Version Date: 2/26/2025

**Product Information Sheet**

# Human Cystinosis iPSCs

## Catalog Number: CR1011-500

| Product Overview | |
|---|---|
| **Product Name** | **Human Cystinosis iPS Cells** |
| Catalog #s | CR1011-500 |
| Quantity | One vial (approx. 500,000 cells) |
| Product Form | Frozen |
| Cell Type | Disease Model iPSCs - Human Cystinosis |
| Reagents Needed | – **Antibiotic** - Penicillin/Streptomycin/Amphotericin B solution or Penicillin/Streptomycin solution, 100x (not included)[1]<br>– **Basement membrane matrix** suitable for adherent cells – based on customer preference, we recommend using Geltrex™ hESC-Qualified Ready-to-Use, Reduced Growth Factor Basement Membrane Matrix manufactured by Thermo Fisher Cat. A1569601)<br>– 70% isopropanol solution<br>– **ROCK Inhibitor** Y-27632 (Dihydrochloride) – based on customer preference<br>– **Cell disassociation reagent** – based on customer preference, we recommend using Gibco™ Versene Solution (Cat. 15040066) or STEMCELL Technologies Gentle Cell Disassociation Reagent (Cat. 100-0485) |

## Product Description

**Cystinosis iPSCs**

Our Cystinosis iPSCs are derived from a 14-year-old male donor of Caucasian descent diagnosed with nephropathic cystinosis, a rare autosomal recessive lysosomal storage disorder. This condition results in the abnormal accumulation of cystine within lysosomes [i], leading to intracellular crystal formation and progressive organ dysfunction. Nephropathic cystinosis is the leading cause of Fanconi syndrome in children, a disorder that disrupts renal tubule function, causing excessive loss of essential nutrients such as carbohydrates, amino acids, potassium, and phosphates in the urine.

The underlying cause of this disorder is mutations in the CTNS gene (chromosome 17p13), which encodes cystinosin [ii], a critical transporter responsible for cystine efflux from lysosomes. This iPSC line provides an essential tool for studying cystinosis-related pathophysiology, drug screening, and potential gene therapy approaches.

Using our patented episomal reprogramming method, we have converted primary fibroblast cells into pluripotent stem cells. Our proprietary transcription factor mix and small molecule chemistry offer a safe, consistent, and efficient reprogramming system, minimizing insertional mutagenesis risks while maintaining high fidelity for disease modeling and regenerative applications. We recommend culturing these iPSCs with our Human iPSC Growth Media (MR1001-K).

To enhance clinical safety, we exclude Myc and Lin28 transcription factors, which are associated with neoplastic transformation [iii]. This ensures a lower clinical risk profile for downstream differentiation into renal, metabolic, and lysosomal disease models for cystinosis research, drug screening, and therapeutic development.

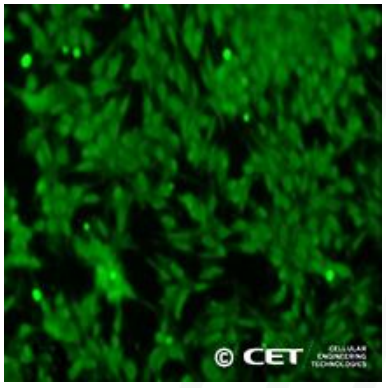
**Key Features & Quality Control:**

- CTNS-mutant iPSC line validated for pluripotency
- Non-integrating, virus-free episomal reprogramming for genomic stability
- Confirmed mycoplasma-free and pathogen-free
- Cryopreserved at low passage for high viability upon thawing

**Applications:**

- Disease modeling for nephropathic cystinosis and lysosomal storage disorders
- Drug discovery for cystine-depleting therapies and CTNS-targeted interventions
- Gene therapy development for personalized medicine
- Differentiation into renal tubule cells, metabolic cells, and other relevant tissues

## Cell Image



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**Specifications:**

- Cell Type: Human induced pluripotent stem cells (hiPSCs)
- Donor Information: 14-year-old male, Caucasian descent
- Reprogramming Method: Non-integrating episomal DNA
- Mutation: CTNS gene mutation (variant details available upon request)
- Storage & Shipping: Cryopreserved, shipped on dry ice

Each vial contains ~500,000 cryopreserved cells, ensuring high viability and reproducibility for disease research and therapeutic development.

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 | 14-year-old |
| Ethnicity | Caucasian |
| Gender | Male |
| Gene, Gene Mutation, Chromosomal Location | CTNS, DEL357GACT or 4-BP DEL, 18GACT (LEU444PRO), 17q13 |



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| Cell History | |
|---|---|
| Depositors | Coriell Institute for Medical Research |
| dbSNP ID | 20602 |
| Product ID | GM17886 |

| **Media Formulation Instructions** (for MR1001-K Human iPSC Growth Media Kit not included) | |
|---|---|
| Defrosting the iPSC Growth Supplement | 1. 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.<br>2. Immediately disinfect the tubes and the bottle containing the iPSC Growth Base Media with 70% isopropanol (not included). |
| Mixing | 1. 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.<br>2. 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 Media[1].<br>3. 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 | 1. Before thawing the cells, substrate-coated dishes should be prepared accordingly.<br>2. 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.<br>3. Remove the Human Cystinosis iPS Cells vial from the dry ice or a storage unit.<br>4. 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.<br>5. Immediately disinfect with 70% isopropanol (not included). |
| Cell plating | 1. Working in a laminar flow hood, open the vial and transfer the contents to a sterile fifteen (15) mL tube.<br>2. 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.<br>3. Centrifuge suspended cells at 200 x g for 10 minutes.<br>4. 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.<br>5. 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.<br>6. Distribute the colonies evenly and gently rock the plate back and forth. Place the dish in an incubator at 37°C, 5% $CO_2$, and 95% humidity.<br>7. 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.<br>8. 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.<br>– Subculture cells at a 1:6 split ratio using cell disassociation reagent (not included). |



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| Storage and Stability | | |
|---|---|---|
| | **Storage Temperature** | **Storage Time** |
| **Human Cystinosis iPSCs**<br>Cat. CR1011-500 | 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)<br>Cat. MR1003-K | | |
| iPSC Growth Base Media | 4°C | 3 months |
| iPSC Growth Supplement | -20°C | Not applicable (use entire contents) |
| complete media<br>*(see Media Formulation Instructions)* | 2-8°C | Not applicable |
| *Avoid repeated freeze-thaw cycles for cells. Avoid repeated exposure to room temperature and light for media.* | | |

[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.



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