



Louisiana

metreleptin (Myalept[®])

Policy # 00437

Original Effective Date: 07/16/2014

Current Effective Date: 07/11/2018

Applies to all products administered or underwritten by Blue Cross and Blue Shield of Louisiana and its subsidiary, HMO Louisiana, Inc. (collectively referred to as the "Company"), unless otherwise provided in the applicable contract. Medical technology is constantly evolving, and we reserve the right to review and update Medical Policy periodically.

When Services May Be Eligible for Coverage

Coverage for eligible medical treatments or procedures, drugs, devices or biological products may be provided only if:

- *Benefits are available in the member's contract/certificate, and*
- *Medical necessity criteria and guidelines are met.*

Based on review of available data, the Company may consider the use of metreleptin (Myalept[®])[†] for the treatment of complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy (LD) to be **eligible for coverage**.

Patient Selection Criteria

Coverage eligibility for metreleptin (Myalept) will be considered when all of the following criteria are met:

- Patient has complications of leptin deficiency due to a diagnosis of congenital or acquired generalized LD; and
- Myalept is NOT used to treat partial LD; and
- Myalept is NOT used in patients with metabolic disease, without concurrent evidence of generalized LD; and
- Myalept is NOT used to treat liver disease, including nonalcoholic steatohepatitis (NASH); and
- Myalept is NOT used in patients with human immunodeficiency virus (HIV)-related LD.

When Services Are Considered Investigational

Coverage is not available for investigational medical treatments or procedures, drugs, devices or biological products.

Based on review of available data, the Company considers the use of metreleptin (Myalept) when patient selection criteria are not met to be **investigational**.*

Background/Overview

Myalept is an injectable leptin analogue indicated for the treatment of complications of leptin deficiency in patients with congenital or acquired generalized LD. Myalept exerts its function by binding and activating the human leptin receptor, which belongs to the Class I cytokine family of receptors that signals through the JAK/STAT transduction pathway. Myalept is for subcutaneous administration and is supplied as a vial containing 11.3 mg of metreleptin. Dosing of Myalept varies by weight and gender. The maximum dose in patients over 40 kg is 10 mg daily.

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Lipodystrophy

LD syndromes are characterized by a loss of adipose tissue, with a resultant deficiency in leptin. Leptin is secreted by adipose tissue and is an important regulator of energy homeostasis, fat, and glucose metabolism. Adipocytes store lipids to meet the fuel requirements of non-adipose tissues during fasting. In patients with generalized LD, the shortage of adipose tissue results in hypertriglyceridemia and deposition of fat in non-adipose tissues, such as liver and muscles. This contributes to metabolic abnormalities including insulin resistance. LD syndromes are classified as either inherited or acquired and then further characterized by the extent of fat loss (generalized or partial). Many LD syndromes are associated with metabolic abnormalities which can result in life threatening comorbidities such as pancreatitis, steatohepatitis, and/or accelerated atherosclerosis. At this time, the only other available therapies for LD include diet modification, antihyperglycemic agents, and/or lipid lowering therapy. The metabolic abnormalities associated with LD are often difficult to treat, even with high doses of available diabetes and lipid-lowering therapies.

FDA or Other Governmental Regulatory Approval

U.S. Food and Drug Administration (FDA)

Myalept was approved in February of 2014 by the FDA for the treatment of complications of leptin deficiency in patients with congenital or acquired generalized LD. There are currently no other approved drugs for the treatment of metabolic abnormalities associated with LD. Current therapies (such as anti-hyperglycemic agents and lipid lowering agents) are directed toward treating the individual abnormalities.

Rationale/Source

This medical policy was developed through consideration of peer-reviewed medical literature generally recognized by the relevant medical community, FDA approval status, nationally accepted standards of medical practice and accepted standards of medical practice in this community, Blue Cross and Blue Shield Association Technology Evaluation Centers (TEC) assessment program and other non-affiliated technology evaluation centers, reference to federal regulations, other plan medical policies, and accredited national guidelines.

Myalept's approval was based on an open-label, single-arm study in patients with congenital or acquired generalized LD and diabetes mellitus, hypertriglyceridemia, and/or increased fasting insulin. There were 48 patients enrolled and the median duration of treatment was 2.7 years. Patients treated with Myalept had mean/median reductions in HbA1c, fasting glucose, and triglycerides at 1 year. The mean change from baseline in HbA1c was -2%. The mean change from baseline in fasting glucose was -49 mg/dL, and the mean change from baseline in fasting triglycerides was -184 mg/dL.

References

1. Myalept [package insert]. AstraZeneca: Wilmington, Delaware. Updated February 2014.
2. Myalept AMCP Dossier. Bristol Myers Squibb. March 2014.

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07/10/2014 Medical Policy Committee review

07/16/2014 Medical Policy Implementation Committee approval. Coverage eligibility unchanged.

06/25/2015 Medical Policy Committee review

07/15/2015 Medical Policy Implementation Committee approval. Coverage eligibility unchanged.

06/30/2016 Medical Policy Committee review

07/20/2016 Medical Policy Implementation Committee approval. Coverage eligibility unchanged.

07/06/2017 Medical Policy Committee review

07/19/2017 Medical Policy Implementation Committee approval. Coverage eligibility unchanged.

07/05/2018 Medical Policy Committee review

07/11/2018 Medical Policy Implementation Committee approval. Coverage eligibility unchanged.

Next Scheduled Review Date: 07/2019

*Investigational – A medical treatment, procedure, drug, device, or biological product is Investigational if the effectiveness has not been clearly tested and it has not been incorporated into standard medical practice. Any determination we make that a medical treatment, procedure, drug, device, or biological product is Investigational will be based on a consideration of the following:

- A. Whether the medical treatment, procedure, drug, device, or biological product can be lawfully marketed without approval of the U.S. Food and Drug Administration (FDA) and whether such approval has been granted at the time the medical treatment, procedure, drug, device, or biological product is sought to be furnished; or
- B. Whether the medical treatment, procedure, drug, device, or biological product requires further studies or clinical trials to determine its maximum tolerated dose, toxicity, safety, effectiveness, or effectiveness as compared with the standard means of treatment or diagnosis, must improve health outcomes, according to the consensus of opinion among experts as shown by reliable evidence, including:
 - 1. Consultation with the Blue Cross and Blue Shield Association technology assessment program (TEC) or other nonaffiliated technology evaluation center(s);
 - 2. Credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community; or
 - 3. Reference to federal regulations.

**Medically Necessary (or "Medical Necessity") - Health care services, treatment, procedures, equipment, drugs, devices, items or supplies that a Provider, exercising prudent clinical judgment, would provide to a patient for the purpose of preventing, evaluating, diagnosing or treating an illness, injury, disease or its symptoms, and that are:

- A. In accordance with nationally accepted standards of medical practice;
- B. Clinically appropriate, in terms of type, frequency, extent, level of care, site and duration, and considered effective for the patient's illness, injury or disease; and
- C. Not primarily for the personal comfort or convenience of the patient, physician or other health care provider, and not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of that patient's illness, injury or disease.

For these purposes, "nationally accepted standards of medical practice" means standards that are based on credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community, Physician Specialty Society recommendations and the views of Physicians practicing in relevant clinical areas and any other relevant factors.

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‡ Indicated trademarks are the registered trademarks of their respective owners.

NOTICE: Medical Policies are scientific based opinions, provided solely for coverage and informational purposes. Medical Policies should not be construed to suggest that the Company recommends, advocates, requires, encourages, or discourages any particular treatment, procedure, or service, or any particular course of treatment, procedure, or service.

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