Effective Date: 08/01/2024
Reviewed: 05/2024
Scope: Medicaid

SOHONOS (palovarotene)

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 Indication
Sohonos is indicated for the reduction in volume of new heterotopic ossification in adults and pediatric patients aged 8 years and older for females and 10 years and older for males with fibrodysplasia ossificans progressiva (FOP).

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

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Initial requests:
   1. Genetic testing results confirming diagnosis of fibrodysplasia ossificans progressiva (FOP) with documented *activin receptor type 1 (ACVR1)* R206H mutation.
   2. Chart notes or medical record documentation supporting signs and symptoms of FOP, including radiologic testing confirming heterotopic ossification.
B. Continuation requests: Chart notes or medical record documentation supporting benefit from therapy.

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who is experienced in the treatment of fibrodysplasia ossificans progressiva (FOP) (e.g., orthopedist, rheumatologist).

IV. CRITERIA FOR INITIAL APPROVAL

Fibrodysplasia ossificans progressiva (FOP)
Authorization of 6 months may be granted for reduction in the volume of new heterotopic ossification in fibrodysplasia ossificans progressiva (FOP) when all of the following criteria are met:
A. Member has a documented genetically confirmed diagnosis of FOP with genetic testing indicating the patient has an *activin receptor type 1 (ACVR1)* R206H mutation.
B. Member has signs and symptoms of FOP (e.g., malformation of the great toe, abnormal vertebral morphology, ectopic ossification in ligament or muscle tissue).
C. Member meets either of the following age criteria:

1. Member is a male 10 years of age or older.
2. Member is a female 8 years of age or older.

D. For members of reproductive potential, documented attestation from provider that the member is not pregnant and appropriate contraception methods will be used at least 1 month before treatment, during treatment, and 1 month after the last dose.

V. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for an indication listed in Section IV when both of the following criteria are met.

A. Member meets either of the following age criteria:
   1. Member is a male 10 years of age or older.
   2. Member is a female 8 years of age or older.

B. Member is experiencing benefit from therapy as evidenced by disease stability or disease improvement (e.g., reduction in the volume of new heterotopic ossification).

VI. QUANTITY LIMIT

A. Sohonos capsule 1 mg- 28 capsules per 28 days, daily dose of 1
B. Sohonos capsule 1.5 mg- 56 capsules per 28 days, daily dose of 2
C. Sohonos capsule 2.5 mg- 28 capsules per 28 days, daily dose of 1
D. Sohonos capsule 5 mg- 28 capsules per 28 days, daily dose of 1
E. Sohonos capsule 10 mg- 56 capsules per 28 days, daily dose of 2

VII. DOSAGE AND ADMINISTRATION

Indication	FDA-recommended Dosing
FOP	<u>Adults and pediatric patients 14 years and older</u> • 5 mg daily • Flare-up Dose: o Stop daily dosing when flare-up dosing begins. o 20 mg daily for 4 weeks, followed by 10 mg daily for 8 weeks (for a total of 12 weeks of flare-up treatment), even if symptoms resolve earlier, then return to daily dosing of 5 mg. o If during the course of flare-up treatment, the patient experiences marked worsening of the original flare-up site or another flare-up at a new location, restart the 12-week flare-up dosing at 20 mg daily. o For flare-up symptoms that have not resolved at the end of the 12-week period, the 10 mg daily dosage may be extended in 4-week intervals and continued until the flare-up symptoms resolve. If new flare-up symptoms

occur after the 5 mg daily dosing is resumed, flare-up dosing may be restarted.

Pediatric Patients Aged 8 to 13 Years for Females and Aged 10 to 13 Years for Males

Weight	Daily Dosage	Week 1 to 4 Flare-up Dosage	Week 5to 12 Flare-up Dosage
10 kg to 19.9 kg	2.5 mg	10 mg	5 mg
20 kg to 39.9 kg	3 mg	12.5 mg	6 mg
40 kg to 59.9 kg	4 mg	15 mg	7.5 mg
≥ 60 kg	5 mg	20 mg	10 mg

- If during the course of flare-up treatment, the patient experiences marked worsening of the original flare-up site or another flare-up at a new location, restart the 12-week flare-up dosing with the Week 1 to 4 dose.
- For flare-up symptoms that have not resolved at the end of the 12-week period, the Week 5 to 12 flare-up dose may be extended in 4-week intervals and continued until the flare-up symptoms resolve. If new flare-up symptoms occur after daily dosing is resumed, flare-up dosing may be restarted.

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

VIII. REFERENCES

1. Sohonos [package insert]. Cambridge, MA: Ipsen Biopharmaceuticals Inc; August 2023.
2. An Efficacy and Safety Study of Palovarotene for the Treatment of Fibrodysplasia Ossificans Progressiva. (MOVE). ClinicalTrials.gov identifier: NCT03312634. Updated March 14, 2023. Accessed April 2, 2024. https://classic.clinicaltrials.gov/ct2/show/NCT03312634
3. Kaplan FS, Mukaddam MA, Baujat, et al. The medical management of fibrodysplasia ossificans progressiva: current treatment considerations. Proc Intl Clin Council FOP. 2022; 2:1-127. Accessed April 2, 2024. https://www.ifopa.org/for_medical_professionals
4. Genetic and Rare Diseases Information Center (GARD). Fibrodysplasia Ossificans Progressiva. Rare Disease Database. Last updated February 2023. Accessed April 2, 2024. https://rarediseases.info.nih.gov