Agalsidase beta

Targets (1)

IDENTIFICATION

Name	Agalsidase beta
Accession Number	DB00103 (BTD00075, BIOD00075)
Type	Biotech
Groups	Approved, Investigational
Biologic Classification	Protein Based Therapies Recombinant Enzymes
Description	Recombinant human alpha-galactosidase A. The mature protein is composed of 2 subunits of 398 residues. Protein is glycosylated and produced by CHO cells
Protein structure	
Protein chemical formula	$C_{2029}H_{3080}N_{544}O_{587}S_{27}$
Protein average weight	45351.6 Da

Sequences

```
>DB00103 sequence
LDNGLARTPTMGWLHWERFMCNLDCQEEPDSCISEKLFMEMAELMVSEGWKDAGYEYLCI
DDCWMAPQRDSEGRLQADPQRFPHGIRQLANYVHSKGLKLGIYADVGNKTCAGFPGSFGY
YDIDAQTFADWGVDLLKFDGCYCDSLENLADGYKHMSLALNRTGRSIVYSCEWPLYMWPF
QKPNYTEIRQYCNHWRNFADIDDSWKSIKSILDWTSFNQERIVDVAGPGGWNDPDMLVIG
NFGLSWNQQVTQMALWAIMAAPLFMSNDLRHISPQAKALLQDKDVIAINQDPLGKQGYQL
RQGDNFEVWERPLSGLAWAVAMINRQEIGGPRSYTIAVASLGKGVACNPACFITQLLPVK
RKLGFYEWTSRLRSHINPTGTVLLQLENTMQMSLKDLL
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Synonyms

- Agalsidasa alfa
- Agalsidasa beta
- Agalsidase alfa
- Alpha-galactosidase A

Prescription Products

Show 10 entries Search

NAME	DOSAGE	STRENGTH	ROUTE	LABELLER	MARKETING START	MARKETING END	
Fabrazyme	Injection, powder, for	5 mg	Intravenous	Genzyme Europe Bv	2001-08-03	Not applicable	

	powder, for solution			Europe Bv			
Fabrazyme	Injection, powder, lyophilized, for solution	5 mg/1mL	Intravenous	Genzyme Corporation	2008-12-17	Not applicable	
Fabrazyme	Injection, powder, for solution	5 mg	Intravenous	Genzyme Europe Bv	2001-08-03	Not applicable	
Fabrazyme	Powder, for solution	35 mg	Intravenous	Sanofi Genzyme, a Division of Sanofi Aventis Canada Inc	2004-04-08	Not applicable	
Fabrazyme	Injection, powder, lyophilized, for solution	5 mg/1mL	Intravenous	Genzyme Corporation	2008-12-17	Not applicable	
Fabrazyme	Injection, powder, for solution	35 mg	Intravenous	Genzyme Europe Bv	2001-08-03	Not applicable	
Fabrazyme	Powder, for solution	5 mg	Intravenous	Sanofi Genzyme, a Division of Sanofi Aventis Canada Inc	2004-09-17	Not applicable	
Fabrazyme	Injection, powder, for solution	5 mg	Intravenous	Genzyme Europe Bv	2001-08-03	Not applicable	
Fabrazyme	Injection, powder, for solution	35 mg	Intravenous	Genzyme Europe Bv	2001-08-03	Not applicable	

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Categories

- Alimentary Tract and Metabolism
- Amino Acids, Peptides, and Proteins
- Enzymes
- Enzymes and Coenzymes
- Fabry Disease
- Galactosidases
- Glycoside Hydrolases
- Hydrolases
- Hydrolytic Lysosomal Neutral Glycosphingolipid-specific Enzyme
- Protein Isoforms
- Proteins

UNII RZD65TSM9U

CAS number 104138-64-9

PHARMACOLOGY

Indication For treatment of Fabry's disease (alpha-galactosidase A deficiency)

Associated Conditions Fabry's Disease

Pharmacodynamics Used in the treatment of Fabry disease, an X-linked genetic disorder of glycosphingolipid metabolism. The disease is characterized by a deficiency of the lysosomal enzyme alpha-galactosidase A, which leads to progressive accumulation of glycosphingolipids, predominantly GL-3, in many body tissues. Clinical manifestations of Fabry disease include renal failure, cardiomyopathy, and cerebrovascular accidents. Fabrazyme is intended to provide an exogenous

agalasidase beta in preventing morbidity.

Mechanism of action

Alpha-galactosidase A catalyzes the hydrolysis of globotriaosylceramide (GL-3) and other a-galactyl-terminated neutral glycosphingolipids, such as galabiosylceramide and blood group B substances to ceramide dihexoside and galactose.

TARGET	ACTIONS	ORGANISM
(A) Globotriaosylceramide	ligand	Humans

Absorption

Not Available

Volume of distribution

Not Available

Protein binding

Not Available

Metabolism

Not Available

Route of elimination

Not Available

Half life

45-102 min

Clearance

- 4.1 +/- 1.2 mL/min/kg [adult patients with Fabry disease,0.3 mg/kg, 1 infusion]
- 4.6 +/- 2.2 mL/min/kg [adult patients with Fabry disease, 0.3 mg/kg, 5 infusions]
- 2.1 +/- 0.7 mL/min/kg [adult patients with Fabry disease, 1 mg/kg, 1 infusion]
- 3.2 +/- 2.6 mL/min/kg [adult patients with Fabry disease, 1 mg/kg, 5 infusions]
- 0.8 +/- 0.3 mL/min/kg [adult patients with Fabry disease, 3 mg/kg, 1 infusion]
- 0.8 +/- 0.4 mL/min/kg [adult patients with Fabry disease, 3 mg/kg, 5 infusions]
- 1.8 +/- 0.8 mL/min/kg [Pediatric Patients with Fabry Disease, 1 mg/kg, 1-3 infusions]
- 4.9 +/- 5.6 mL/min/kg [Pediatric Patients with Fabry Disease, 1 mg/kg, 7 infusions]
- 2.3 +/- 2.2 mL/min/kg [Pediatric Patients with Fabry Disease, 1 mg/kg, 11 infusions]

Toxicity

Not Available

Affected organisms

Humans and other mammals

Pathways

Not Available

Pharmacogenomic Effects/ADRs

Not Available

INTERACTIONS

Drug Interactions

ALL DRUGS | APPROVED | VET APPROVED | NUTRACEUTICAL | ILLICIT | WITHDRAWN | INVESTIGATIONAL | EXPERIMENTAL

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DRUG	INTERACTION
Amiodarone	The therapeutic efficacy of Agalsidase beta can be decreased when used in combination with Amiodarone.
Chloroquine	The therapeutic efficacy of Agalsidase beta can be decreased when used in combination with Chloroquine.
Gentamicin	The therapeutic efficacy of Agalsidase beta can be decreased when used in combination with Gentamicin.

Food Interactions Not Available

REFERENCES

General References

1. Schaefer RM, Tylki-Szymanska A, Hilz MJ: Enzyme replacement therapy for Fabry disease: a systematic review of available evidence. Drugs. 2009 Nov 12;69(16):2179-205. doi: 10.2165/11318300-000000000-00000. [PubMed:19852524]
2. El Dib RP, Pastores GM: Enzyme replacement therapy for Anderson-Fabry disease. Cochrane Database Syst Rev. 2010 May 12;(5):CD006663. doi: 10.1002/14651858.CD006663.pub2. [PubMed:20464743]
3. Lim-Melia ER, Kronn DF: Current enzyme replacement therapy for the treatment of lysosomal storage diseases. Pediatr Ann. 2009 Aug;38(8):448-55. [PubMed:19725195]

External Links

UniProt	P06280
Genbank	X14448
PubChem Substance	46508538
ChEMBL	CHEMBL2108888
PharmGKB	PA164746527
RxList	RxList Drug Page
Drugs.com	Drugs.com Drug Page
Wikipedia	Agalsidase_beta

ATC Codes

A16AB03 — Agalsidase alfa
- A16AB — Enzymes
- A16A — OTHER ALIMENTARY TRACT AND METABOLISM PRODUCTS
- A16 — OTHER ALIMENTARY TRACT AND METABOLISM PRODUCTS
- A — ALIMENTARY TRACT AND METABOLISM

A16AB04 — Agalsidase beta
- A16AB — Enzymes
- A16A — OTHER ALIMENTARY TRACT AND METABOLISM PRODUCTS
- A16 — OTHER ALIMENTARY TRACT AND METABOLISM PRODUCTS
- A — ALIMENTARY TRACT AND METABOLISM

AHFS Codes 44:00.00 — Enzymes

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CLINICAL TRIALS

Clinical Trials

Show 10 entries Search

PHASE	STATUS	PURPOSE	CONDITIONS	COUNT
1	Completed	Treatment	Fabry's Disease	2
2	Completed	Not Available	Fabry's Disease	1
2	Completed	Treatment	Fabry's Disease	8
2	Terminated	Treatment	Chronic Kidney Disease, Stage IV (Severe) / Fabry's Disease	1
3	Completed	Treatment	Fabry's Disease	5
3	Recruiting	Treatment	Fabry's Disease	1
4	Completed	Not Available	Fabry's Disease	1
4	Completed	Treatment	Fabry's Disease	6
4	Recruiting	Treatment	Fabry's Disease	1
4	Withdrawn	Treatment	Fabry's Disease	1

PHARMACOECONOMICS

Manufacturers Not Available

Packagers Genzyme Inc. Shire Inc.

Dosage forms

Show 10 entries Search

FORM	ROUTE	STRENGTH
Injection, powder, for solution	Intravenous	35 mg
Injection, powder, for solution	Intravenous	5 mg
Injection, powder, lyophilized, for solution	Intravenous	5 mg/1mL
Powder, for solution	Intravenous	35 mg
Powder, for solution	Intravenous	5 mg
Injection, solution, concentrate	Intravenous	1 mg/ml
Solution	Intravenous	1 mg

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Prices

Show 10 entries Search

UNIT DESCRIPTION	COST	UNIT
Fabrazyme 35 mg vial	5403.6USD	vial
Fabrazyme 5 mg vial	771.6USD	vial

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Patents

Show 10 entries Search

PATENT NUMBER	PEDIATRIC EXTENSION	APPROVED	EXPIRES (ESTIMATED)	
CA2265464	No	2007-06-26	2017-09-12	

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PROPERTIES

State Liquid

Experimental Properties

PROPERTY	VALUE	SOURCE
hydrophobicity	-0.307	Not Available
isoelectric point	5.17	Not Available

TAXONOMY

Description Not Available

Kingdom Organic Compounds

Super Class Organic Acids

Class Carboxylic Acids and Derivatives

Direct Parent	Peptides
Alternative Parents	Not Available
Substituents	Not Available
Molecular Framework	Not Available
External Descriptors	Not Available

TARGETS

1. Globotriaosylceramide

Kind
Group

Organism
Humans

Pharmacological action
Yes

Actions
Ligand

References

1. Schaefer RM, Tylki-Szymanska A, Hilz MJ: Enzyme replacement therapy for Fabry disease: a systematic review of available evidence. Drugs. 2009 Nov 12;69(16):2179-205. doi: 10.2165/11318300-000000000-00000. [PubMed:19852524]
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3. Lim-Melia ER, Kronn DF: Current enzyme replacement therapy for the treatment of lysosomal storage diseases. Pediatr Ann. 2009 Aug;38(8):448-55. [PubMed:19725195]

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