

**Protocol for Myalept® (metreleptin)
Approved July 2021**

Background:

***Lipodystrophy syndromes** are a group of genetic or acquired disorders in which the body is unable to produce and maintain healthy fat tissue. The medical condition is characterized by abnormal or degenerative conditions of the body's adipose tissue.*

***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.*

Criteria for approval:

Myalept will be approved when **ALL** the following are met:

1. Diagnosis of congenital generalized lipodystrophy (CGL) or acquired generalized lipodystrophy (AGL) associated with leptin deficiency
2. Myalept will be used as an adjunct to diet modification
3. Myalept is prescribed by or in consultation with a specialist in the disease (e.g. endocrinologist, cardiologist)
4. There is documentation of baseline values for triglycerides, HbA1c, fasting glucose levels prior to initiating treatment
5. Weight must be received for drugs that have weight-based dosing
6. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Initial Approval: 3 months

Exclusion criteria:

Myalept will not be approved for the following:

1. Treatment of complications of partial lipodystrophy
2. Treatment of liver disease, including nonalcoholic steatohepatitis (NASH)
3. Treatment of HIV-related lipodystrophy
4. Treatment of patients with metabolic disease (diabetes mellitus, hypertriglyceridemia), without concurrent evidence of generalized lipodystrophy

Continuation of therapy:

1. Documentation of positive clinical response as demonstrated by follow-up lab data
2. Myalept is prescribed by or in consultation with a specialist in the disease (e.g. endocrinologist, cardiologist)
3. Myalept is being used as an adjunct to diet modification
4. Weight must be received for drugs that have weight-based dosing
5. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Renewal Approval: 6 months**WARNING: RISK OF ANTI-METRELEPTIN ANTIBODIES WITH NEUTRALIZING ACTIVITY AND RISK OF LYMPHOMA**

See full prescribing information for complete boxed warning. Anti-metroleptin 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 antimetroleptin antibodies with neutralizing activity in patients with severe infections or loss of efficacy during Myalept treatment. Contact Amryt Pharmaceuticals DAC at 1-866-216-1526 for neutralizing antibody testing. 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. Myalept is available only through a restricted program called the Myalept REMS PROGRAM.

References:

1. Myalept [packet insert]. Bristol-Myers Squibb Company Princeton, NJ 08543. February 2014
2. Brown RJ, Araujo-Vilar D, et al. The Diagnosis and Management of Lipodystrophy Syndromes: A Multi-Society Practice Guideline. *J Clin Endocrinol Metab.* 2016 Dec;101(12): 4500–4511.
3. Araújo-Vilar D, Santini F. Diagnosis and treatment of lipodystrophy: a step-by-step approach. *Journal of Endocrinological Investigation* (2019) 42:61–73
4. *Clinical Pharmacology* (online database). Tampa FL: Gold Standard Inc.: 2019. Updated periodically