MEDICAL MUTUAL®

Drug Policy

Policy:	Actimmune (interferon gamma-1b subcutaneous injection)	Annual Review Date: 08/24/2023 Last Revised Date: 08/24/2023

OVERVIEW

Actimmune, an interferon gamma, is indicated for the following uses:

- **Chronic granulomatous disease** (CGD), reducing the frequency and severity of serious infections.
- **Severe, malignant osteopetrosis** (SMO), delaying time to disease progression age.

In both disorders, the exact mechanism(s) of Actimmune's treatment effect has not been established. Changes in superoxide levels during Actimmune therapy do not predict efficacy and should not be used to assess patient response to therapy.

POLICY STATEMENT

This policy involves the use of Actimmune. Prior authorization is recommended for pharmacy benefit coverage of Actimmune. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Actimmune as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Actimmune be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Actimmune is recommended in those who meet the following criteria:

FDA-Approved Indications

1. **Chronic Granulomatous Disease.** Approve for 1 year if the patient meets of the following criteria (A and B):
   - **A)** Diagnosis has been established by a molecular genetic test identifying a gene-related mutation linked to chronic granulomatous disease; AND
     Note: Examples of gene-related mutations linked to chronic granulomatous disease include biallelic pathogenic variants in *CYBA*, *CYBB*, *NCF1*, *NCF2*, and *NCF4*.
   - **B)** The medication is prescribed by, or in consultation with, an immunologist.

2. **Malignant Osteopetrosis, Severe Infantile.** Approve for 1 year if the patient meets of the following criteria (A and B):
   - **A)** Diagnosis has been established by ONE of the following (i or ii)
     - **i.** Patient has had a radiographic (X-ray) imaging demonstrating skeletal features related to osteopetrosis; OR
     - **ii.** Patient has had a molecular genetic test identifying a gene-related mutation linked to severe, infantile malignant osteopetrosis; AND

       Note: Examples of genes linked to osteopetrosis include *CA2, CLCN7, IKBLG, ITGB3, LRP5, OSTM1, PLEKHM1, SNX10, TCIRG1, TNFRSF11A,* and *TNFSF11.*
   - **B)** The medication is prescribed by, or in consultation with, an endocrinologist.

Initial Approval/ Extended Approval.
A) *Initial Approval:* 365 days
B) *Extended Approval:* 365 days

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Actimmune has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Actimmune® subcutaneous injection [prescribing information]. Lake Forest, IL: Horizon; May 2021.
2. National Institute of Allergy and Infectious Diseases. Chronic granulomatous disease (CGD). Available at: https://www.niaid.nih.gov/diseases-conditions/chronic-granulomatous-disease-cgd#:~:text=Chronic%20granulomatous%20disease%20(CGD)%20is,threatening%20bacterial%20and%20fungal%20infections. Last reviewed May 20, 2022. Accessed on March 29, 2023.

3. Genetics Home Reference. National Institutes of Health, U.S. National Library of Medicine. Available at https://ghr.nlm.nih.gov/. Accessed on March 29, 2023. Search terms: chronic granulomatous disease.
4. Bonilla F, Khan D, Ballas Z, et al. Practice parameter for the diagnosis and management of primary immunodeficiency. *The Journal of Allergy and Clinical Immunology*. 2015;136:5:1186-1205.e78.
5. Charoenngam N, Nasr A, Shirvani A, Holick MF. Hereditary metabolic bone diseases: a review of pathogenesis, diagnosis and management. *Genes*. 2022;13:1880.
6. Genetics Home Reference. National Institutes of Health, U.S. National Library of Medicine. Available at https://ghr.nlm.nih.gov/. Accessed on March 29, 2023. Search terms: osteopetrosis.
7. Wu C, Econs M, DiMeglio L, et al. Diagnosis and management of osteopetrosis: consensus guidelines from the osteopetrosis working group. *J Clin Endocrinol Metab*. 2017;102:3111-3123.