

Product Summary

Human Gaucher Type 1 iPSCs

Catalog Number: CR1009-500

Product Overview				
Product Name	Human Gaucher Type 1 iPS Cells			
Catalog #s	CR1009-500			
Quantity	One vial (approx. 500,000 cells)			
Product Form	Frozen			
Cell Type	Disease Model iPSCs - Human Gaucher Type 1			
Reagents Needed	- Antibiotic - Penicillin/Streptomycin/Amphotericin B solution or Penicillin/Streptomycin solution, 100x (not included)¹ - Basement membrane matrix suitable for adherent cells – based on customer preference, we recommend using Geltrex™ hE Qualified Ready-to-Use, Reduced Growth Factor Basement Membrane Matrix manufactured by Thermo Fisher Cat. A156960 - 70% isopropanol solution - ROCK Inhibitor Y-27632 (Dihydrochloride) – based on customer preference - Cell disassociation reagent – based on customer preference, we recommend using Gibco™ Versene Solution (Cat. 15040066 - STEMCELL Technologies Gentle Cell Disassociation Reagent (Cat. 100-0485)			

Product Description

Human Gaucher's Type 1 iPSCs

Our Gaucher's Type 1 iPSC line is derived from a 1-year-old male donor of African American descent diagnosed with Gaucher's Type 1, the most common lysosomal storage disorder. A defective glucocerebrosidase (GCase) enzyme causes this disease by impairing the breakdown of glucocerebroside, a sphingolipid normally processed within lysosomes. As a result, glucocerebroside accumulates in tissues such as the spleen, liver, kidneys, lungs, brain, and bone marrow, leading to progressive neurological complications, hepatosplenomegaly, anemia, and bone abnormalities [i].

This iPSC line provides a valuable platform for studying Gaucher disease mechanisms, screening potential treatments, and developing gene therapy strategies. Using our patented episomal reprogramming method, we have generated a high-fidelity, non-integrating iPSC line that maintains genomic integrity while ensuring consistent, efficient reprogramming. We recommend culturing this cell line with our Human iPSC Growth Media (MR1001-K).

We enhance clinical safety by excluding Myc and Lin28 transcription factors from reprogramming, as they are linked to neoplastic formation [iii]. This approach lowers the clinical risk profile of downstream differentiated cells, making this iPSC line an ideal model for Gaucher disease research, drug discovery, and regenerative medicine.

Key Features & Quality Control:

- Glucocerebrosidase-deficient iPSC line validated for pluripotency
- Non-integrating episomal reprogramming for genomic stability
- Mycoplasma-free and pathogen-free certification
- Cryopreserved at low passage to ensure high post-thaw viability

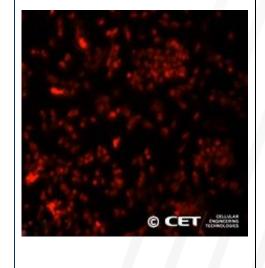
Applications of Gaucher's Type 1 iPSCs:

- Disease modeling for lysosomal storage disorders
- Drug screening for glucocerebrosidase enzyme replacement and chaperone therapy
- Gene therapy development for precision medicine applications
- Differentiation into macrophages, hepatocytes, and neuronal models

Specifications:

- Cell Type: Human induced pluripotent stem cells (iPSCs)
- Donor Information: 1-year-old male, African American descent

Cell Image



FOR RESEARCH APPLICATIONS ONLY. NOT FOR DIAGNOSTIC OR THERAPEUTIC USE.

- Reprogramming Method: Non-integrating episomal DNA
- Mutation: GBA1 gene mutation (variant details available upon request)
- Culture Conditions: Feeder-free, compatible with standard iPSC growth media
- Storage & Shipping: Cryopreserved, shipped on dry ice

Each vial contains ~500,000 cryopreserved cells, providing a robust, reproducible model for lysosomal storage disease research and therapeutic advancements.

Note: This product is designed and tested to function with Cellular Engineering Technologies Inc. ("CET") product MR1001-K Human iPS Cell Growth Media (not included). Although investigators are welcome to use this product with other media formulations, CET cannot and will not guarantee this product's performance. Additionally, such use of third-party media with this product will void CET's warranty should they not function as indicated. Please refer to CET's Terms & Conditions, available at www.cet.bio.

Cell Characteristics				
Growth Properties	Adherent			
Donor Age	1 year old			
Ethnicity	African American			
Gender	Male			
Gene, Gene Mutation, Chromosomal Location	GBA, LEU483PRO (LEU444PRO), 1448T>C, 1q21			
Media Formulation Instructions (for MR1001-K Human iPSC Growth Media Kit <u>not included</u>)				
Defrosting the iPSC Growth Supplement	 Defrost the iPSC Growth Supplement at 4°C (the day before the media is prepared) and 5 mL of antibiotic/antimycotic solution (not included) in a 37°C water bath until the ice in the tubes is no longer visible. Never defrost the iPSC Growth Supplement in a 37°C water bath. It is normal for the iPSC Growth Supplement to appear hazy or have suspended solutes. Gently mix by inversion. Immediately disinfect the tubes and the bottle containing the iPSC Growth Base Media with 70% isopropanol (not included). 			
Mixing	 Working in a laminar flow hood, remove 12mL of iPSC Growth Base Media (not included with cells) from the bottle and discard. This and all other procedures must be done in a sterile manner. Add the complete contents of the iPSC Growth Supplement to the iPSC Growth Base Media. Add 5mL of the antibiotic/antimycotic solution to the iPSC Growth Base Media¹. Cap the bottle containing the mixed liquid solution and gently swirl a few times. This formulated media is now considered complete media and ready to use with cells. 			

Cell Thawing and Plating Instructions					
Cell thawing	 Before thawing the cells, substrate-coated dishes should be prepared accordingly. Thirty (30) minutes before thawing the iPS cells, the coating solution on the plates must be entirely replaced with complete media (see Media Formulation Instructions) containing five (5) uM ROCK Inhibitor Y-27632 (not included) and equilibrated to room temperature. Remove the Human Gaucher Type 1 iPSC vial from the dry ice or a storage unit. Defrost the vial of cells in a 37°C water bath with constant, moderate agitation until ice in the ampoule is barely visible. DO NOT OVERTHAW. Immediately disinfect with 70% isopropanol (not included). 				
Cell plating	 Working in a laminar flow hood, open the vial and transfer the contents to a sterile fifteen (15) mL tube. Very slowly, add approximately nine (9) mL of complete media (see Media Formulation Instructions) containing five (5) uM ROCK Inhibitor Y-27632, pre-warmed to 37°C before use. Centrifuge suspended cells at 200 x g for 10 minutes. Decant the medium and gently resuspend the pellet in 6 mL of complete media containing 5 uM ROCK Inhibitor Y-27632. Do this gently to avoid shearing the colonies. Gently pipette the resuspended cells onto the previously coated dishes. One vial of iPSCs contains enough colonies to seed six (6) wells of a standard six (6)-well tissue culture plate or three (3)- one-hundred (100) mm tissue culture dishes. Distribute the colonies evenly and gently rock the plate back and forth. Place the dish in an incubator at 37°C, 5% CO₂, and 95% humidity. After 24 hours, aspirate media from the dish and replenish with fresh complete media (WITHOUT 5uM ROCK Inhibitor Y-27632), pre-warmed to 37°C before use. Repeat media changes every 24 hours. 				

Observation and expansion

- The cells should attach over 24 hours. It is normal for these cells to grow slowly initially for one week after thawing and for some colonies to be shed during media changes.
- Subculture cells at a 1:6 split ratio using cell disassociation reagent (not included).

Storage and Stability					
	Storage Temperature	Storage Time			
Human Gaucher Type 1 iPSCs Cat. CR1009	Upon arrival, place the cells at a temperature below -130°C, preferably in liquid nitrogen vapor, until ready for use	12 months			
Human iPSC Growth Media Kit (not included) Cat. MR1003-K					
iPSC Growth Base Media	4°C	3 months			
iPSC Growth Supplement	-20°C	Not applicable (use entire contents)			
complete media (see Media Formulation Instructions)	2-8°C	Not applicable			
Avoid repeated freeze-thaw cycles for cells. Avoid re	peated exposure to room temperature and light for medic	а.			

¹ These solutions should be portioned in 5mL aliquots, stored at -20°C, and never frozen/thawed. Although antimycotics are unnecessary, CET highly recommends their usage for long-term cell culture. Antibiotics and antimycotics should not be used as an alternative to proper aseptic techniques.