Pharmacy Focus:
Genglycos™ for Glycogen Storage Disease Type Ia (GSDIa)

Key Takeaways[1-3]

Glycogen storage disease type Ia (GSDIa) is a rare genetic disorder involving life-threatening hypoglycemia. It is a chronic condition that requires lifelong management to avoid disease complications.

GSDIa has historically been treated by the oral administration of cornstarch slurries, an approach that serves as a source of energy.

Genglycos™ is a new gene therapy for GSDIa that was approved for adults and children eight years and older. This therapy is used with continued nutrition support to help reduce daily cornstarch slurries. The estimated cost of Genglycos is $2,700,000 for the one-time infusion, not including ancillary costs.

Disease Overview[1-5]

Description: GSDIa (also known as von Gierke disease) is a rare genetic disorder in which affected patients are unable to break down glycogen. Glycogen is the main source of energy in the body, but it must be broken down into glucose for the body to utilize the energy.

Characteristics:

- Disease onset typically occurs in infants at three to four months of age when the time between feedings is increased. Onset may also occur at birth or later in childhood.
- Physical characteristics of the disease include an enlarged liver (resulting in a distended abdomen), full cheeks, short stature, thin extremities, and yellow fatty deposits in the skin.
- Symptoms include fatigue, irritability, or seizures. There also may be delayed developmental or growth milestones.

Subtypes & Causes:

- There are two subtypes of glycogen storage disease (GSD): Ia and Ib. GSDIa comprises 80% of GSD diagnoses.
- On a molecular level, GSDIa is caused by deficiency of the enzyme glucose-6-phosphatase-α (G6Pase), which results in impaired formation and breakdown of glycogen.

Severity: If untreated, GSDIa can result in a range of complications from chronic illnesses, organ damage, and life-threatening episodes of low blood sugar.

Occurrence:

- GSDIa occurs in approximately one per every 100,000 live births worldwide.
- Males and females are affected equally.
- While GSDIa is diagnosed in different ethnic groups worldwide, it is more common in the Ashkenazi Jewish population, affecting approximately 1 in every 20,000 individuals in that group.
- It is estimated that there are 1,500 to 2,500 patients affected in the U.S.

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Treatment[2-5]

GSDIa is generally treated through dietary management. Specifically, slurries of cornstarch and water are taken by mouth throughout the day to raise glucose levels so the body has a source of energy to function. The dose of cornstarch varies based on age, activity level, and metabolic needs (e.g., the dose may be higher during puberty or pregnancy). For example, based on ideal body weight, a young child may require 1 g/kg to 1.6 g/kg every three to four hours throughout the day, including overnight. This means patients and caregivers must wake up throughout the night or risk severe complications, including possible death.

Genglycos™ is a new gene therapy for patients with GSDIa. It is administered as a single infusion that provides the missing *G6PC* gene to liver cells, restoring the enzyme required for the breakdown of glycogen to glucose.

Genglycos™	
Generic Name	pariglasgene brecaparvovec-opnr
Manufacturer	Ultragenyx Pharmaceutical Inc.
Approval	August 20, 2026
Use	Adult and pediatric patients eight years and older with GSDIa
How It Works	Delivers a working copy of the missing *G6PC* gene to liver cells to help restore the enzyme needed to break down glycogen to glucose
Procedure Location	Provider-administered at a Qualified Treatment Center (QTC)
Timeframe	Will be available through QTCs within 30 to 60 days after approval
Efficacy	In 20 patients, at week 96, researchers observed that reductions in orally administered cornstarch resulted in improved glucose levels
Estimated Cost	$2,700,000 for the one-time administration
Associated Benefit	Medical

HMConnects™

As part of the HMConnects cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing, and collaborating on the content of current policies, monitoring trends, and implementing appropriate cost savings techniques. Additional practices include the prevention of stockpiling, working to ensure prescriptions are filled via in-network pharmacies, and assessing to determine if patients are properly dosed based on weight and lab values when appropriate. All of these services are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com.**

References: [1]Kruger E, Nedzesky J, Thomas N, et al. Glycogen Storage Disease Type Ia: A Retrospective Claims Analysis of Complications, Resource Utilization, and Cost of Care. *JHEOR*. 2025;12(1):13-21.; [2]Emerging Therapy Solutions. (2025). *Glycogen storage disease type Ia clinical compendium: Clinical features, diagnosis, treatment and care management*.; Reuters. (2026, August 19).; [3]*Ultragenyx's gene therapy for rare metabolic disorder secures U.S. FDA nod*. https://www.reuters.com/business/healthcare-pharmaceuticals/ultragenyxs-gene-therapy-rare-metabolic-disorder-secures-us-fda-nod-2026-08-19/; [4]Ultragenyx Pharmaceutical. (2026, August 19). *Ultragenyx announces U.S. FDA approval of GENGLYCOS™ gene therapy, the first-ever FDA-approved treatment designed to treat the underlying cause of Glycogen Storage Disease Type Ia (GSDIa)* [Press release].; [5]IPD Analytics. (2026). *DTX401 for Glycogen Storage Disease 1a: Budget impact model.*

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MTG-3704 (8/26)