Clinical Policy Title:	metreleptin
Policy Number:	RxA.230
Drug(s) Applied:	Myalept®
Original Policy Date:	02/07/2020
Last Review Date:	06/10/2021
Line of Business Policy Applies to:	All lines of business

Background

Myalept® is a leptin analog indicated as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy.

Limitation(s) of use:

- The safety and effectiveness of Myalept® for the treatment of complications of partial lipodystrophy have not been established.
- The safety and effectiveness of Myalept® for the treatment of liver disease, including nonalcoholic steatohepatitis (NASH), have not been established.
- Myalept® is not indicated for use in patients with HIV-related lipodystrophy.
- Myalept® is not indicated for use in patients with metabolic disease, including diabetes mellitus and hypertriglyceridemia, without concurrent evidence of generalized lipodystrophy.

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
metreleptin (Myalept®)	Replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy	Body Weight ≤ 40 kg: 0.06 to 0.13 mg/kg SC once daily (adjust in increments of 0.02 mg/kg) Body Weight > 40 kg: • Males: 2.5 to 10 mg SC once daily (adjust in increments of 1.25 to 2.5 mg/day) • Females: 5 to 10 mg SC once daily (adjustin increments of 1.25 to 2.5 mg/day)	Weight ≤ 40 kg: 0.13 mg/kg/day Weight > 40 kg: 10 mg/day

Dosage Forms

- Lyophilized cake in vial to be reconstituted: 11.3 mg/vial (5 mg/mL after reconstitution)

Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria. The provision of provider samples does not guarantee coverage under the provisions of the pharmacy benefit administer by RxAdvance. All criteria for initial approval must be met in order to obtain coverage.

I. Initial Approval Criteria

A. Leptin Deficiency (must meet all)**:**

1. Diagnosis of leptin deficiency;
2. Age ≥ 1 year;
3. Member has congenital or acquired generalized lipodystrophy;
4. Dose does not exceed (a or b):
    a. Body weight ≤ 40 kg: 0.13 mg/kg per day;
    b. Body weight > 40 kg: 10 mg per day;

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

A. Leptin Deficiency (must meet all)**:**

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy;
2. Member is responding positively to therapy;
3. If request is for a dose increase, new dose does not exceed (a or b):
    a. Body weight ≤ 40 kg: 0.13 mg/kg per day;
    b. Body weight > 40 kg: 10 mg per day;

Approval Duration
Commercial: 6 months
Medicaid: 12 months

B. Appendices

APPENDIX A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
HIV: Human immunodeficiency virus
NASH: Nonalcoholic steatohepatitis

APPENDIX B: Therapeutic Alternatives
None

APPENDIX C: Contraindications/Boxed Warnings

- Contraindication(s):
    - General obesity not associated with congenital leptin deficiency: Myalept® has not been shown to be effective in treating general obesity, and the development of anti- metreleptin antibodies with neutralizing activity has been reported in obese patients treated with Myalept®.
    - Hypersensitivity (eg, anaphylaxis, urticaria, generalized rash) to metreleptin or any component of the formulation.

- Boxed Warning(s):
  - Risk of anti-metreleptin antibodies with neutralizing activity and risk of lymphoma.

References

1. Myalept® Prescribing Information. Cambridge, MA: Aegerion Pharmaceuticals, Inc; December 2019,Available at http://www.Myalept®pro.com. Accessed February 18, 2021.

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy established.	01/2020	02/07/2020
Policy was reviewed: 1. Policy description table was updated 2. Limitation(s) of use was updated 3. Dosing information indication updated 4. Continued therapy approval criteria II.A.1. was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance…" Appendix B, therapeutic alternative rephrased to "none" 5. Initial therapy and continued therapy approval duration for "commercial" was updated Appendix C, contraindications were updated 7. References were updated	06/19/2020	09/14/2020
Policy was reviewed: 1. **Last review date was updated.** 2. **Clinical policy verbiage added " The provision of provider samples does not guarantee…".** 3. **Continued Therapy criteria II.A.1 was rephrased from "Currently receiving medication that has been authorized by RxAdvance..."** 4. **Trademark symbol updated**	02/19/2021	06/10/2021

to "®" from "™" for drug name Myalept®. 5. **References were reviewed and updated.** 6. **Background updated to: Myalept® is a leptin analog indicated as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy.** 7. **Dosing updated to specify <u>Body Weight.</u>**		