| Reference number |
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| 2051-A |

# SPECIALTY GUIDELINE MANAGEMENT

## CEREZYME (imiglucerase)

### 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 Indications
Cerezyme is indicated for long-term enzyme replacement therapy (ERT) for pediatric and adult patients with a confirmed diagnosis of type 1 Gaucher disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, hepatomegaly, or splenomegaly.

B. Compendial Uses
1. Gaucher disease type 2
2. Gaucher disease type 3

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

### II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review: beta-glucocerebrosidase enzyme assay or genetic testing results supporting diagnosis.

### III. CRITERIA FOR INITIAL APPROVAL

**A. Gaucher disease type 1**
Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

**B. Gaucher disease type 2**
Authorization of 12 months may be granted for treatment of Gaucher disease type 2 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

**C. Gaucher disease type 3**
Authorization of 12 months may be granted for treatment of Gaucher disease type 3 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.

### IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for Gaucher disease type 1, type 2, or type 3 who are not experiencing an inadequate response or any intolerable adverse events from therapy.

## V. REFERENCES

1. Cerezyme [package insert]. Cambridge, MA: Genzyme Corporation; April 2018.
2. Altarescu G, Hill S, Wiggs E, et al. The efficacy of enzyme replacement therapy in patients with chronic neuronopathic Gaucher's disease. *J Pediatr.* 2001;138:539-547.
3. Erikson A, Forsberg H, Nilsson M, Astrom M, Mansson JE. Ten years' experience of enzyme infusion therapy of Norrbottnian (type 3) Gaucher disease. *Acta Paediatr*. 2006;95:312-317.
4. Pastores GM, Hughes DA. Gaucher Disease. [Updated February 26, 2015]. In: Pagon RA, Adam MP, Ardinger HH, et al, editors. GeneReviews® [Internet]. Seattle, WA: University of Washington, Seattle; 1993-2016.
5. Kaplan P, Baris H, De Meirleir L, et al. Revised recommendations for the management of Gaucher disease in children. *Eur J Pediatr.* 2013;172:447-458.
6. American Society of Health System Pharmacists. AHFS Drug Information. Bethesda, MD. Electronic version, 2021. Available with subscription. URL: http://online.lexi.com/crlsql/servlet/crlonline. Accessed January 28, 2021.
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