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5.30.045

Section:	Prescription Drugs	**Effective Date:**	July 1, 2025
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	July 1, 2014
Subject:	Myalept	**Page:**	1 of 5

Last Review Date: June 12, 2025

Myalept

Description

Myalept (metreleptin)

Background
Myalept (metreleptin for injection) is used as replacement therapy to treat the complications of leptin deficiency, in addition to diet, in patients with congenital generalized or acquired generalized lipodystrophy (1).

Generalized lipodystrophy is a condition associated with a lack of fat tissue. Patients with congenital generalized lipodystrophy are born with little or no fat tissue. Patients with acquired generalized lipodystrophy generally lose fat tissue over time. Because the hormone leptin is made by fat tissue, patients with generalized lipodystrophy have very low leptin levels. Leptin regulates food intake and other hormones, such as insulin (1).

Regulatory Status
FDA-approved indication: 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 (1).

Limitations of Use:
The safety and effectiveness of Myalept for the treatment of complications of partial lipodystrophy and 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 (1).

Myalept carries a boxed warning regarding anti-metreleptin antibodies with neutralizing activity have been identified in patients treated with Myalept. The consequences are not well characterized but could include inhibition of endogenous leptin action and loss of Myalept efficacy. Worsening metabolic control and/or severe infection have been reported. Test for anti-metreleptin antibodies with neutralizing activity in patients with severe infections or loss of efficacy during Myalept treatment (1).

Myalept is contraindicated in patients with general obesity not associated with congenital leptin deficiency (1).

T-cell lymphoma has been reported in patients with acquired generalized lipodystrophy, both treated and not treated with Myalept. Carefully consider the benefits and risks of treatment with Myalept in patients with significant hematologic abnormalities and/or acquired generalized lipodystrophy (1).

Because of the risks associated with the development of neutralizing antibodies and lymphoma, Myalept is available only through the Myalept Risk Evaluation and Mitigation Strategy (REMS) Program. Under this REMS program, prescribers must be certified with the program by enrolling in and completing training. Pharmacies must be certified with the program and only dispense Myalept after receipt of the Myalept REMS Prescription Authorization Form for each new prescription (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Myalept may be considered **medically necessary** if the conditions indicated below are met.

Myalept may be considered **investigational** for all other indications.

Prior-Approval Requirements

Diagnosis

Patient must have the following:

Leptin Deficiency

AND ALL of the following:
1. Congenital or acquired generalized lipodystrophy
2. Used as an adjunct therapy to diet
3. Physician must be enrolled in the Myalept Risk Evaluation and Mitigation Strategy (REMS) Program

AND NONE of the following:
1. HIV-related lipodystrophy
2. Partial lipodystrophy
3. Liver disease, including non-alcoholic steatohepatitis (NASH)

Prior – Approval *Renewal* Requirements

Diagnosis

Patient must have the following:

Leptin Deficiency

AND ALL of the following:
1. Congenital or acquired generalized lipodystrophy
2. Used as an adjunct therapy to diet
3. Reduction from baseline complications due to leptin deficiency

AND NONE of the following:
1. HIV-related lipodystrophy
2. Partial lipodystrophy
3. Liver disease, including non-alcoholic steatohepatitis (NASH)

Policy Guidelines

Pre - PA Allowance

None

Prior – Approval Limit

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

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. It is contraindicated in patients with general obesity. It is not approved for use in patients with HIV-related lipodystrophy or in patients with metabolic disease, including diabetes mellitus and hypertriglyceridemia, without concurrent evidence of generalized lipodystrophy. Myalept is available only through a restricted program called the Myalept Risk Evaluation and Mitigation Strategy (REMS) Program (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Myalept while maintaining optimal therapeutic outcomes.

References

1. Myalept [package insert]. Parma, Italy: Chiesi Farmaceutici S.p.A.; March 2024.

Policy History

Date	Action
July 2014	New addition to PA
September 2014	Annual review and reference update
September 2015	Annual editorial review
September 2016	Annual editorial review and reference update. Policy code changed from 5.08.19 to 5.30.45.
June 2018	Annual editorial review
December 2019	Annual review
December 2020	Annual review and reference update
June 2021	Annual review and reference update

June 2022	Annual review and reference update
June 2023	Annual review. Changed policy number to 5.30.045
September 2023	Annual review
June 2024	Annual review
September 2024	Annual review
June 2025	Annual review and reference update

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 12, 2025 and is effective on July 1, 2025.