TROY TECHNICAL

# iPS & REGENERATIVE MEDICINE

## WEEKLY REPORT

October 31 - November 6, 2026 | Issue 2026-W44

**Clinical evidence, manufacturability, reimbursement and scalable quality**

| 12 | 10 |
|---|---|
| ANALYZED ARTICLES | PRIORITY THEMES |

Issued 2026-11-06 | English edition | fixed approved cover artwork



# iPS & Regenerative Medicine: the boundary moved from isolated claims to qualification evidence

12 English articles were reviewed independently from the Japanese edition. Ten themes are retained for management decisions.

SIGNAL 01 | CELL SOURCE

## HSPC Gene Therapy: Automated Clinical-Scale Manufacturing via CCR5

Researchers have demonstrated a fully automated, high-throughput manufacturing platform for hematopoietic stem and progenitor cell (HSPC) gene therapy.

SIGNAL 02 | PROCESS & MATERIALS

## ATSN-301: Dual AAV.SPR-MYO7A Safety and Preclinical Development

Atsena Therapeutics has developed ATSN-301, a dual AAV.SPR vector system designed to treat Usher Syndrome Type 1B by reconstituting the full-length MYO7A protein.

SIGNAL 03 | QUALITY CONTROL

## Sumitomo Pharma: iPS Cell Therapy for Parkinson's Timeline

Sumitomo Pharma has performed the first transplant of induced Pluripotent Stem (iPS) cells to treat Parkinson's disease at Kyoto University Hospital.

SIGNAL 04 | CLINICAL USE

## Amsoki-cel (OBX-115): Phase 1/2 Efficacy in ICI-Resistant Melanoma

Amsoki-cel, an engineered TIL cell therapy using regulatable membrane-bound IL-15, achieved an objective response rate (ORR) of 66.7% in a key efficacy set of patients with ICI-resistant advanced melanoma.

DECISION

**Focus this week's management attention on clinical evidence, manufacturability, reimbursement and scalable quality. Move only when the linked evidence can be reproduced under your own operating conditions.**

THREE MANAGEMENT MOVES

1. Buyers: separate research findings from clinical readiness and define release, safety and follow-up gates.
2. Suppliers: connect materials, equipment and assays to repeatable cell quality and regulatory evidence.
3. Executives: track patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EVIDENCE DISCIPLINE

FACT states what the collected English source reports. BUSINESS READ and ACTION are editorial interpretations. UNKNOWN lists information that the source file does not establish. A linked article is not treated as independent confirmation of every claim.

# Five numbers or evidence anchors that require conditions

**66.7%**
A08

SOURCE-BOUND CONDITION
Amsoki-cel, an engineered TIL cell therapy using regulatable membrane-bound IL-15, achieved an objective response rate (ORR) of 66.7% in a key efficacy set of patients with ICI-resistant advanced melanoma.

**P02**
A11

SOURCE-BOUND CONDITION
Atsena Therapeutics has developed ATSN-301, a dual AAV.SPR vector system designed to treat Usher Syndrome Type 1B by reconstituting the full-length MYO7A protein.

**P03**
A12

SOURCE-BOUND CONDITION
Sumitomo Pharma has performed the first transplant of induced Pluripotent Stem (iPS) cells to treat Parkinson's disease at Kyoto University Hospital.

**P04**
A08

SOURCE-BOUND CONDITION
Amsoki-cel, an engineered TIL cell therapy using regulatable membrane-bound IL-15, achieved an objective response rate (ORR) of 66.7% in a key efficacy set of patients with ICI-resistant advanced melanoma.

**P05**
A09

SOURCE-BOUND CONDITION
Researchers have developed a single liver-sparing myotropic AAV that ameliorates muscular dystrophy in humanized mice using base editing.

HOW TO USE THESE NUMBERS
Each value is shown with the condition stated in the collected English article. Do not compare values across different test methods, device sizes, operating temperatures or commercial stages without normalizing those conditions.

## Where the value chain moved

Each stage combines one leading theme and one supporting theme. The report treats handoffs as evidence transfers, not decorative arrows.

1

### Cell source

P01/P06

Researchers have demonstrated a fully automated, high-throughput manufacturing platform for hematopoietic stem and progenitor.

2

### Process & materials

P02/P07

Atsena Therapeutics has developed ATSN-301, a dual AAV.SPR vector system designed to treat Usher Syndrome Type 1B by.

3

### Quality control

P03/P08

Sumitomo Pharma has performed the first transplant of induced Pluripotent Stem (iPS) cells to treat Parkinson's disease at.

4

### Clinical use

P04/P09

Amsoki-cel, an engineered TIL cell therapy using regulatable membrane-bound IL-15, achieved an objective response rate (ORR).

5

### Access & scale

P05/P10

Researchers have developed a single liver-sparing myotropic AAV that ameliorates muscular dystrophy in humanized mice using.

EVIDENCE REQUIRED AT EACH HANDOFF

| | |
|---|---|
| Cell source to Process & materials | Transfer test conditions, acceptance criteria, failure data, owner and decision date. Recheck patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement. |
| Process & materials to Quality control | Transfer test conditions, acceptance criteria, failure data, owner and decision date. Recheck patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement. |
| Quality control to Clinical use | Transfer test conditions, acceptance criteria, failure data, owner and decision date. Recheck patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement. |
| Clinical use to Access & scale | Transfer test conditions, acceptance criteria, failure data, owner and decision date. Recheck patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement. |

COMMON CONTROL POINT

**No theme moves to a commercial commitment until a named owner has reproduced the relevant evidence and documented patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.**

# Priority map: maturity and next decision locus




| | |
|---|---|
| P01 | HSPC Gene Therapy: Automated Clinical-Scale Manufacturing via CCR5<br>System / adoption \| cue: Clinical |
| P02 | ATSN-301: Dual AAV.SPR-MYO7A Safety and Preclinical Development<br>System / adoption \| cue: system |
| P03 | Sumitomo Pharma: iPS Cell Therapy for Parkinson's Timeline<br>Tech / process \| cue: no system/business cue |
| P04 | Amsoki-cel (OBX-115): Phase 1/2 Efficacy in ICI-Resistant Melanoma<br>System / adoption \| cue: Phase 1 |
| P05 | Myotropic AAV Base Editing: Muscular Dystrophy Treatment in Mice<br>Tech / process \| cue: no system/business cue |
| P06 | FASN-SREBP1 Axis: Potential Parkinson's Therapeutic Target<br>System / adoption \| cue: platform |
| P07 | hiPSC Mammary Gland Organoids: Functional Model for Breast Research<br>System / adoption \| cue: platform |
| P08 | Human Lymphatic Organoids: Developmental Routes and Function<br>System / adoption \| cue: platform |
| P09 | CAR-T Double Knockout Screen: NR4A1-SOCS3 Synergistic Pair<br>Tech / process \| cue: no system/business cue |
| P10 | Human iPSC-derived motor neurons: Rostrocaudal identity pipeline<br>Tech / process \| cue: no system/business cue |

**REPRODUCIBLE POSITIONING RULE**

Horizontal position uses five discrete stages: Research, Prototype, Joint evaluation, Product adoption and Scale. The vertical bands classify the next decision locus: Tech / process, System / adoption, or Business / rules (including supply systems). Article-title cues take precedence; the overview is used only when the title is silent. These are categories, not impact ranks. Bubble diameter reflects the editorial score.

FACT is source-bound. BUSINESS READ, ACTION and UNKNOWN are explicit editorial layers.

## P01 HSPC Gene Therapy: Automated Clinical-Scale Manufacturing via CCR5

A04 | Cell source | Pilot

2026-10-30

FACT

Researchers have demonstrated a fully automated, high-throughput manufacturing platform for hematopoietic stem and progenitor cell (HSPC) gene therapy. The system produces gene-edited HSPCs with an anti-HIV antibody transgene at the CCR5 locus while maintaining purity, differentiation potential, and in vivo function. This automation addresses the cost and scalability limitations of manual workflows to broaden patient access.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P01.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 21/25 | MATURITY 2/5 | DECISION LOCUS: SYSTEM / ADOPTION

## P02 ATSN-301: Dual AAV.SPR-MYO7A Safety and Preclinical Development

A11 | Process & materials | Pilot

2026-10-29

FACT

Atsena Therapeutics has developed ATSN-301, a dual AAV.SPR vector system designed to treat Usher Syndrome Type 1B by reconstituting the full-length MYO7A protein. A six-month GLP toxicology study established a No Observed Adverse Effect Level at 1.2E11 vg/eye, supporting the candidate's advancement toward clinical use.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P02.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 21/25 | MATURITY 2/5 | DECISION LOCUS: SYSTEM / ADOPTION

FACT is source-bound. BUSINESS READ, ACTION and UNKNOWN are explicit editorial layers.

## P03 Sumitomo Pharma: iPS Cell Therapy for Parkinson's Timeline

A12 | Quality control | Research

2026-10-31

FACT

Sumitomo Pharma has performed the first transplant of induced Pluripotent Stem (iPS) cells to treat Parkinson's disease at Kyoto University Hospital. The therapy, which received conditional approval in Japan in March, aims to restore dopamine-producing brain cells.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P03.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 21/25 | MATURITY 1/5 | DECISION LOCUS: TECH / PROCESS

## P04 Amsoki-cel (OBX-115): Phase 1/2 Efficacy in ICI-Resistant Melanoma

A08 | Clinical use | Research

2026-11-03

FACT

Amsoki-cel, an engineered TIL cell therapy using regulatable membrane-bound IL-15, achieved an objective response rate (ORR) of 66.7% in a key efficacy set of patients with ICI-resistant advanced melanoma. By eliminating the need for high-dose IL-2, the therapy aims to reduce systemic toxicity while maintaining anti-tumor activity.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P04.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 20/25 | MATURITY 1/5 | DECISION LOCUS: SYSTEM / ADOPTION

FACT is source-bound. BUSINESS READ, ACTION and UNKNOWN are explicit editorial layers.

## P05 Myotropic AAV Base Editing: Muscular Dystrophy Treatment in Mice

A09 | Access & scale | Pilot

2026-11-02

FACT

Researchers have developed a single liver-sparing myotropic AAV that ameliorates muscular dystrophy in humanized mice using base editing. This approach targets muscle tissue while avoiding the liver, providing a potential pathway for improving gene therapy precision in muscular dystrophy.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P05.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 19/25 | MATURITY 2/5 | DECISION LOCUS: TECH / PROCESS

## P06 FASN-SREBP1 Axis: Potential Parkinson's Therapeutic Target

A03 | Cell source | Research

2026-11-01

FACT

Phenotypic screening in patient-derived iPSC-dopamine neurons has identified the FASN-SREBP1 axis as a potential therapeutic target for Parkinson's disease (PD). The study demonstrates that inhibiting fatty acid synthase (FASN) restores intracellular calcium release and reduces α-synuclein release. These findings provide a tractable discovery platform for identifying targets that can be validated in vivo.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P06.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 19/25 | MATURITY 1/5 | DECISION LOCUS: SYSTEM / ADOPTION

FACT is source-bound. BUSINESS READ, ACTION and UNKNOWN are explicit editorial layers.

## P07 hiPSC Mammary Gland Organoids: Functional Model for Breast Research

A02 | Process & materials | Pilot

2026-10-30

FACT

Researchers have developed a stepwise differentiation protocol to derive functional mammary gland organoids (MGOs) from human induced pluripotent stem cells (hiPSCs). These MGOs exhibit polarized bilayered epithelium and respond to endocrine stimulation, providing a new platform for studying breast cancer pathogenesis and development.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P07.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 19/25 | MATURITY 2/5 | DECISION LOCUS: SYSTEM / ADOPTION

## P08 Human Lymphatic Organoids: Developmental Routes and Function

A06 | Quality control | Pilot

2026-11-05

FACT

Researchers have developed three-dimensional lymphatic organoids from human pluripotent stem cells using two distinct mesodermal routes. These organoids reconstruct functional lymphatic vessels capable of interstitial tracer transport and immune-cell interaction, providing a platform for disease modeling and tissue repair.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P08.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 19/25 | MATURITY 2/5 | DECISION LOCUS: SYSTEM / ADOPTION

FACT is source-bound. BUSINESS READ, ACTION and UNKNOWN are explicit editorial layers.

## P09 CAR-T Double Knockout Screen: NR4A1-SOCS3 Synergistic Pair

A07 | Clinical use | Pilot

2026-11-04

FACT

Researchers utilized a high-throughput in vivo CRISPR double-knockout (DKO) screen to identify gene pairs that enhance CAR-T cell efficacy. The NR4A1-SOCS3 DKO pair demonstrated superior antitumour potency and long-term tumour control by upregulating metabolic pathways and mitochondrial activity.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P09.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 19/25 | MATURITY 2/5 | DECISION LOCUS: TECH / PROCESS

## P10 Human iPSC-derived motor neurons: Rostrocaudal identity pipeline

A01 | Access & scale | Pilot

2026-10-30

FACT

Researchers have developed a unified pipeline to generate human induced pluripotent stem cell (iPSC)-derived motor neurons (iMNs) with defined hindbrain, cervical, and lumbar identities. By optimizing morphogen combinations and treatment windows, the study produced region-specific populations that exhibit enhanced electrical activity and synchronized network firing compared to conventional methods. This provides a reproducible cellular model for studying regional motor neuron development and selective degeneration.

WHY IT MATTERS

The decision relevance is clinical evidence, manufacturability, reimbursement and scalable quality. The signal should be tested at the application and operating-system level, not accepted from a headline alone.

BUSINESS READ

For operating companies, compare the reported change with current qualification, sourcing and product-roadmap assumptions. For suppliers, connect materials, equipment and assays to repeatable cell quality and regulatory evidence.

ACTION

Within 30 days, separate research findings from clinical readiness and define release, safety and follow-up gates; record the evidence and owner against P10.

UNKNOWN

Still unproven or incompletely stated: patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement.

EDITORIAL SCORE 19/25 | MATURITY 2/5 | DECISION LOCUS: TECH / PROCESS

# Playbook and 0-5 year roadmap

| STAKEHOLDER | NOW \| 0-30 DAYS | NEXT \| 1-12 MONTHS | LATER \| 1-5 YEARS |
|---|---|---|---|
| **Operating companies** | separate research findings from clinical readiness and define release, safety and follow-up gates | Run production-like qualification with explicit acceptance and stop criteria. | Build a portfolio and architecture that can absorb technology and supplier changes. |
| **Materials, equipment and technology suppliers** | connect materials, equipment and assays to repeatable cell quality and regulatory evidence | Create shared reliability, process-window and failure-analysis data with customers. | Standardize interfaces, traceability, lifecycle support and recovery services. |
| **Trading, investment and legal teams** | Map patient count, endpoints, comparators, adverse events, CMC consistency and reimbursement. | Contract for capacity, quality, change notice, liability, alternatives and exit. | Diversify regional, technical and customer concentration risk. |

ROADMAP

NOW | 0-30 DAYS
**Evidence ledger**
Owner: Strategy + quality
Deliverable: claim, source, condition, owner

NOW | 0-30 DAYS
**Risk screen**
Owner: Procurement + legal
Deliverable: unknowns, alternatives, stop

NEXT | 1-12 MONTHS
**Joint qualification**
Owner: R&D + supplier
Deliverable: process window and failure data

NEXT | 1-12 MONTHS
**Commercial gate**
Owner: Business + finance
Deliverable: unit economics and capacity

LATER | 1-5 YEARS
**Platform strategy**
Owner: Executive team
Deliverable: portfolio and interface roadmap

LATER | 1-5 YEARS
**Service layer**
Owner: Operations + channel
Deliverable: monitoring, maintenance and recovery

GO / NO-GO GATES

| GATE | OWNER | REQUIRED EVIDENCE | NO-GO CONDITION |
|---|---|---|---|
| **G1 Evidence** | **Quality** | Source, test conditions and reproducibility | No traceable evidence |
| **G2 Integration** | **R&D** | Interface, process window and failure analysis | Cannot meet system conditions |
| **G3 Commercial** | **Business** | Capacity, total cost, contract and alternatives | Economics or supply cannot be supported |
| **G4 Lifecycle** | **Operations** | Monitoring, maintenance, change notice and exit | No accountable operating plan |

## ANALYZED ARTICLES | ITEMS 01-12

Every title and URL is displayed in full. URLs wrap without shortening and remain clickable. A verified-reference label means the translated HTML did not retain its original source URL.

| ID | FULL ARTICLE TITLE | FULL SOURCE / VERIFIED REFERENCE URL - CLICKABLE |
|---|---|---|
| A01 | Human iPSC-derived motor neurons: Rostrocaudal identity pipeline | https://doi.org/10.64898/2026.10.27.761477 |
| A02 | hiPSC Mammary Gland Organoids: Functional Model for Breast Research | https://doi.org/10.64898/2026.10.28.762519 |
| A03 | FASN-SREBP1 Axis: Potential Parkinson's Therapeutic Target | https://doi.org/10.64898/2026.10.27.761977 |
| A04 | HSPC Gene Therapy: Automated Clinical-Scale Manufacturing via CCR5 | https://doi.org/10.64898/2026.10.28.758978 |
| A05 | Hypoimmune Limbal Stem Cells: Off-the-Shelf LSCD Therapy Proof-of-Concept | https://doi.org/10.21203/rs.3.rs-11142612/v1 |
| A06 | Human Lymphatic Organoids: Developmental Routes and Function | https://doi.org/10.64898/2026.11.03.764135 |
| A07 | CAR-T Double Knockout Screen: NR4A1-SOCS3 Synergistic Pair | https://doi.org/10.1038/s41586-026-10956-z |
| A08 | Amsoki-cel (OBX-115): Phase 1/2 Efficacy in ICI-Resistant Melanoma | https://doi.org/10.1038/s41591-026-04743-6 |
| A09 | Myotropic AAV Base Editing: Muscular Dystrophy Treatment in Mice | https://doi.org/10.1172/JCI202378 |
| A10 | NPC1 Base Editing Screens: Identifying Second-Site Corrector Mutations | https://doi.org/10.64898/2026.10.30.763201 |
| A11 | ATSN-301: Dual AAV.SPR-MYO7A Safety and Preclinical Development | https://doi.org/10.64898/2026.10.24.760972 |
| A12 | Sumitomo Pharma: iPS Cell Therapy for Parkinson's Timeline | https://www.philstar.com/lifestyle/health-and-family/2026/10/31/2566919/1st-patient-undergoes-pioneering-stem-cell-treatment-parkinsons |

# iPS_RegenerativeMedicine — Selected Articles

Date: 2026-11-08

Articles: 12

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#07 CAR-T Double Knockout Screen: NR4A1–SOCS3 Synergistic Pair

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#09 Myotropic AAV Base Editing: Muscular Dystrophy Treatment in Mice

#10 NPC1 Base Editing Screens: Identifying Second-Site Corrector Mutations

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# #01 Human iPSC-derived motor neurons: Rostrocaudal identity pipeline

Published October 30, 2026 bioRxiv USA



## OVERVIEW

Researchers have developed a unified pipeline to generate human induced pluripotent stem cell (iPSC)-derived motor neurons (iMNs) with defined hindbrain, cervical, and lumbar identities. By optimizing morphogen combinations and treatment windows, the study produced region-specific populations that exhibit enhanced electrical activity and synchronized network firing compared to conventional methods. This provides a reproducible cellular model for studying regional motor neuron development and selective degeneration.

## IN DEPTH

### Key Findings

The study establishes a three-protocol pipeline capable of reproducibly generating hindbrain/rhombomere-associated motor neurons (RiMNs), cervical motor neurons (CiMNs), and lumbar motor neurons (LiMNs). These populations were generated using iPSC lines from six non-diseased donors balanced by sex. Transcriptomic and protein analyses confirmed graded rostrocaudal specification and region-appropriate subtype features that align with human week 9 fetal rostrocaudal HOX programs.

### Technical Details

To overcome the limitations of conventional differentiation—which often results in heterogeneous HOX profiles and limited control over identity—the researchers optimized developmental morphogen combinations and treatment windows. The resulting regional iMN populations were evaluated using several analytical methods:

- **High-density multielectrode array (MEA) analysis:** Revealed synchronized network firing, electrically active neurite networks, and enhanced axonal signal propagation compared to conventionally differentiated iMNs.
- **Spike-sorted recordings:** Identified distinct electrophysiological profiles associated with each specific protocol.
- **Molecular profiling:** Confirmed the identity of RiMNs, CiMNs, and LiMNs through protein and transcriptomic analysis.

### Background and Context

Conventional methods for producing iMNs are efficient but lack the precision required to define the rostrocaudal axis. This lack of regional identity limits the ability of researchers to conduct anatomically resolved studies on how motor neurons develop or degenerate in specific areas of the spinal cord and brainstem.

## Significance and Outlook

This unified pipeline enables the comparative analysis of anatomically defined human motor neuron populations. By providing region-matched cellular models, the research supports more precise investigations into motor neuron development, the mechanisms behind selective degeneration, and the evaluation of therapeutic responses across different anatomical regions.

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Source: https://doi.org/10.64898/2026.10.27.761477

Collected: November 07, 2026 | Automated Research System (AI)

# #02 hiPSC Mammary Gland Organoids: Functional Model for Breast Research

Published October 30, 2026 bioRxiv



## OVERVIEW

Researchers have developed a stepwise differentiation protocol to derive functional mammary gland organoids (MGOs) from human induced pluripotent stem cells (hiPSCs). These MGOs exhibit polarized bilayered epithelium and respond to endocrine stimulation, providing a new platform for studying breast cancer pathogenesis and development.

## IN DEPTH

### Key Findings

The study establishes a method to reprogram human induced pluripotent stem cells (hiPSCs) into functional mammary organoids (MGOs). These organoids reproducibly form a polarized structure consisting of basal layers (KRT14+, TP63+) and luminal layers (KRT8+, GATA3+). Notably, approximately 25% of the luminal cells express the oestrogen receptor (ERα). The MGOs demonstrate functional maturity by accumulating lipids and expressing β-casein when stimulated with prolactin.

### Technical Details

The differentiation process follows a stepwise protocol in a defined medium, directing hiPSCs through the following stages:

- Induction of non-neural ectoderm
- Formation of mammary placodes
- Lineage maturation

Single-cell transcriptomics analysis confirms the presence of basal, luminal hormone-sensing, luminal secretory, and stromal populations. These populations align transcriptionally with adult human breast epithelium. Furthermore, the MGOs maintain lineage fidelity after serial passaging and are responsive to endocrine disruption. In vivo validation was conducted by transplanting MGOs into cleared mouse mammary fat pads, where they generated branched mammary ductal networks.

### Background & Context

The human mammary gland develops through sequential lineage restrictions, moving from surface ectoderm to a hormone-responsive bilayered epithelium. Despite this known biological pathway, a tractable in vitro model capable of capturing these specific developmental events had previously remained elusive.

## Significance & Outlook

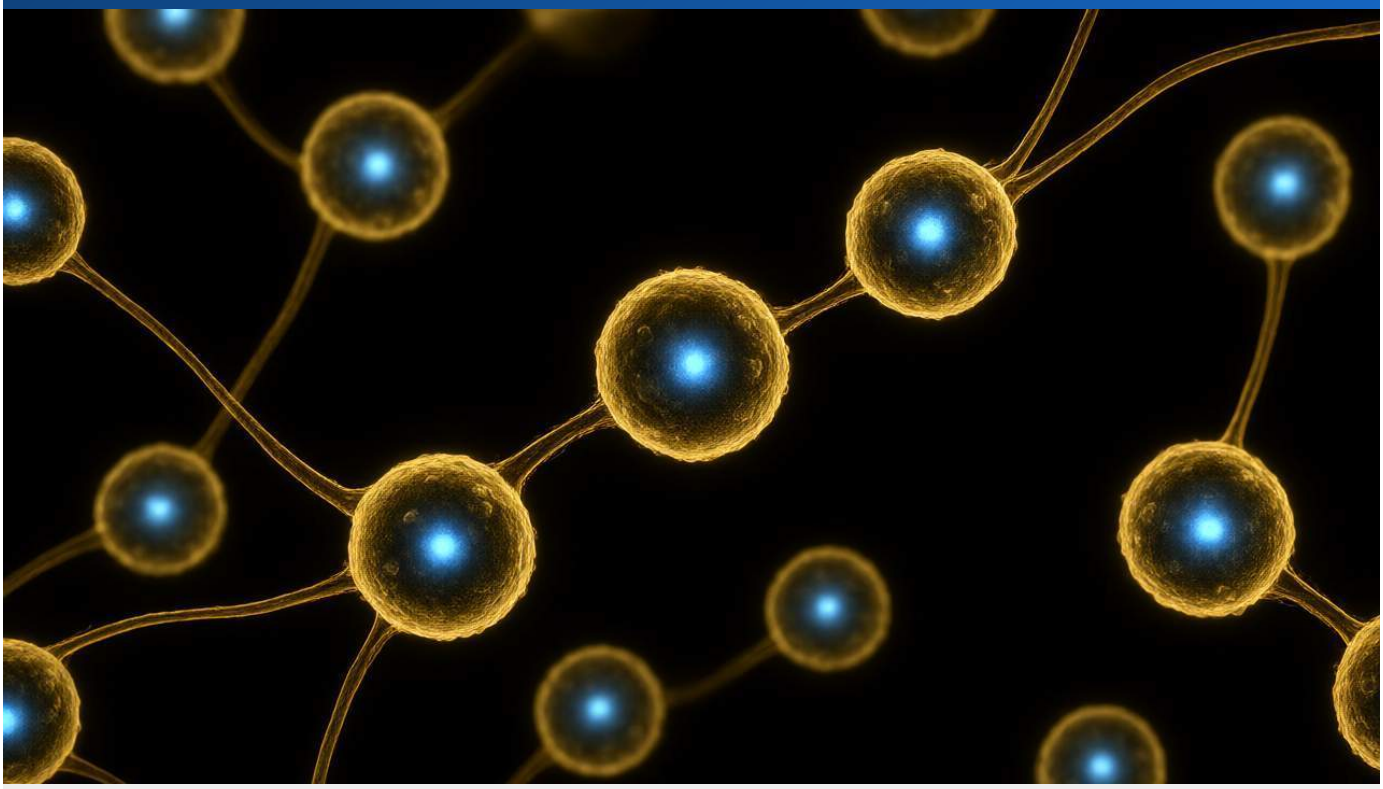
The ability to generate functional mammary epithelium from hiPSCs provides a scalable platform for biomedical research. This model allows researchers to dissect the complexities of mammary development and endocrine signaling. Additionally, it offers a controlled environment to study the mechanisms underlying breast cancer pathogenesis.

Source: https://doi.org/10.64898/2026.10.28.762519

Collected: November 07, 2026 | Automated Research System (AI)

# #03 FASN-SREBP1 Axis: Potential Parkinson's Therapeutic Target

Published November 01, 2026 bioRxiv



## OVERVIEW

Phenotypic screening in patient-derived iPSC-dopamine neurons has identified the FASN-SREBP1 axis as a potential therapeutic target for Parkinson's disease (PD). The study demonstrates that inhibiting fatty acid synthase (FASN) restores intracellular calcium release and reduces α-synuclein release. These findings provide a tractable discovery platform for identifying targets that can be validated in vivo.

## IN DEPTH

### Key Findings

Researchers have identified the FASN-SREBP1 axis as a candidate therapeutic pathway in Parkinson's disease. By conducting a phenotypic screen of 4,108 compounds in GBA-N370S (GBA-NS) induced pluripotent stem cell dopaminergic neurons (iPSC-DANs), the study identified fatty acid synthase (FASN) as the most enriched protein target. The results indicate that FASN inhibition increases intracellular calcium release, increases active β-glucocerebrosidase (GCase), and reduces both ER stress and extracellular α-synuclein release.

### Technical Details

The study utilized calcium dyshomeostasis as the primary phenotype for screening, as GBA-N370S iPSC-DANs exhibit impaired intracellular calcium release. The correlation between the ability of compounds to rescue calcium release and biochemical potency against FASN was confirmed across multiple chemical series. Mechanistically, FASN inhibition activated sterol regulatory element-binding protein 1 (SREBP1). The researchers found that SREBP1 overexpression alone was sufficient to increase intracellular calcium release, establishing a FASN-SREBP1-calcium homeostasis axis in human neurons.

To validate these findings in vivo, the FASN inhibitor BI 99179 was administered to a Parkinson's mouse model. The inhibitor achieved brain exposure, reduced brain fatty acid levels, and induced an SRE-associated transcriptional response.

### Background & Context

Modeling Parkinson's disease in vitro is challenging, particularly when seeking robust functional phenotypes for target discovery. Patient-derived iPSC-DANs offer a platform to address this, specifically by leveraging known pathophysiology such as calcium dyshomeostasis. Untargeted lipidomics were employed in this study to demonstrate broad remodelling of the neuronal lipidome following FASN inhibition, confirming target engagement.

## Significance & Outlook

This research establishes that functional screening in patient iPSC-neurons is a viable discovery platform for identifying targets that can be transitioned to in vivo models. By nominating the FASN-SREBP1 axis, the study provides a potential new direction for therapeutic intervention in PD, specifically targeting lipid-calcium regulatory mechanisms to rescue multiple pathogenic pathways.

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Source: https://doi.org/10.64898/2026.10.27.761977

Collected: November 07, 2026 | Automated Research System (AI)

# #04 HSPC Gene Therapy: Automated Clinical-Scale Manufacturing via CCR5

Published October 30, 2026 bioRxiv USA



## OVERVIEW

Researchers have demonstrated a fully automated, high-throughput manufacturing platform for hematopoietic stem and progenitor cell (HSPC) gene therapy. The system produces gene-edited HSPCs with an anti-HIV antibody transgene at the CCR5 locus while maintaining purity, differentiation potential, and in vivo function. This automation addresses the cost and scalability limitations of manual workflows to broaden patient access.

## IN DEPTH

### Key Findings

The study confirms that clinical-scale doses of gene-edited HSPCs (geHSPCs) can be produced using a fully automated, high-throughput manufacturing platform without compromising key product attributes. The process successfully integrated an anti-HIV antibody transgene at the CCR5 locus, achieving editing efficiencies comparable to small-scale processing. Furthermore, geHSPCs transplanted into mice demonstrated the ability to engraft and reconstitute multiple hematopoietic lineages, proving that repopulation capacity remains preserved after automated processing.

### Technical Details

The automated platform executes an end-to-end workflow designed for clinical-scale production. The sequence of operations includes:

- Post-thaw HSPC inoculation
- Cas9 electroporation
- AAV6 transduction
- Harvesting of the final product

The resulting geHSPCs exhibited high purity and preserved differentiation potential, meeting the necessary release criteria for therapeutic use.

### Background & Context

Gene therapies utilizing HSPCs offer curative potential for various hematologic diseases. However, the industry has relied heavily on manual manufacturing workflows, which are costly and difficult to scale. These inefficiencies create a bottleneck that limits patient access to these potentially curative treatments.

## Significance & Outlook

By transitioning from manual to automated, high-throughput manufacturing, this approach provides a path toward more cost-effective and scalable production of HSPC gene therapies. The ability to maintain in vivo function and product quality at a clinical scale suggests that automated platforms can overcome current manufacturing barriers, potentially increasing the availability of these therapies for a larger patient population.

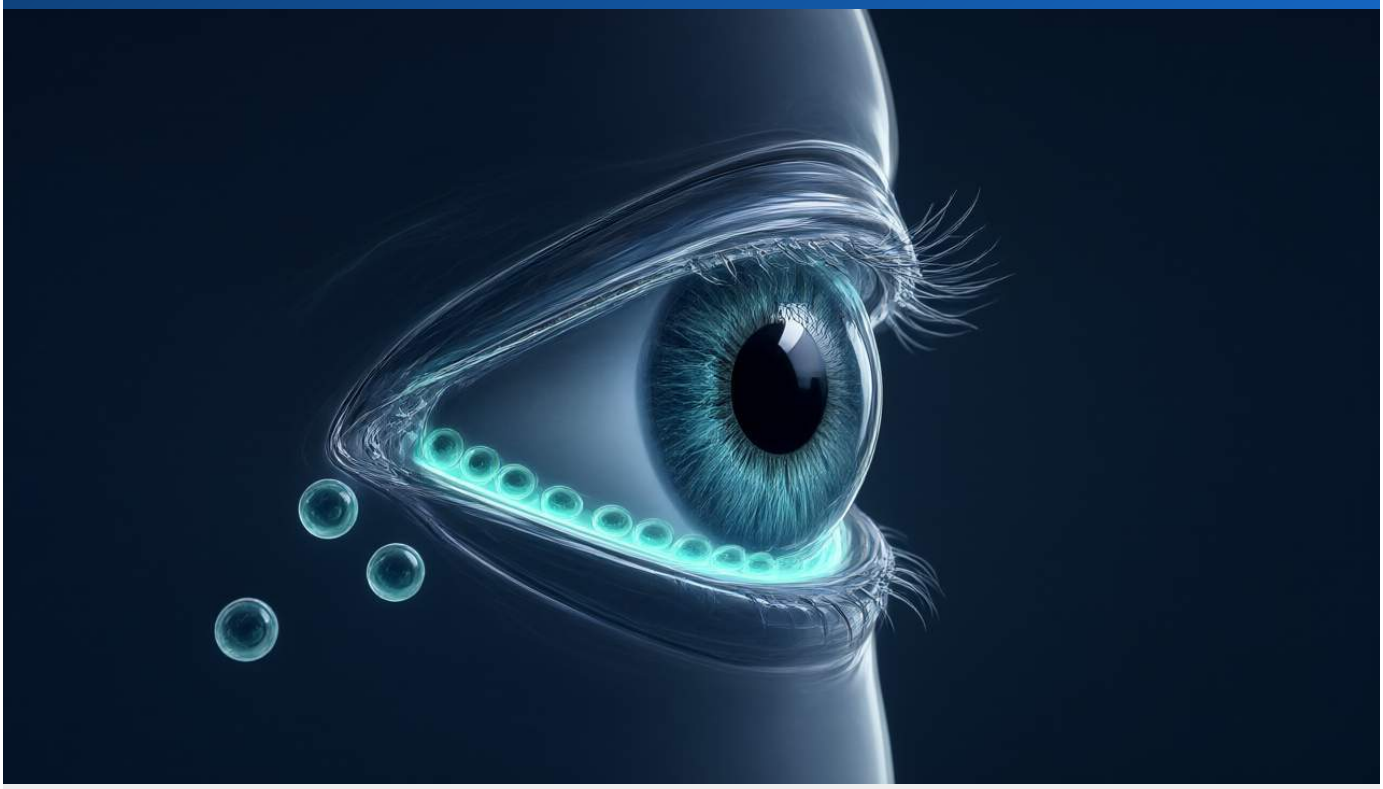
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Source: https://doi.org/10.64898/2026.10.28.758978

Collected: November 07, 2026 | Automated Research System (AI)

# #05 Hypoimmune Limbal Stem Cells: Off-the-Shelf LSCD Therapy Proof-of-Concept

Published October 30, 2026 Research Square



## OVERVIEW

Researchers have developed hypoimmune limbal stem cells (LSCs) engineered to evade allogeneic immune recognition while maintaining stem cell identity and differentiation capacity. The approach utilizes CRISPR-Cas9 and lentiviral modifications to eliminate the need for systemic immunosuppression in treating limbal stem cell deficiency (LSCD).

## IN DEPTH

### Key Findings

The study demonstrates an in vitro proof-of-concept for a universal, off-the-shelf allogeneic limbal stem cell (LSC) product. By engineering LSCs to evade immune detection, the researchers achieved complete in vitro evasion of $CD4^+$ and $CD8^+$ T cell responses and the abrogation of macrophage-mediated phagocytosis.

### Technical Details

The generation of these hypoimmune LSCs involved several specific genetic and cultural interventions:

- **Immune Evasion:** Simultaneous CRISPR-Cas9-mediated knockout of B2M and CIITA was combined with lentiviral CD47 overexpression.
- **Expansion:** Long-term culture supported by a ROCK inhibitor enabled stable LSC expansion exceeding 202 population doublings.
- **Validation:** The engineered cells preserved their transcriptomic identity and full differentiation competence despite the extensive expansion and genetic modifications.

### Background & Context

Limbal stem cell deficiency (LSCD) is a condition characterized by the irreversible loss of the limbal epithelial stem cell pool, leading to progressive corneal opacification and vision loss. Current treatment strategies face significant hurdles:

- **Allogeneic grafts:** These typically require long-term systemic immunosuppression and often exhibit poor long-term survival.
- **Autologous transplants:** While free from rejection, approximately one-third of these grafts fail, a result partially attributed to donor variability.

## Significance & Outlook

This framework provides a scalable method for treating both unilateral and bilateral LSCD. By reducing donor-dependent variability and eliminating the requirement for systemic immunosuppression, this hypoimmune LSC approach moves toward a standardized, off-the-shelf therapeutic product for patients suffering from high morbidity and vision loss associated with LSCD.

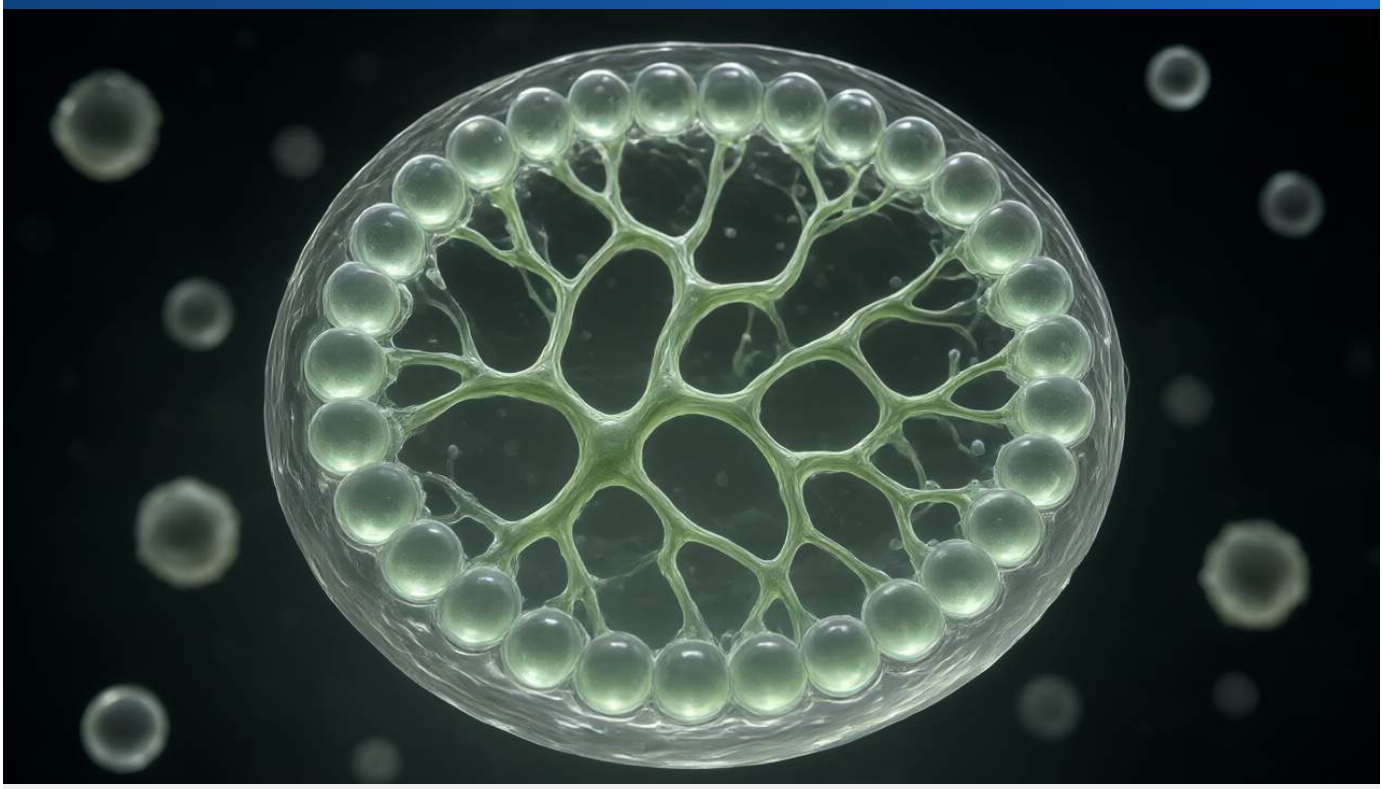
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Source: https://doi.org/10.21203/rs.3.rs-11142612/v1

Collected: November 07, 2026 | Automated Research System (AI)

# #06 Human Lymphatic Organoids: Developmental Routes and Function

Published November 05, 2026 bioRxiv USA



## OVERVIEW

Researchers have developed three-dimensional lymphatic organoids from human pluripotent stem cells using two distinct mesodermal routes. These organoids reconstruct functional lymphatic vessels capable of interstitial tracer transport and immune-cell interaction, providing a platform for disease modeling and tissue repair.

## IN DEPTH

### Key Findings

The study establishes a method to generate functional human lymphatic organoids from human pluripotent stem cells. By leveraging single-cell and spatial analyses of human embryos, the researchers identified that both lateral plate and paraxial mesoderm converge on venous endothelial intermediates with cardinal vein-like characteristics before lymphatic specification occurs.

### Technical Details

The development of these organoids followed two distinct mesodermal trajectories. The resulting three-dimensional structures exhibited the following characteristics:

- Formation of capillary-like vascular networks.
- Presence of emerging button-like junctions.
- Distinct endothelial and stromal programs.
- Transcriptional alignment with human fetal lymphatic endothelium.

Functional validation was conducted through both in vitro and in vivo testing. In vitro, the organoids supported antigen accumulation and immune-cell interactions. Following transplantation, the organoids demonstrated the ability to perform interstitial tracer uptake and transport.

### Background and Context

The lymphatic vasculature is essential for maintaining fluid balance, supporting tissue repair, and regulating immune surveillance. Despite its importance, the specific mechanisms by which human lymphatic vessels arise and acquire their specialized functions have remained poorly understood until the identification of these convergent developmental routes.

## Significance and Outlook

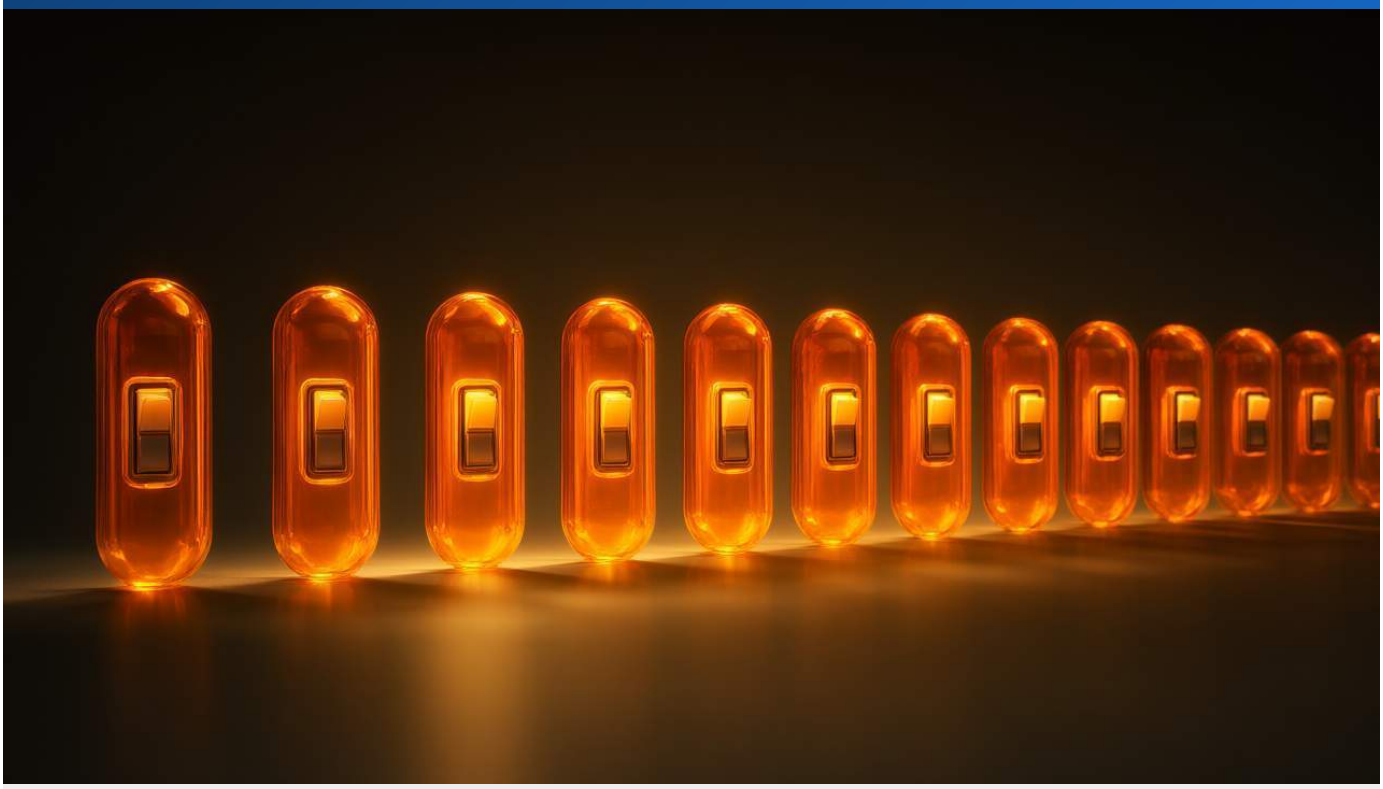
This platform allows for the investigation of how different developmental origins influence the specification, vascular organization, and overall function of human lymphatic vessels. These findings have direct applications in the fields of tissue repair and the modeling of lymphatic diseases.

Source: https://doi.org/10.64898/2026.11.03.764135

Collected: November 07, 2026 | Automated Research System (AI)

# #07 CAR-T Double Knockout Screen: NR4A1–SOCS3 Synergistic Pair

Published November 04, 2026 Nature



## OVERVIEW

Researchers utilized a high-throughput in vivo CRISPR double-knockout (DKO) screen to identify gene pairs that enhance CAR-T cell efficacy. The NR4A1–SOCS3 DKO pair demonstrated superior antitumour potency and long-term tumour control by upregulating metabolic pathways and mitochondrial activity.

## IN DEPTH

### Key Findings

The high-throughput in vivo CAR-T double-knockout (DKO) CRISPR screen identified multiple synergistic gene pairs that improve antitumour efficacy. The most effective combination identified is the NR4A1–SOCS3 DKO. This specific pair outperformed several benchmark single-gene and dual-gene modifications in both T cells and CAR-T cells.

### Technical Details

Analysis via whole-transcriptome profiling and in vivo single-cell RNA sequencing indicates that NR4A1–SOCS3 DKO specifically upregulates metabolic pathways. This results in CAR-T cells with significantly higher mitochondrial activity and metabolic fitness. These modified cells exhibited potent antitumour efficacy and long-term tumour control across various tumour models without compromising safety features.

The study utilized several genomic resources for data analysis, including:

- Human reference genome GRCh38 Gencode v44 (bulk RNA-seq) and v37 (single-cell RNA-seq).
- Human reference genome GRCh38 Gencode v47 and ENCODE blacklist regions (WGS).
- Molecular Signatures Database (MSigDB v7.4) and the ProjecTILs single-cell T cell atlas dataset.

Data including CRISPR screen sequencing, bulk and single-cell RNA-seq, CUT&RUN, and WGS have been deposited in the Gene Expression Omnibus (GSE272887, GSE335215) and the Sequence Read Archive (PRJNA1477126).

### Background & Context

While CAR-T cell therapy has transformed the treatment of haematological malignancies, its overall efficacy is limited by intrinsic negative regulatory mechanisms within T cells. This study addresses these limitations by employing a DKO screening approach to rapidly discover combination targets that can overcome these intracellular checkpoints.

## Significance & Outlook

The identification of NR4A1–SOCS3 as a promising pair of intracellular checkpoints provides a pathway for engineering high-performance CAR-T therapies. By demonstrating the utility of high-throughput in vivo DKO screening, this research establishes a method for the rapid discovery of synergistic gene targets to enhance cancer immunotherapy.

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Source: https://doi.org/10.1038/s41586-026-10956-z

Collected: November 07, 2026 | Automated Research System (AI)

# #08 Amsoki-cel (OBX-115): Phase 1/2 Efficacy in ICI-Resistant Melanoma

Published November 03, 2026 Nature Medicine USA



## OVERVIEW

Amsoki-cel, an engineered TIL cell therapy using regulatable membrane-bound IL-15, achieved an objective response rate (ORR) of 66.7% in a key efficacy set of patients with ICI-resistant advanced melanoma. By eliminating the need for high-dose IL-2, the therapy aims to reduce systemic toxicity while maintaining anti-tumor activity.

## IN DEPTH

### Key Findings

Pooled analysis from a completed phase 1 first-in-human (FIH) trial and an ongoing phase 1/2 Agni-01 trial showed that 10 out of 15 patients in the Key Efficacy Set (treated at the recommended phase 2 dose, or RP2D) achieved objective responses, representing an ORR of 66.7% (95% CI 38–88%), including two complete responses. In the Agni-01 phase 2 preliminary group, the ORR was 66.7% (6/9). No dose-limiting toxicities, treatment-related mortality, or requirements for ICU care were reported across the 37 treated patients.

### Technical Details

Amsoki-cel (OBX-115) is an autologous tumor-infiltrating lymphocyte (TIL) therapy engineered to express membrane-bound interleukin-15 (mbIL15). The expression is controlled by a carbonic anhydrase-2 (CA2) drug-responsive domain (DRD). In the basal state, the mbIL15-DRD fusion protein is degraded; however, the administration of acetazolamide (ACZ), a CA2 inhibitor, stabilizes the DRD and induces mbIL15 expression on the cell surface. This mechanism allows for pharmacologic regulation of cell expansion and persistence without the need for systemic interleukin-2 (IL-2).

### Background and Context

Standard non-engineered TIL therapies require high-dose intravenous IL-2 for expansion and efficacy, which often causes substantial clinical toxicity and limits patient eligibility. Amsoki-cel addresses this by using regulatable mbIL15, which results in a more robust manufacturing process and a T cell phenotype that is more stem-like and less terminally exhausted than conventional TILs. The study population consisted of patients with advanced melanoma who were resistant to immune checkpoint inhibitors (ICI).

## Significance and Outlook

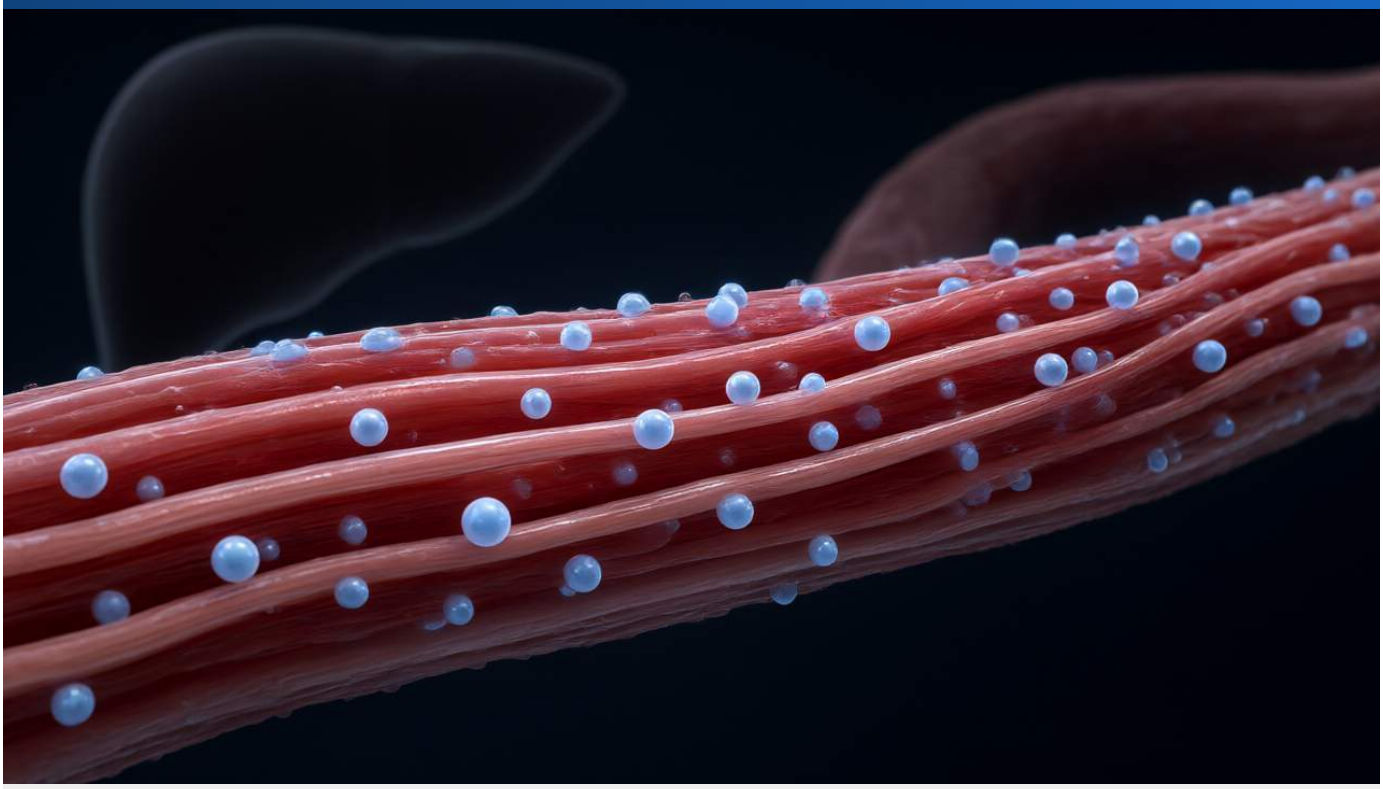
The most common grade 3 or higher treatment-emergent adverse events were neutropenia (64.9%), leukopenia/lymphopenia (56.8% each), and anemia/thrombocytopenia (37.8% each). While an early patient experienced vasogenic edema in a previously irradiated brain metastasis—leading to an 80% reduction in maximum amsoki-cel dose and a 75% reduction in ACZ dose—subsequent dose re-escalation demonstrated favorable safety. A separate phase 2 pivotal Agni-01 cohort is currently ongoing.

Source: https://doi.org/10.1038/s41591-026-04743-6

Collected: November 07, 2026 | Automated Research System (AI)

# #09 Myotropic AAV Base Editing: Muscular Dystrophy Treatment in Mice

Published November 02, 2026 The Journal of Clinical Investigation USA



## OVERVIEW

Researchers have developed a single liver-sparing myotropic AAV that ameliorates muscular dystrophy in humanized mice using base editing. This approach targets muscle tissue while avoiding the liver, providing a potential pathway for improving gene therapy precision in muscular dystrophy.

## IN DEPTH

### Key Findings

A study published in The Journal of Clinical Investigation demonstrates that a single administration of a liver-sparing myotropic AAV can ameliorate muscular dystrophy in humanized mice. The treatment utilizes base editing to achieve these results, specifically focusing on muscle-directed delivery to reduce off-target effects in the liver.

### Technical Details

The research involves the use of a myotropic AAV (Adeno-Associated Virus) vector designed to be liver-sparing. This engineering ensures that the base editing machinery is delivered preferentially to muscle tissues rather than accumulating in the liver. The study was conducted using humanized mice models to evaluate the efficacy of the base editing approach in treating muscular dystrophy.

### Background & Context

The research was a collaborative effort involving multiple institutions, including the University of Texas Southwestern Medical Center (Department of Molecular Biology, Hamon Center for Regenerative Science and Medicine, and Senator Paul D. Wellstone Muscular Dystrophy Specialized Research Center), Harvard Medical School (Grousbeck Gene Therapy Center, Schepens Eye Research Institute, and Massachusetts Eye and Ear Infirmary), The Broad Institute of Harvard and MIT, and Cima Universidad de Navarra. Key contributors include YCP and HL as co-first authors, with Eric N. Olson serving as the corresponding author.

### Significance & Outlook

By utilizing a liver-sparing myotropic AAV, this method addresses a primary challenge in gene therapy: the tendency of AAV vectors to sequester in the liver, which can limit therapeutic efficacy in muscles and increase the risk of hepatotoxicity. The successful amelioration of muscular dystrophy in humanized mice via base editing suggests a more targeted and potentially safer mechanism for treating genetic muscle diseases.

Source: https://doi.org/10.1172/JCI202378

# #10 NPC1 Base Editing Screens: Identifying Second-Site Corrector Mutations

Published November 02, 2026 bioRxiv USA



## OVERVIEW

Researchers used tiled adenine and cytosine base editing screens to identify second-site corrector mutations that rescue misfolded NPC1 proteins. The study discovered a promiscuous variant capable of correcting multiple pathogenic mutations, restoring cholesterol clearance in patient-derived fibroblasts and induced neurons.

## IN DEPTH

### Key Findings

Tiled base editing screens have identified rare intragenic suppressor mutations that rescue the function of misfolded NPC1 proteins. A significant discovery is a promiscuous second-site variant capable of correcting various different pathogenic NPC1 mutations, rather than being limited to a single specific allele.

### Technical Details

The study utilized tiled adenine and cytosine base editing to perform mutagenesis of the endogenous NPC1 locus in haploid human cells. These cells carried the prevalent NPC1 I1061T disease allele. To identify effective correctors, the researchers employed phenotypic reporters focusing on protein abundance and cholesterol transport. The resulting corrector mutations were found to be distributed across multiple domains of the protein.

### Background & Context

NPC1 is a lysosomal cholesterol transporter; when it misfolds, it causes Niemann-Pick type C disease. While therapeutic base editing can directly revert approximately 30% of disease-causing missense mutations to wild-type sequences, many mutations remain unreachable. Installing second-site corrector mutations offers a strategy to improve protein stability or function across multiple pathogenic alleles when the original mutation cannot be directly corrected.

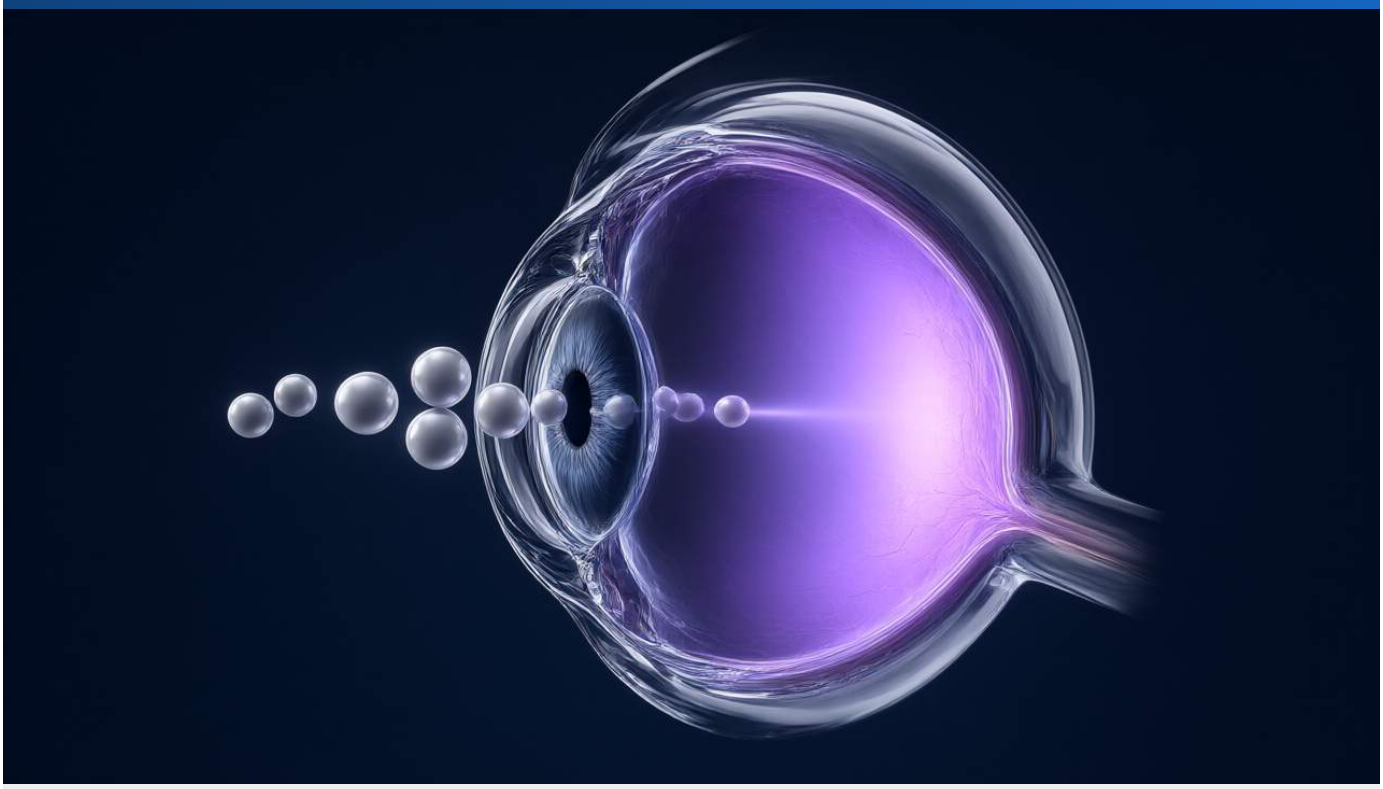
### Significance & Outlook

The identified corrector mutations improve protein stability, promote lysosomal trafficking, and facilitate ER exit. The efficacy of these mutations was validated in patient-derived fibroblasts and induced neurons, where they successfully restored cholesterol clearance. This research establishes in situ base editing screens as a viable method for discovering actionable second-site corrector mutations for therapeutic applications.

Source: https://doi.org/10.64898/2026.10.30.763201

# #11 ATSN-301: Dual AAV.SPR-MYO7A Safety and Preclinical Development

Published October 29, 2026 bioRxiv USA



## OVERVIEW

Atsena Therapeutics has developed ATSN-301, a dual AAV.SPR vector system designed to treat Usher Syndrome Type 1B by reconstituting the full-length MYO7A protein. A six-month GLP toxicology study established a No Observed Adverse Effect Level at 1.2E11 vg/eye, supporting the candidate's advancement toward clinical use.

## IN DEPTH

### Key Findings

The preclinical evaluation of ATSN-301, a dual vector system using the AAV.SPR capsid and smCBA promoter, demonstrated that the treatment is well tolerated across all tested doses. A six-month GLP toxicology study identified the No Observed Adverse Effect Level (NOAEL) as 1.2E11 vg/eye. In cynomolgus macaques, the AAV.SPR capsid enabled supraphysiological MYO7A expression at distal retinal sites and the macula without the need for foveal detachment.

### Technical Details

Because the MYO7A coding sequence exceeds the standard packaging capacity of adeno-associated virus (AAV), researchers utilized dual AAV hybrid and overlap platforms. To optimize the reconstitution of the full-length protein in retinal pigment epithelium and photoreceptors, the team implemented the following modifications:

- Adjustment of cDNA split points.
- Reduction of non-coding sequences.

These optimizations resulted in higher levels of full-length MYO7A and a substantial reduction in truncated protein production.

### Background & Context

Usher syndrome type 1B (USH1B) is caused by mutations in the MYO7A gene and represents over 50% of all USH1 cases. It is characterized as the most severe form of the syndrome, where patients are born with profound deafness and vestibular dysfunction, later developing progressive retinitis pigmentosa. Currently, there are no approved treatments for this condition.

## Significance & Outlook

The ability to achieve effective protein expression without foveal detachment is a critical safety consideration for USH1B patients who still possess remaining central vision. By overcoming the packaging limitations of AAV and demonstrating safety in non-human primates, ATSN-301 provides a viable path toward clinical intervention for patients with this severe genetic disorder.

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Source: https://doi.org/10.64898/2026.10.24.760972

Collected: November 07, 2026 | Automated Research System (AI)

# #12 Sumitomo Pharma: iPS Cell Therapy for Parkinson's Timeline

Published October 31, 2026　Philstar.com　フィリピン



## OVERVIEW

Sumitomo Pharma has performed the first transplant of induced Pluripotent Stem (iPS) cells to treat Parkinson's disease at Kyoto University Hospital. The therapy, which received conditional approval in Japan in March, aims to restore dopamine-producing brain cells.

## IN DEPTH

### Key Findings

Sumitomo Pharma has initiated the first patient treatment using a pioneering stem-cell therapy for Parkinson's disease. The first transplant was conducted in October at Kyoto University Hospital following conditional approval from Japanese regulators in March. The company plans to perform transplants on 20 to 30 patients by March 2029 and is currently conducting a clinical trial in the United States.

### Technical Details

The treatment utilizes induced Pluripotent Stem (iPS) cells derived from healthy donors. These cells are induced from mature, specialized cells back to a juvenile state and then developed into precursors of dopamine-producing brain cells, which are absent in Parkinson's patients. The procedure involves transplanting millions of these iPS cells directly into the patient's brain.

In a prior clinical trial involving seven patients aged 50 to 69, implants of either five million or 10 million cells were administered to both sides of the brain. Monitoring over a two-year period revealed no major adverse effects, and four of the patients demonstrated symptom improvement.

### Background & Context

Parkinson's disease is a chronic, degenerative neurological disorder affecting the motor system, characterized by shaking and movement difficulties. According to the Parkinson's Foundation, more than 10 million people worldwide live with the condition. Current therapies are limited to improving symptoms and do not slow or halt the progression of the disease.

## Significance & Outlook

The therapy will move toward full approval in Japan if safety and efficacy are confirmed over the next seven years. This development follows a broader trend of iPS cell applications in Japan; a hospital in Osaka recently performed the world's first surgery using iPS cell sheets of heart muscle to restore heart function and form new blood vessels, a therapy that also received conditional approval in March.

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Source: https://www.philstar.com/lifestyle/health-and-family/2026/10/31/2566919/1st-patient-undergoes-pioneering-stem-cell-treatment-parkinsons

Collected: November 07, 2026 | Automated Research System (AI)