Pharmacy Medical Necessity Guidelines:
Myalept® (metreleptin)

Effective: November 4, 2025

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	Rx	Department to Review	RxUM
These pharmacy medical necessity guidelines apply to the following: ☒ Tufts Health RITogether – A Rhode Island Medicaid Plan		**Fax Numbers:** RXUM: 617.673.0939	

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

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

The safety and effectiveness of Myalept (metreleptin) for the treatment of complications of partial lipodystrophy have not been established.

The safety and effectiveness of Myalept (metreleptin) for the treatment of liver disease, including nonalcoholic steatohepatitis, have not been established.

Myalept (metreleptin) is not indicated for use in patients with human immunodeficiency virus-related lipodystrophy.

Myalept (metreleptin) is not indicated for use in patients with metabolic disease, without concurrent evidence of generalized lipodystrophy.

Myalept (metreleptin) is available only through a Risk Evaluation and Mitigation Strategy (REMS) program.

COVERAGE GUIDELINES

The plan may authorize coverage of Myalept (metreleptin) for Members, when the following criteria are met:

1. Member has a diagnosis of congenital or acquired generalized lipodystrophy due to a leptin deficiency

AND

2. Conventional treatments for metabolic disturbances have failed (e.g., lifestyle modification, antidiabetic agents, statins)

AND

3. Documentation Myalept (metreleptin) is being used as an adjunct to diet modification

AND

4. The prescribing physician is an endocrinologist

AND

5. Documentation the Member has **at least** one of the following conditions:
   a) Diabetes Mellitus
   b) Hypertriglyceridemia
   c) Insulin Resistance

LIMITATIONS

- Coverage of Myalept is limited to 30 injections per 30 days.
- The plan will not provide coverage of Myalept (metreleptin) for:
  a) Treatment of complications of partial lipodystrophy.
  b) Treatment of liver disease, including nonalcoholic steatohepatitis.
  c) Treatment of human immunodeficiency virus-related lipodystrophy.
  d) Treatment of metabolic disease, including diabetes mellitus and hypertriglyceridemia, without concurrent evidence of generalized lipodystrophy.
  e) Treatment of general obesity not associated with congenital leptin deficiency.

CODES

None

REFERENCES

1. Chan JL, Lutz K, Cochran E, et al. Clinical effects of long-term metreleptin treatment in patients with lipodystrophy. *Endocr Pract*. 2011; 17(6):922-32.
2. Garg A. Acquired and Inherited Lipodystrophies. *N Engl J Med*. 2004; 350:1220-1234.
3. Handelsman Y, Oral EA, Bloomgarden ZT, et al. The clinical approach to the detection of lipodystrophy - an AACE consensus statement. *Endocr Pract*. 2013; 19(1):107-16.
4. Myalept prescribing information. Dublin, Ireland: Amryt Pharmaceuticals DAC; March 2022.
5. Oral EA, Simha V, Ruiz E, et al. Leptin-replacement therapy for lipodystrophy. *N Engl J Med*. 2002; 346:570-578.

APPROVAL HISTORY

September 13, 2022: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. November 14, 2023: Effective December 1, 2023, updated the Pharmacy UM fax number. No criteria updates.
2. November 12, 2024: No changes
3. November 4, 2025: No changes

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.