PBGM01: Investigational Gene Therapy for Infantile GM1 Gangliosidosis

PBGM01 uses a virus (AAV) to deliver a functional copy of the gene (GLB1) that is defective in GM1 gangliosidosis to the brain, liver, heart and other organs in children with early and late infantile GM1.

Once the gene is delivered into the cell, the goal is to have the gene make β-galactosidase to prevent build-up of gangliosides in the tissues.

For more information:

For Patients and Caregivers: patientservices@passagebio.com

For Healthcare Professionals: medinfo@passagebio.com

How Does Gene Therapy Work?

1. A normal healthy copy of the GLB1 gene is produced.
2. Gene is inserted into an Adeno-Assiated Virus (AAV) to create a viral vector.
3. AAV vector is injected into the cerebrospinal fluid (CSF) that flows around the brain and spinal cord.
4. Some cells take up AAV vector and begin to make functional β-galactosidase enzyme, which is released into the CSF.
5. Secreted β-galactosidase enzyme can be used by other cells throughout the brain and body which may slow or stop progression of GM1.

www.PassageBio.com
How is PBGM01 administered?

PBGM01 is administered through an injection into the space just below the base of the brain called the cisterna magna.

The goal of this approach is to maximize delivery to the brain.

What is Required to Participate in this Phase 1/2 Trial?

<table>
<thead>
<tr>
<th>Age</th>
<th>Early Onset Infantile (Type 1 GM1)</th>
<th>Late Onset Infantile (Type 2a GM1)</th>
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<tbody>
<tr>
<td>Onset of Symptoms</td>
<td>Before 6 months</td>
<td>6 – 18 months (12 – 36 months for cohort 1)</td>
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Developmental Requirement

- At least 2 of the following milestones:
  - Shows ability to move arms and legs intentionally
  - Look at object for at least 3 seconds
  - Ability to roll head
  - Vocalizes with intention
  - Able to track with eyes
  - Interacts with caregiver

Disease Confirmation

- Genetic confirmation
- Low blood β-galactosidase activity

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Where can I find out more about the PBGM01 trial?

- We have dosed our first patient and are continuing to recruit participants.
- Planned site locations include United States, Brazil, Canada, Turkey, and the United Kingdom.
- For full details about this program, please visit ClinicalTrials.gov #NCT04713475

If you are interested in possibly participating, please complete the referral form at [www.pbgm01.com](http://www.pbgm01.com)

No-Cost GM1 genetic testing available at Invitae.com