Fabrazyme

Description

Fabrazyme (agalsidase beta)

Background
Fabry disease is an X-linked genetic disorder of glycosphingolipid metabolism. Deficiency of the lysosomal enzyme α-galactosidase A leads to progressive accumulation of glycosphingolipids, predominantly GL-3, in many body tissues, starting early in life and continuing over decades. Clinical manifestations of Fabry disease include renal failure, cardiomyopathy, and cerebrovascular accidents. Accumulation of GL-3 in renal endothelial cells may play a role in renal failure (1).

Regulatory Status
FDA-approved indication: Fabrazyme is indicated for use in patients with Fabry disease. Fabrazyme reduces globotriaosylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types (1).

Life-threatening anaphylactic and severe allergic reactions have been observed in some patients during Fabrazyme infusions. If severe allergic or anaphylactic reactions occur, immediately discontinue administration of Fabrazyme and provide necessary emergency treatment. Patients with advanced Fabry disease may have compromised cardiac function, which may predispose them to a higher risk of severe complications from infusion reactions. Appropriate medical support measures should be readily available when Fabrazyme is administered because of the potential for severe infusion reactions (1).

Safety and efficacy in patients younger than 8 years of age have not yet been evaluated (1).
Fabrazyme may be considered medically necessary for the treatment of Fabry disease in patients 8 years of age and older.

Fabrazyme may be considered investigational in patients less than 8 years of age and for all other indications.

**Prior-Approval Requirements**

**Age**

8 years old or older

**Diagnosis**

Patient must have the following:

- Fabry disease

**Prior – Approval Renewal Requirements**

Same as above

**Pre - PA Allowance**

None

**Prior - Approval Limits**

**Duration**

2 years

**Prior – Approval Renewal Limits**

Same as above

**Rationale**
Summary

Fabrazyme is indicated for use in patients with Fabry disease. Fabrazyme reduces globotriaosylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types. Life-threatening anaphylactic and severe allergic reactions have been observed in some patients during Fabrazyme infusions (1). Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Fabrazyme while maintaining optimal therapeutic outcomes.

References


Policy History

<table>
<thead>
<tr>
<th>Date</th>
<th>Action</th>
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<tbody>
<tr>
<td>March 2010</td>
<td>Age updated to current package insert recommendations. The safety and efficacy of Fabrazyme were assessed in a multi-national, multi-center, uncontrolled, open-label study in 16 pediatric patients with Fabry disease, ages 8 to 16 years. Patients younger than 8 years of age were not included in clinical studies. The safety and efficacy in patients younger than 8 years of age have not been evaluated. No new safety concerns were identified in pediatric patients in this study, and the overall safety and efficacy profile of Fabrazyme treatment in pediatric patients was found to be consistent with that seen in adults.</td>
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<tr>
<td>September 2011</td>
<td>Annual editorial review and reference update</td>
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<td>September 2012</td>
<td>Annual editorial review and reference update</td>
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<td>June 2013</td>
<td>Annual editorial review and reference update</td>
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<tr>
<td>September 2014</td>
<td>Annual editorial review and reference update</td>
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<tr>
<td>September 2015</td>
<td>Annual review</td>
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<tr>
<td>September 2016</td>
<td>Annual editorial review</td>
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<tr>
<td>December 2017</td>
<td>Policy number change from 5.08.07 to 5.30.35</td>
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<tr>
<td>November 2018</td>
<td>Annual review</td>
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<tr>
<td>December 2019</td>
<td>Annual editorial review and reference update. Changed approval duration from lifetime to 2 years</td>
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Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective on January 1, 2020.