BlueCross BlueShield of Arizona
An Independent Licensee of the Blue Cross and Blue Shield Association

MEDICAL COVERAGE GUIDELINES
SECTION: DRUGS

ORIGINAL EFFECTIVE DATE: 07/16/12
LAST REVIEW DATE: 07/08/14
LAST CRITERIA REVISION DATE:
ARCHIVE DATE:

FABRAZYME®

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Medical Coverage Guideline must be read in its entirety to determine coverage eligibility, if any.

The section identified as "Description" defines or describes a service, procedure, medical device or drug and is in no way intended as a statement of medical necessity and/or coverage.

The section identified as "Criteria" defines criteria to determine whether a service, procedure, medical device or drug is considered medically necessary or experimental or investigational.

State or federal mandates, e.g., FEP program, may dictate that any drug, device or biological product approved by the U.S. Food and Drug Administration (FDA) may not be considered experimental or investigational and thus the drug, device or biological product may be assessed only on the basis of medical necessity.

Medical Coverage Guidelines are subject to change as new information becomes available.

For purposes of this Medical Coverage Guideline, the terms "experimental" and "investigational" are considered to be interchangeable.

Description:

Fabry disease is an inherited disorder caused by the deficiency of an enzyme called alpha-galactosidase A or alpha-GAL. This enzyme is needed to metabolize lipids, fat-like substances that include oils, waxes, and fatty acids. A mutation in the gene that controls the alpha-GAL enzyme causes insufficient breakdown of lipids, which build up to harmful levels in the eyes, kidneys, autonomic nervous system, and cardiovascular system. Fabrazyme® (agalsidase beta) is intended to replace the missing alpha-GAL enzyme.

Lipidosis is a generalized term for disorder of lipid metabolism. Lipidoses include Fabry disease as well as other diseases to include, but not limited to, GM1 gangliosidoses, GM2 gangliosidoses, Gaucher disease, Niemann-Pick disease, fucosidosis, Schindler disease, metachromatic leukodystrophy (MLD), Krabbe disease, multiple sulfatase deficiency, Farber disease, and Wolman disease.

FABRAZYME (cont.)

Criteria:

- Fabrazyme is considered ***medically necessary*** with documentation of **ALL** of the following:

  1. Individual is diagnosed with Fabry disease
  2. Dosage is not greater than 1mg/kg administered as an intravenous (IV) infusion
  3. Infusion is not greater than every two weeks

- If above criteria are not met, Fabrazyme for all other indications not previously listed is considered ***experimental or investigational*** based upon:

  1. Lack of final approval from the Food and Drug Administration, and
  2. Insufficient scientific evidence to permit conclusions concerning the effect on health outcomes, and
  3. Insufficient evidence to support improvement of the net health outcome, and
  4. Insufficient evidence to support improvement of the net health outcome as much as, or more than, established alternatives, and
  5. Insufficient evidence to support improvement outside the investigational setting.

  These indications include, *but are not limited to:*

  - Lipidosis

Resources:

FDA Product Approval Information for Fabrazyme:

- 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).

  The recommended dosage of Fabrazyme is 1 mg/kg body weight infused every two weeks as an intravenous (IV) infusion. Patients should receive antipyretics prior to infusion.