: Safety and effectiveness have not been established for the treatment of transfusional iron overload in patients with myelodysplastic syndrome or in patients with Diamond Blackfan anemia.

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.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions[™] that Ferriprox is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Transfusional Iron Overload (must meet all):

1. Diagnosis of transfusional iron overload due to one of the following (a, b, or c):
 - a. Thalassemia syndromes;
 - b. Sickle cell disease;
 - c. Other anemia
2. Member meets one of the following (a or b):
 - a. For Ferriprox tablets: Age \geq 8 years;
 - b. For Ferriprox oral solution: Age \geq 3 years;
3. Transfusion history of \geq 100 mL/kg of packed red blood cells (e.g., \geq 20 units of packed red blood cells for a 40 kg person) and a serum ferritin level $>$ 1,000 mcg/L;
4. Dose does not exceed 99 mg/kg per day.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Transfusional Iron Overload (must meet all):

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
2. Current documentation (within the past 30 days) shows serum ferritin level \geq 500 mcg/L;
3. If request is for a dose increase, new dose does not exceed 99 mg/kg per day.

Approval duration: 12 months

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.

Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

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 policy – ERX.PA.01 or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

ANC: absolute neutrophil count

DFO-DFP: deferiprone-deferoxamine

FDA: Food and Drug Administration

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Box Warnings;

- Contraindication(s): hypersensitivity to deferiprone or to any of the excipients in the formulation
- Boxed warning(s): agranulocytosis/neutropenia
 - Can cause agranulocytosis that can lead to serious infections and death. Neutropenia may precede the development of agranulocytosis.
 - Measure the absolute neutrophil count (ANC) before starting Ferriprox therapy and monitor the ANC weekly on therapy.
 - Interrupt Ferriprox if infection develops, and monitor the ANC more frequently.
 - Advise patients taking Ferriprox to report immediately any symptoms indicative of infection.

Appendix D: Combination Therapy

A multicentre randomized open-label trial was designed to assess the effectiveness of long-term sequential deferiprone-deferoxamine (DFO-DFP) versus DFP alone to treat thalassaemia major. The decrease of serum ferritin levels during the treatment period was statistically significantly higher in sequential DFP-DFO patients compared with DFP-alone patients (P = 0.005). Kaplan-Meier survival analysis for the two chelation treatments did not show any statistically significant differences (long-rank test, P = 0.3145). Evidence exists to support the use of combination therapy with Ferriprox (deferiprone) and Desferal (deferoxamine) in patients with severe iron overload or overt iron-related morbidity.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Transfusional iron overload	75 mg/kg PO in 2 or 3 divided doses for a total daily dose of 75 to 99 mg/kg/day in 2 or 3 divided doses	99 mg/kg/day

VI. Product Availability

- Oral solution: 100 mg/mL
- Tablets: 500 mg, 1,000 mg (three times a day) with functional scoring, 1,000 mg (twice a day) with functional scoring

VII. References

1. Ferriprox Tablets Prescribing Information. Rockville, MD: ApoPharma USA, Inc.; April 2021. Available at www.ferriprox.com. Accessed May 13, 2021.
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3. Desferal Prescribing Information. East Hanover, NJ: Novartis Pharmaceuticals Corporation; October 2020. Available at <https://dailymed.nlm.nih.gov/dailymed/>. Accessed May 13, 2021.
4. Exjade Prescribing Information. East Hanover, NJ: Novartis Pharmaceuticals Corporation; July 2020. Available at <http://www.us.exjade.com/>. Accessed May 13, 2021.
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7. Hoffbrand AV, Taher A, Cappellini MD. How I treat transfusional iron overload. *Blood.* November 1, 2012; 120(18): 3657-3669.
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Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	04.21.21	05.21
3Q 2021 annual review: RT4: added new indication for sickle cell and other anemias transfusional iron overload with pediatric expansions; references reviewed and updated.	07.16.21	08.21

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.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. 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.

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