SPECIALTY GUIDELINE MANAGEMENT

MYALEPT (metreleptin)

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.

A. FDA-Approved Indication
Myalept 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.

Limitations of Use:
A. The safety and effectiveness of Myalept for the treatment of complications of partial lipodystrophy have not been established.
B. The safety and effectiveness of Myalept for the treatment of liver disease, including nonalcoholic steatohepatitis (NASH), have not been established.
C. Myalept is not indicated for use in patients with HIV-related lipodystrophy.
D. Myalept is not indicated for use in patients with metabolic disease, including diabetes mellitus and hypertriglyceridemia, without concurrent evidence of congenital or acquired generalized lipodystrophy.

B. Compendial Use
Partial lipodystrophy in patients with confirmed leptin deficiency and metabolic abnormalities

All other indications are considered experimental/investigational and are not a covered benefit.

II. EXCLUSIONS

Coverage will not be provided for members with any of the following exclusions:
A. HIV-related lipodystrophy
B. Generalized obesity not associated with generalized lipodystrophy

III. CRITERIA FOR INITIAL APPROVAL

Lipodystrophy
Authorization of 12 months may be granted for treatment of lipodystrophy when ALL of the following criteria are met:
A. Member has a diagnosis of congenital generalized lipodystrophy (i.e., Berardinelli-Seip syndrome), acquired generalized lipodystrophy (i.e., Lawrence syndrome), or partial lipodystrophy
B. Member has leptin deficiency confirmed by laboratory testing
C. Member has at least one complication of lipodystrophy (e.g., diabetes mellitus, hypertriglyceridemia, increased fasting insulin level)

IV. CONTINUATION OF THERAPY

Lipodystrophy
Authorization of 12 months may be granted to members requesting continuation of treatment for when ALL of the following criteria are met:

A. All initial authorization criteria are met
B. Member has experienced an improvement from baseline in metabolic control (e.g., improved glycemic control, decrease in triglycerides, decrease in hepatic enzyme levels)

V. REFERENCES

1. Myalept [package insert]. Cambridge, MA: Aegerion Pharmaceuticals, Inc.; September 2015.
2. Brown RJ, Araujo-Vilar D, Cheung PT, et al. The diagnosis and management of lipodystrophy syndromes: A multi-society practice guideline. *J Clin Endocrinol Metab*. 2016;101(12):4500-4511.
3. Handelsman Y, Oral AE, Bloomgarden ZT, et al. The clinical approach to the detection of lipodystrophy – an AACE consensus statement. *Endocr Pract.* 2013;19:107-116.
4. Chan JL, Lutz K, Cochran E, et al. Clinical effects of long-term metreleptin treatment in patients with lipodystrophy. *Endocr Pract.* 2011;17:922-932.
5. Garg A. Lipodystrophies: genetic and acquired body fat disorders. *J Clin Endocrinol Metab.* 2011;96:3313-3325.
6. Rodriguez AJ, Mastronardi CA, Paz-Filho GJ. New advances in the treatment of generalized lipodystrophy: role of metreleptin. *Ther Clin Risk Manag.* 2015; 11:1391-1400.