Market Applicability														
Market	**DC**	**FL & FHK**	**FL MMA**	**FL LTC**	**GA**	**KS**	**KY**	**MD**	**NJ**	**NV**	**NY**	**TN**	**TX**	**WA**
Applicable	X	X	NA	NA	X	NA	X	X	X	X	X	NA	NA	NA

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Enzyme Replacement Therapy for Gaucher Disease

Override(s)	Approval Duration
Prior Authorization	1 Year

Medications
Cerezyme (imiglucerase) ELELYSO (taliglucerase alfa) VPRIV (velaglucerase alfa)

APPROVAL CRITERIA

Requests for enzyme replacement therapy for Gaucher disease [Cerezyme (imiglucerase), Elelyso (taliglucerase) and Vpriv (velaglucerase)] may be approved if the following criteria are met:

I. Individual is 18 years of age and older with a diagnosis of **type 1** Gaucher disease and the following criteria are met:
   A. Type 1 Gaucher disease is confirmed by either (Weinreb, 2004; Wang, 2011):
      1. Deficiency in gluocerebrosidase enzyme activity as measured in the white blood cells or skin fibroblasts; **OR**
      2. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome;

   AND

   B. Individual has clinically significant manifestations of Gaucher disease including (Andersson, 2005; Weinreb, 2004):
      1. Skeletal disease (such as but not limited to avascular necrosis, Erlenmeyer flask deformity, osteopenia or pathological fracture); **OR**
      2. Two or more of the following:
         a. Clinically significant hepatomegaly; **OR**
         b. Clinically significant splenomegaly; **OR**
         c. Hemoglobin at least 1.0g/dL below lower limit for normal for age and sex; **OR**
         d. Platelet count less than or equal to 120,000mm^3;

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.
CRX-ALL-0366-19

Market Applicability														
Market	**DC**	**FL & FHK**	**FL MMA**	**FL LTC**	**GA**	**KS**	**KY**	**MD**	**NJ**	**NV**	**NY**	**TN**	**TX**	**WA**
Applicable	X	X	NA	NA	X	NA	X	X	X	X	X	NA	NA	NA

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OR

II. Individual is less than 18 years of age with a diagnosis of **type 1** Gaucher disease and the following criteria are met:
   A. Type 1 Gaucher disease is confirmed by either (Kaplan, 2013; Wang, 2011):
      1. Deficiency in glucocerebrosidase activity as measured in the white blood cells or skin fibroblasts; **OR**
      2. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome;

   AND
   B. Individual has clinically significant manifestations of Gaucher disease (such as but not or growth failure) (Andersson, 2005);

OR

III. Individual is 18 years of age or older with a diagnosis of **type 3** Gaucher disease and the following criteria are met (Kaplan, 2013):
   A. Type 3 Gaucher disease is confirmed by genotype testing indicating mutation of two alleles of the glucocerebrosidase genome (Kaplan, 2013; Wang, 2011); **AND**
   B. Individual has clinically significant manifestations of Gaucher disease including (Andersson, 2005; Weinreb, 2004):
      1. Skeletal disease (such as but not limited to avascular necrosis, Erlenmeyer flask deformity, osteopenia or pathological fracture); **OR**
      2. Two or more of the following:
         a. Clinically significant hepatomegaly**; OR**
         b. Clinically significant splenomegaly**; OR**
         c. Hemoglobin at least 1.0 g/dL below lower limit for normal for age and sex)**; OR**
         d. Platelet count less than or equal to 120,000mm^3;

   AND
   C. There are neurological findings consistent with type 3 Gaucher disease based on neurological evaluation including brain imaging [magnetic resonance imaging (MRI) or computed tomography (CT)] and electroencephalography (EEG) (Vellodi, 2009);

OR

IV. Individual is less than 18 years of age with type 3 Gaucher disease and the following criteria are met (Kaplan, 2013):
   A. Type 3 Gaucher disease is confirmed by genotype testing indicating mutation of two alleles of the glucocerbrosidase genome (Kaplan, 2013; Wang, 2011); **AND**

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Market Applicability														
Market	**DC**	**FL & FHK**	**FL MMA**	**FL LTC**	**GA**	**KS**	**KY**	**MD**	**NJ**	**NV**	**NY**	**TN**	**TX**	**WA**
Applicable	X	X	NA	NA	X	NA	X	X	X	X	X	NA	NA	NA

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B. Individual has clinically significant manifestations of Gaucher disease (such as but not limited to hepatomegaly, splenomegaly, anemia, thrombocytopenia, skeletal disease or growth failure) (Andersson, 2005);
AND
C. There are neurological findings consistent with type 3 Gaucher disease based on neurological evaluation including brain imaging [magnetic resonance imaging (MRI) or computed tomography (CT)] and electroencephalography (EEG) (Vellodi, 2009).

Enzyme replacement therapy for Gaucher disease [Cerezyme (imiglucerase), Elelyso (taliglucerase) and Vpriv (velaglucerase)] may **not** be approved for the following:

I. Individuals with type 2 Gaucher disease; **OR**
II. Use in conjunction with another enzyme replacement therapy agent or substrate reduction therapy agent [Cerdelga (eliglustat), Zavesca (miglustat)] for the treatment of Gaucher disease.

State Specific Mandates		
State name N/A	Date effective N/A	Mandate details (including specific bill if applicable) N/A

Key References:

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Market Applicability														
Market	**DC**	**FL & FHK**	**FL MMA**	**FL LTC**	**GA**	**KS**	**KY**	**MD**	**NJ**	**NV**	**NY**	**TN**	**TX**	**WA**
Applicable	X	X	NA	NA	X	NA	X	X	X	X	X	NA	NA	NA

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