

CUT DOWN ON CORNSTARCH WITH GENGLYCOS*



The first and only FDA-approved gene therapy used along with nutritional management to help reduce daily cornstarch intake in individuals 8 years and older with glycogen storage disease type Ia (GSDIa).

Learn more about the study results inside.

*Based on the average daily cornstarch intake results at week 48 in a randomized clinical study of GENGLYCOS vs placebo.

WHAT IS GENGLYCOS (pariglasgene brecaparvovec-opnr)?

GENGLYCOS is a prescription gene therapy used along with nutritional management to help reduce the amount of cornstarch taken daily in adults and children 8 years and older with Glycogen Storage Disease type Ia (GSDIa).

This use is approved under accelerated approval based on clinical study results that measured reduction in daily cornstarch intake. Verification of a clinical benefit may be needed for continued approval of GENGLYCOS.

IMPORTANT SAFETY INFORMATION

Do not use GENGLYCOS if you have severe liver scarring (fibrosis or cirrhosis).

Please see inside pages and full Prescribing Information for Important Safety Information.

 **genglycos™**
pariglasgene
breCAParvovec-opnr
suspension for IV infusion

How is GENGLYCOS designed to work?

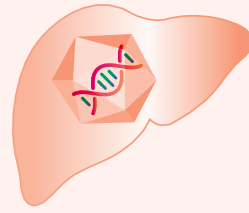
GENGLYCOS gene therapy is designed to address GSD1a at the genetic level



GENGLYCOS is a one-time intravenous (IV) infusion delivered in the arm. It is designed to deliver a working copy of the *G6PC* gene into the body. The *G6PC* gene gives instructions for the body to make G6Pase enzymes, which help regulate glucose levels.



The working copy of the *G6PC* gene is delivered to a specific part of the body using a special vehicle, or “vector,” called adeno-associated virus serotype 8 (AAV8).



The vector is intended to target liver cells, where most glucose and glycogen processing occurs. Once inside the liver cells, the new gene helps create G6Pase enzymes, which allow the body to release glucose when needed.



Learn more about GENGLYCOS

IMPORTANT SAFETY INFORMATION (continued)

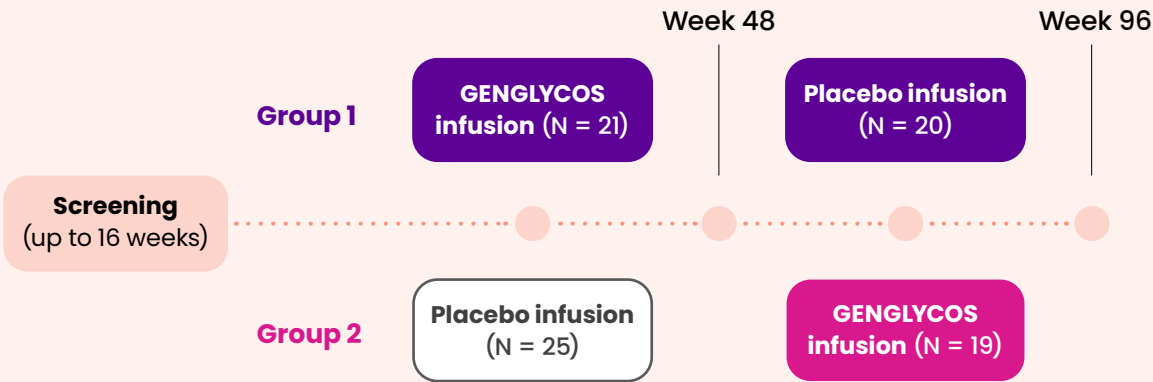
What is the most important information I should know about GENGLYCOS?

- Allergic and infusion reactions, including anaphylaxis, occurred with GENGLYCOS treatment. Some of these reactions have been severe. You will be closely monitored during and after infusion for signs and symptoms including hives, flushing, low blood pressure, wheezing, trouble breathing, chest tightness, nausea, vomiting, headache, stomach pain, lightheadedness, flu-like symptoms, shivering, rash, and high blood pressure. If you think you’re having a reaction to the infusion, contact your healthcare provider immediately.

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How was GENGLYCOS studied?

The efficacy of GENGLYCOS was studied in 46 study participants aged 8 years and older with GSD1a



- Participants were split up into 2 groups: Group 1 (N = 21) and Group 2 (N = 25)
 - The “N” represents the number of participants in each group
- Participants received 2 infusions during the study: 1 GENGLYCOS infusion and 1 placebo infusion
- Results were collected at week 48 to assess the effects of GENGLYCOS, and patients were followed up with at week 96
- All participants and treating doctors were blinded in the study. This means they didn’t know who received a placebo infusion or a GENGLYCOS infusion at the beginning of the study

IMPORTANT SAFETY INFORMATION (continued)

- GENGLYCOS can cause certain liver enzymes to increase. You will receive oral corticosteroids with or without other medications after your infusion and will receive regular blood tests to monitor your liver function. Contact your healthcare provider right away if you experience unusual tiredness, yellowing of your skin and/or eyes, dark urine, nausea, vomiting, or pain in the upper right side of your stomach area, or if you miss a dose of your corticosteroid or vomit after taking it.
- Do not take any medicines, including herbal products or nutritional supplements, before confirming with your healthcare provider that they are not harmful to the liver.
- Talk to your healthcare provider about the possible risks of drinking alcohol following GENGLYCOS infusion.



Less daily cornstarch with GENGLYCOS

The main goal of the study was to see if GENGLYCOS could reduce average total daily cornstarch consumption compared with placebo at week 48

At week 48, Group 1 saw greater average reductions in daily cornstarch consumption compared with Group 2 from the start of the study. Before receiving their first infusion, the average amount of cornstarch consumed per day was 294 grams for Group 1 and 272 grams for Group 2.

Group 1 (N = 21) **41%** ↓
less daily cornstarch

Group 2 (N = 25) **10%** ↓
less daily cornstarch

Those in Group 1 consumed, on average, 41% less cornstarch per day, compared with 10% less in Group 2.

Further reductions in average daily cornstarch consumption were seen in Group 1 at **week 96**.

Group 1 (n = 15*) **61%** ↓
less daily cornstarch

Those in Group 1 consumed an average of 61% less cornstarch per day from the start of the study.

*This “n” value reflects the number of study participants from Group 1 who were available during the time of data collection at week 96.

Cornstarch reduction in the clinical study

- At the start of the study, the average cornstarch consumption for Group 1 was nearly **300 grams per day**
- For this group, 41% less cornstarch means about **120 fewer grams per day**
- This means that after 48 weeks, their average daily consumption was about **180 grams**

What could less cornstarch look like for you?

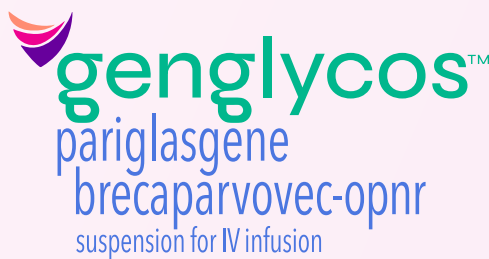
IMPORTANT SAFETY INFORMATION (continued)

- Problems with adrenal glands, including serious events, have occurred in patients receiving GENGLYCOS during corticosteroid treatment. Signs of adrenal gland problems can include unusual tiredness, weakness, loss of appetite, nausea, vomiting, low blood pressure, low sodium levels in the blood, and low blood sugar. A severe form called adrenal crisis can cause very low blood pressure, sudden severe stomach pain, or loss of consciousness. You will be monitored for signs and symptoms of adrenal gland problems. Do not stop taking your corticosteroid suddenly—follow your provider’s instructions.

Please see inside pages and full [Prescribing Information](#) for Important Safety Information.

IMPORTANT SAFETY INFORMATION (continued)

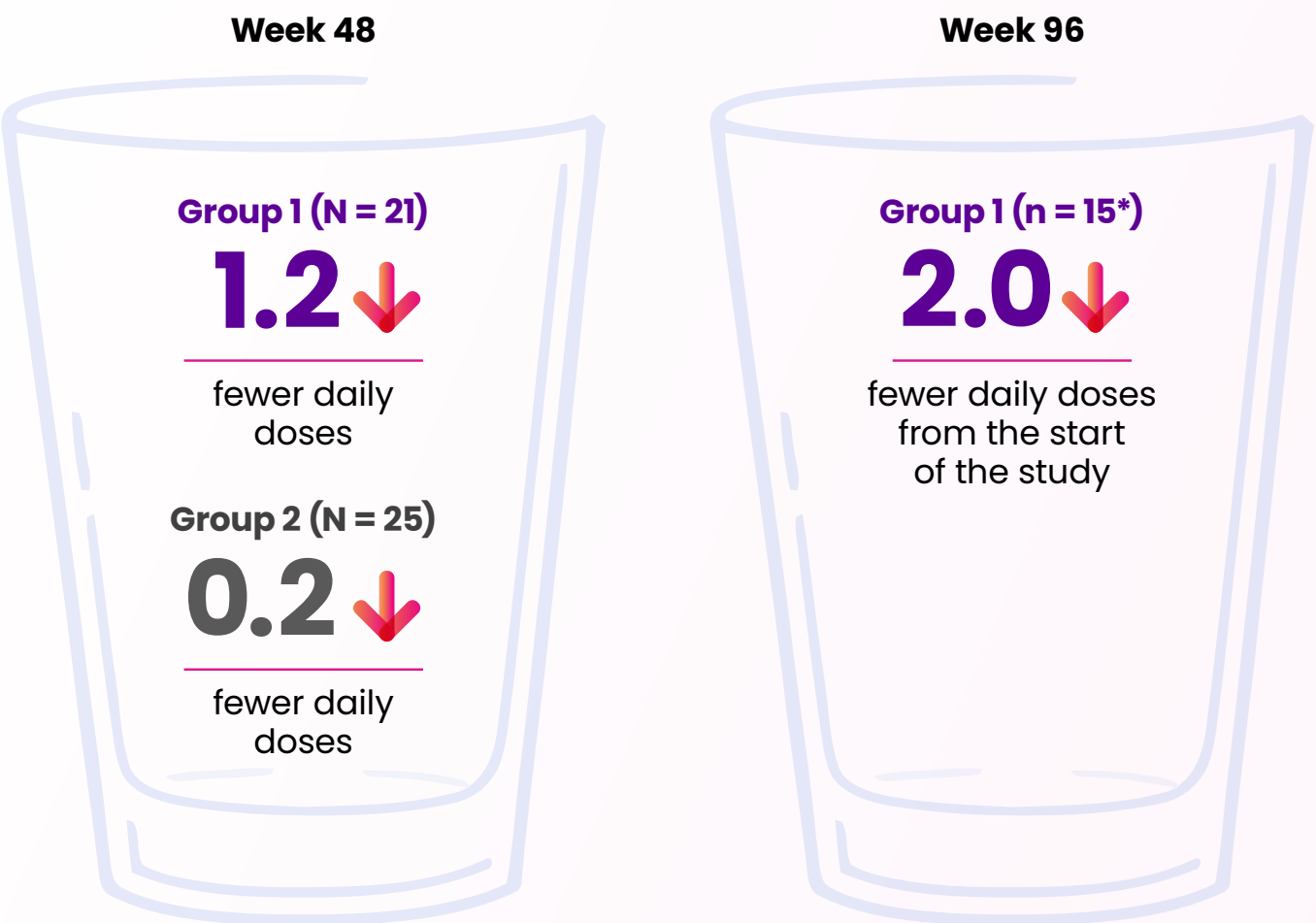
- There is a theoretical risk of tumor growth with gene therapies like GENGLYCOS; no cases have been reported in GENGLYCOS clinical studies. Contact your healthcare provider and Ultragenyx Pharmaceutical Inc. at **1-888-756-8657** if a tumor develops.



Fewer daily cornstarch doses

The average number of daily doses was reduced in Group 1 at week 48, and further reductions were seen at week 96

At the beginning of the study, the average number of daily cornstarch doses was 5.8 for Group 1 and 5.1 for Group 2.



*This "n" value represents the number of study participants from Group 1 who were available during the time of data collection at week 96.

IMPORTANT SAFETY INFORMATION (continued)

Are there any considerations for vaccination schedules and GENGLYCOS?

- Talk to your healthcare provider to determine if your vaccination schedule needs to be adjusted to accommodate corticosteroid and other medication administration prior to and after GENGLYCOS infusion.
- Vaccines should be avoided 1 month prior to GENGLYCOS administration.

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Similar blood glucose results

Similar hypoglycemic blood glucose values were seen in both Group 1 and Group 2 at week 48

Over a 4-week period during the study, blood glucose readings from continuous glucose monitors were collected and analyzed to determine how often participants reported blood glucose values in the hypoglycemic range (<70 mg/dL). These readings were compared against the percentage of values in the hypoglycemic range at the start of the study (Group 1 = 2.5% and Group 2 = 1.9%).

Week	Group	Participants	Percentage in Hypoglycemic Range
Week 48:	Group 1	N = 21	+3.1%
	Group 2	N = 25	+0.1%
Week 96:	Group 1	n = 19*	+5.2%

IMPORTANT SAFETY INFORMATION (continued)

What do I need to know about pregnancy and breastfeeding with GENGLYCOS?

- It is not known if GENGLYCOS can harm your baby or affect your ability to become pregnant. GENGLYCOS should not be used during pregnancy.
- It is not known if GENGLYCOS passes into breast milk. Talk to your healthcare provider about the best way to feed your baby if you take GENGLYCOS.



GENGLYCOS safety

The safety of GENGLYCOS was evaluated in 46 study participants

Seven serious side effects were observed in a GENGLYCOS study, including anaphylaxis/infusion reaction (2), adrenal gland problems (2), high lactate level (2), and low blood sugar (1).

The table below shows the most common side effects, which occurred in ≥10% of study participants from Groups 1 and 2 (N = 46) during the first 48 weeks.

Most common side effects	GENGLYCOS (N = 21)	Placebo (N = 25)
Increased liver enzymes	71%	12%
Nausea	38%	16%
Headache	24%	12%
High triglycerides*	29%	8%
Adrenal gland problems†	24%	0%
Constipation	19%	0%
High blood glucose	14%	0%
Acne or acne-like skin rash	19%	0%
Cushingoid features‡	14%	0%
Severe allergic reaction	10%	0%

*High levels of a type of fat in the blood.

†Including low adrenal hormone levels.

‡Changes caused by high steroid levels such as rounded face, weight gain, or increased body fat.

Safety was also studied through week 96, and in a separate study of 12 adult participants with GSD1a who were followed for 52 weeks. No additional side effects were seen.

Additional safety considerations



Allergic and infusion reactions

Allergic and infusion reactions, including anaphylaxis, occurred with GENGLYCOS treatment. Some of these reactions have been severe.

You will be closely monitored during and after infusion for signs and symptoms including hives, flushing, low blood pressure, wheezing, trouble breathing, chest tightness, nausea, vomiting, headache, stomach pain, lightheadedness, flu-like symptoms, shivering, rash, and high blood pressure.



Elevated liver enzymes

GENGLYCOS can cause certain liver enzymes to increase.

You will receive oral corticosteroids with or without other medications after your infusion and will receive regular blood tests to monitor your liver function. Contact your healthcare provider right away if you experience unusual tiredness, yellowing of your skin and/or eyes, dark urine, nausea, vomiting, or pain in the upper right side of your stomach area, or if you miss a dose of your corticosteroid or vomit after taking it.



Effect on adrenal glands

Problems with adrenal glands, including serious events, have occurred in patients receiving GENGLYCOS during corticosteroid treatment.

Signs of adrenal gland problems can include unusual tiredness, weakness, loss of appetite, nausea, vomiting, low blood pressure, low sodium levels in the blood, and low blood sugar. A severe form called adrenal crisis can cause very low blood pressure, sudden severe stomach pain, or loss of consciousness.



Inform your doctor if you experience any side effects

These are not all the possible side effects of GENGLYCOS. Talk to your healthcare provider about any side effects that bother you or don't go away.

Starting your GENGLYCOS journey

Once you decide on treatment with GENGLYCOS, your doctor will schedule a few tests to determine if you're eligible.



AAV8 antibody test

AAV8 is the vector used to deliver GENGLYCOS into your liver cells. GENGLYCOS is not for use in individuals who have detectable AAV8 antibodies.

AAV8 is a common virus among the general population, with some studies estimating that approximately 20% to 30% of people may have naturally developed antibodies. If you have detectable AAV8 antibodies, it simply means that you were exposed to AAV8 at some point in your life.



Liver function test

Your doctor will check your liver function before administering GENGLYCOS. Individuals with certain forms of liver damage, such as severe fibrosis or cirrhosis, are not eligible for treatment.



Adrenal gland test

Your doctor will check your adrenal glands and certain hormone levels before administering GENGLYCOS.



Other tests of metabolic health

Your doctor may request other tests to gather a better understanding of your metabolic health before administering GENGLYCOS.



Vaccines

Vaccines should be avoided 1 month prior to GENGLYCOS administration.

IMPORTANT SAFETY INFORMATION (continued)

What do I need to know about contraception and sperm donation with GENGLYCOS?

- Women of childbearing potential should use an effective method of contraception for at least 12 months after administration of GENGLYCOS.
- For 6 months after GENGLYCOS administration, men should not donate sperm, and men capable of fathering a child and their female partner should avoid pregnancy and use an effective form of birth control.

Please see inside pages and full [Prescribing Information](#) for Important Safety Information.

What to expect on infusion day

GENGLYCOS is a one-time intravenous (IV) infusion that takes around 3 hours

Before infusion



Your care team will walk you through the treatment process and give you acetaminophen and nondrowsy antihistamines 30 to 60 minutes before the infusion.

During infusion



GENGLYCOS will be infused slowly into a vein at a Qualified Treatment Center. This is a healthcare institution with care teams that specialize in gene therapy.

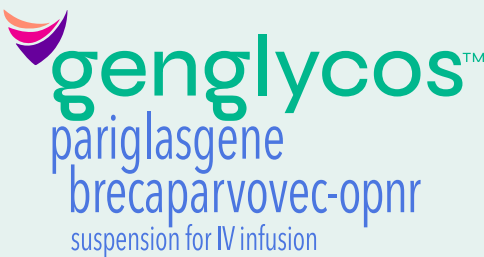


As with any medical procedure, your care team will closely monitor you during your infusion for any side effects. If an infusion reaction occurs, your doctor may pause the treatment and care will be provided as appropriate. **Possible signs and symptoms of infusion reactions may include hives, flushing, low blood pressure, wheezing and coughing, shortness of breath, chest tightness, nausea, vomiting, headache, abdominal pain, lightheadedness, flu-like symptoms, shivering, rash, and high blood pressure.**

IMPORTANT SAFETY INFORMATION (continued)

Are there any precautions that need to be considered when handling a patient's bodily waste?

- Small amounts of GENGLYCOS can travel through the patient's blood and be temporarily passed through urine, stool, and saliva. Talk to your healthcare provider about proper hygiene when handling patient body waste. Precautions should be followed for 3 months after GENGLYCOS infusion.



After-infusion care

Work with your care team on a plan for monitoring and tracking your progress



Your doctor will prescribe a daily corticosteroid starting 2 weeks after infusion and lasting at least 8 weeks

- Corticosteroids are given as immunosuppressive therapy with the goal of reducing potential side effects of treatment with GENGLYCOS
- Increased liver enzymes are a common immune system response and may be seen as your body adjusts to the gene therapy



Your doctor will perform regular liver function tests for the first few months after treatment:

- Weekly while you're taking corticosteroids and for 2 weeks after you finish
- Every 2 to 4 weeks until month 6, and as needed after month 6



Your doctor will perform regular adrenal gland function tests:

- Before and after treatment with GENGLYCOS, and when requested by your doctor

IMPORTANT SAFETY INFORMATION (continued)

What are the possible or likely side effects of GENGLYCOS?

Seven serious side effects were observed in a GENGLYCOS study, including anaphylaxis/infusion reaction (2), adrenal gland problems (2), high lactate level (2), and low blood sugar (1).

The most common side effects that occurred in individuals treated with GENGLYCOS in a study were:

- | | |
|--|--|
| • Increased liver enzymes | • Constipation |
| • Nausea | • High blood sugar |
| • Headache | • Acne or acne-like skin rash |
| • High triglycerides (high levels of a type of fat in the blood) | • Cushingoid features (changes caused by high steroid levels such as rounded face, weight gain, or increased body fat) |
| • Low adrenal hormone levels or adrenal gland problems | • Anaphylaxis (severe allergic reaction) |

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Ongoing nutritional management

Continue following up with your care team to create a GSDIa nutritional management plan that fits your unique needs



Dietary adjustments

As your body adjusts to treatment with GENGLYCOS, your doctor may need to make changes to meet your nutritional goals, such as:

- Adjusting your daily cornstarch intake and/or frequency
- Making modifications to your meal plan
- Changing the vitamin or mineral supplements that you take



Blood tests and monitoring

Continue monitoring your blood glucose levels via finger sticks and/or a continuous blood glucose monitor.

- Regular monitoring helps you to understand your body's response to adjustments after GENGLYCOS
- Your doctor may recommend certain blood tests to assess your overall metabolic health

IMPORTANT SAFETY INFORMATION (continued)

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You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.



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UltraCare Patient Services can help throughout your journey

Get assistance from experienced professionals who can answer your questions regarding treatment access, logistical challenges, and more.



Understand
insurance coverage



Determine your eligibility
for financial assistance



Navigate
travel logistics



Access available
resources

Our UltraCare Gene Therapy team is here to support you before, during, and after treatment.

Learn more at UltraCareSupport.com



Please see inside pages and full [Prescribing Information](#) for Important Safety Information.

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suspension for IV infusion