

# Specialty Guideline Management

## Opsumit

### Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
|------------|--------------|
| Opsumit    | macitentan   |

### 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.

#### FDA-approved Indications<sup>1</sup>

Opsumit is an endothelin receptor antagonist (ERA) indicated for the treatment of pulmonary arterial hypertension (PAH, World Health Organization [WHO] Group 1) to reduce the risks of disease progression and hospitalization for PAH. Effectiveness was established in a long-term study in PAH patients with predominantly WHO Functional Class II-III symptoms treated for an average of 2 years. Patients had idiopathic and heritable PAH, PAH caused by connective tissue disorders, and PAH caused by congenital heart disease with repaired shunts.

All other indications are considered experimental/investigational and not medically necessary.

### Prescriber Specialties

This medication must be prescribed by or in consultation with a pulmonologist or cardiologist.

# Coverage Criteria

## Pulmonary Arterial Hypertension (PAH)<sup>1-5</sup>

Authorization of 12 months may be granted for treatment of PAH when ALL of the following criteria are met:

- Member has PAH defined as WHO Group 1 class of pulmonary hypertension (refer to Appendix).
- PAH was confirmed by either of the following criteria:
  - Pretreatment right heart catheterization with all of the following results:
    - Mean pulmonary arterial pressure (mPAP) > 20 mmHg
    - Pulmonary capillary wedge pressure (PCWP) ≤ 15 mmHg
    - Pulmonary vascular resistance (PVR) > 2 Wood units. For pediatric members, pulmonary vascular resistance index (PVRI) > 3 Wood units x m<sup>2</sup> is also acceptable.
  - For infants less than one year of age, PAH was confirmed by Doppler echocardiogram if right heart catheterization cannot be performed.

## Continuation of Therapy

Authorization of 12 months may be granted for members with an indication listed in the coverage criteria section who are currently receiving the requested medication through a paid pharmacy or medical benefit, and who are experiencing benefit from therapy as evidenced by disease stability or disease improvement.

## Appendix

### WHO Classification of Pulmonary Hypertension (PH)<sup>3</sup>

Note: Patients with heritable PAH or PAH associated with drugs and toxins might be long-term responders to calcium channel blockers.

#### Group 1: Pulmonary Arterial Hypertension (PAH)

- Idiopathic
  - Long-term responders to calcium channel blockers
- Heritable
- Associated with drugs and toxins
- Associated with:

- Connective tissue disease
- Human immunodeficiency virus (HIV) infection
- Portal hypertension
- Congenital heart disease
- Schistosomiasis
- PAH with features of venous/capillary (pulmonary veno-occlusive disease [PVOD]/pulmonary capillary hemangiomatosis [PCH]) involvement
- Persistent PH of the newborn

## Group 2: PH associated with Left Heart Disease

- Heart failure:
  - With preserved ejection fraction
  - With reduced or mildly reduced ejection fraction
  - Cardiomyopathies with specific etiologies (i.e., hypertrophic, amyloid, Fabry disease, and Chagas disease)
- Valvular heart disease:
  - Aortic valve disease
  - Mitral valve disease
  - Mixed valvular disease
- Congenital/acquired cardiovascular conditions leading to post-capillary PH

## Group 3: PH associated with Lung Diseases and/or Hypoxia

- Chronic obstructive pulmonary disease (COPD) and/or emphysema
- Interstitial lung disease
- Combined pulmonary fibrosis and emphysema
- Other parenchymal lung diseases (i.e., parenchymal lung diseases not included in Group 5)
- Nonparenchymal restrictive diseases:
  - Hypoventilation syndromes
  - Pneumonectomy
- Hypoxia without lung disease (e.g., high altitude)
- Developmental lung diseases

## Group 4: PH associated with Pulmonary Artery Obstructions

- Chronic thromboembolic PH
- Other pulmonary artery obstructions:
  - Sarcomas (high- or intermediate-grade or angiosarcoma)
  - Other malignant tumors (e.g., renal carcinoma, uterine carcinoma, germ-cell tumors of the testis)
  - Non-malignant tumors (e.g., uterine leiomyoma)
  - Arteritis without connective tissue disease
  - Congenital pulmonary artery stenoses
  - Hydatidosis

## Group 5: PH with Unclear and/or Multifactorial Mechanisms

- Hematological disorders, including inherited and acquired chronic hemolytic anemia and chronic myeloproliferative disorders
- Systemic disorders: Sarcoidosis, pulmonary Langerhans cell histiocytosis, and neurofibromatosis type 1
- Metabolic disorders, including glycogen storage diseases and Gaucher disease
- Chronic renal failure with or without hemodialysis
- Pulmonary tumor thrombotic microangiopathy
- Fibrosing mediastinitis
- Complex congenital heart disease

## References

1. Opsumit [package insert]. Titusville, NJ: Actelion Pharmaceuticals US, Inc.; March 2024.
2. Simonneau G, Montani D, Celermajer DS, et al. Haemodynamic definitions and updated clinical classification of pulmonary hypertension. *Eur Respir J*. 2019;53(1):1801913. doi:10.1183/13993003.01913-2018
3. Kovacs G, Bartolome S, Denton CP, et al. Definition, classification and diagnosis of pulmonary hypertension. *Eur Respir J*. 2024;64(4):2401324. doi: 10.1183/13993003.01324-2024
4. Chin KM, Gaine SP, Gerges C, et al. Treatment algorithm for pulmonary arterial hypertension. *Eur Respir J*. 2024;64(4):2401325. doi: 10.1183/13993003.01325-2024
5. Ivy D, Rosenzweig EB, Abman SH, et al. Embracing the challenges of neonatal and paediatric pulmonary hypertension. *Eur Respir J*. 2024;64(4):2401345. doi: 10.1183/13993003.01345-2024