TROY TECHNICAL

iPS & REGENERATIVE MEDICINE

WEEKLY REPORT

November 8 - November 14, 2026 | Issue 2026-W45

Clinical evidence, manufacturability, reimbursement and scalable quality

16	10
ANALYZED ARTICLES	PRIORITY THEMES

Issued 2026-11-14 | English edition | fixed approved cover artwork



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

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

SIGNAL 01 | CELL SOURCE

Intrasplenic Cell Transplantation: Feasibility in OIDP Pig Models

Researchers demonstrated that the spleen can serve as an ectopic transplantation site for human stem cell-derived hepatocyte-like cells (iPSC-HLCs) and induced tissue progenitor cell-derived islets (iTPC-derived islets) in operational immunodeficient pigs.

SIGNAL 02 | PROCESS & MATERIALS

CTX310: CRISPR Therapeutics Phase 1b Data for Severe Hypertriglyceridemia

A single-course treatment of CTX310 reduced circulating ANGPTL3 by 65% and triglycerides (TG) by 63% at Day 30 in patients with severe hypertriglyceridemia.

SIGNAL 03 | QUALITY CONTROL

Human iPS-derived Hypothalamic-Pituitary Organoids: Efficient Induction Method via Feeder Cell Optimization

Researchers at CiRA have developed a method to improve the differentiation efficiency of hypothalamic-pituitary organoids by optimizing the ratio of two types of feeder cells.

SIGNAL 04 | CLINICAL USE

Kamau Therapeutics: Nula-cel RMAT designation and SCD trial data

Kamau Therapeutics has received FDA Regenerative Medicine Advanced Therapy (RMAT) designation for nula-cel, a gene-corrected stem cell therapy for sickle cell disease.

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.

Numbers and evidence anchors that require conditions

65%
A06

SOURCE-BOUND CONDITION
A single-course treatment of CTX310 reduced circulating ANGPTL3 by 65% and triglycerides (TG) by 63% at Day 30 in patients with severe hypertriglyceridemia.

83%
A07

SOURCE-BOUND CONDITION
In systemic sclerosis (SSc) patients, 83% achieved a clinical response at Month 3, while all nine patients with idiopathic inflammatory myopathy (IIM) showed moderate or major improvement.

90%
A01

SOURCE-BOUND CONDITION
A single-course CRISPR adenine base editing treatment targeting ANGPTL3 achieved a > 90% reduction in circulating protein in nonhuman primates, lasting up to 3 years.

71 patients
A09

SOURCE-BOUND CONDITION
The therapy demonstrated a favorable benefit-risk profile in 71 patients, suggesting a potential "immune reset" for severe autoimmune diseases.

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 demonstrated that the spleen can serve as an ectopic transplantatio n site for human stem cell-derived.

2

Process & materials

P02/P07

A single-course treatment of CTX310 reduced circulating ANGPTL3 by 65% and triglycerides (TG) by 63% at Day 30 in patients.

3

Quality contr ol

P03/P08

Researchers at CiRA have developed a method to improve the differentiation efficiency of hypothalamic-pituitary organoids by.

4

Clinical use

P04/P09

Kamau Therapeutics has received FDA Regenerative Medicine Advanced Therapy (RMAT) designation for nula-cel, a gene-corrected.

5

Access & scale

P05/P10

The FDA has proposed draft guidance to accelerate personalize d gene therapies for ultrarare diseases by allowing the approval.

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	Intrasplenic Cell Transplantation: Feasibility in OIDP Pig Models System / adoption | cue: operational
P02	CTX310: CRISPR Therapeutics Phase 1b Data for Severe Hypertriglyceridemia Tech / process | cue: no system/business cue
P03	Human iPS-derived Hypothalamic-Pituitary Organoids: Efficient Induction Method via Feeder Cell Optimization Tech / process | cue: no system/business cue
P04	Kamau Therapeutics: Nula-cel RMAT designation and SCD trial data System / adoption | cue: trial
P05	FDA Gene Therapy Guidance: Personalized Platform Approval Roadmap Business / rules | cue: FDA
P06	Cancer Cachexia: Cardiac Wasting Signaling in hiPSC-CMs Business / rules | cue: contractile
P07	KIR3DL2-directed CAR T-cells: Preclinical efficacy for TCLs Tech / process | cue: no system/business cue
P08	Zugo-cel: CRISPR Therapeutics' Phase 1 Autoimmune Trial Data System / adoption | cue: Phase 1
P09	ANGPTL3 Base Editing: Durable Lipid Lowering via GalNAc-LNP System / adoption | cue: clinical
P10	Rese-cel Phase 1/2 Data: Cabaletta Bio's Autoimmune Results 2026 System / adoption | cue: Phase 1

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 Intrasplenic Cell Transplantation: Feasibility in OIDP Pig Models

A04 | Cell source | Product adoption

Prior release 2026-11-06

FACT

Researchers demonstrated that the spleen can serve as an ectopic transplantation site for human stem cell-derived hepatocyte-like cells (iPSC-HLCs) and induced tissue progenitor cell-derived islets (iTPC-derived islets) in operational immunodeficient pigs (OIDP). The study confirmed the production of human albumin and C-peptide in the serum, suggesting the spleen's viability as a clinically relevant alternative to the liver.

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 20/25 | MATURITY 4/5 | DECISION LOCUS: SYSTEM / ADOPTION

P02 CTX310: CRISPR Therapeutics Phase 1b Data for Severe Hypertriglyceridemia

A06 | Process & materials | Product adoption

2026-11-09

FACT

A single-course treatment of CTX310 reduced circulating ANGPTL3 by 65% and triglycerides (TG) by 63% at Day 30 in patients with severe hypertriglyceridemia. The in vivo CRISPR/Cas9 therapy was well tolerated with no treatment-related serious adverse events, suggesting a potential one-time treatment for high-risk lipid disorders.

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 20/25 | MATURITY 4/5 | DECISION LOCUS: TECH / PROCESS

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

P03 Human iPS-derived Hypothalamic-Pituitary Organoids: Efficient Induction Method via Feeder Cell Optimization

A10 | Quality control | Product adoption

Prior release 2026-11-06

FACT

Researchers at CiRA have developed a method to improve the differentiation efficiency of hypothalamic-pituitary organoids by optimizing the ratio of two types of feeder cells. The team also established a "Weekend-free" induction protocol using a stable bFGF mutant to reduce the frequency of medium exchanges. These advancements reduce researcher workload and enhance the production of functional pituitary hormone-producing cells for disease research and regenerative medicine.

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 20/25 | MATURITY 4/5 | DECISION LOCUS: TECH / PROCESS

P04 Kamau Therapeutics: Nula-cel RMAT designation and SCD trial data

A15 | Clinical use | Joint evaluation

2026-11-12

FACT

Kamau Therapeutics has received FDA Regenerative Medicine Advanced Therapy (RMAT) designation for nula-cel, a gene-corrected stem cell therapy for sickle cell disease. Preliminary Phase 1/2 RESTORE trial data shows all three patients treated with the optimized process achieved transfusion independence and zero vaso-occlusive events. This therapy aims to provide a lifelong cure by correcting the root mutation rather than compensating with fetal hemoglobin.

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 3/5 | DECISION LOCUS: SYSTEM / ADOPTION

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

P05 FDA Gene Therapy Guidance: Personalized Platform Approval Roadmap

A16 | Access & scale | Joint evaluation

2026-11-08

FACT

The FDA has proposed draft guidance to accelerate personalized gene therapies for ultrarare diseases by allowing the approval of a core platform rather than individual drug versions. This framework enables the customization of treatments for specific genetic mutations without requiring separate clinical trials for every variation.

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 20/25 | MATURITY 3/5 | DECISION LOCUS: BUSINESS / RULES

P06 Cancer Cachexia: Cardiac Wasting Signaling in hiPSC-CMs

A12 | Cell source | Product adoption

2026-11-09

FACT

Researchers have developed a human induced pluripotent stem cell-derived cardiac myocyte (hiPSC-CM) platform to model cancer cachexia. The study identifies that tumor-induced cardiac wasting is driven by the suppression of the AKT/mTORC1/S6 signaling axis, leading to contractile dysfunction and metabolic dysregulation.

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 20/25 | MATURITY 4/5 | DECISION LOCUS: BUSINESS / RULES

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

P07 KIR3DL2-directed CAR T-cells: Preclinical efficacy for TCLs

A11 | Process & materials | Pilot

Prior release 2026-11-06

FACT

Researchers have developed a novel CAR T-cell therapy targeting KIR3DL2 (CD158k) to treat cutaneous and systemic T-cell lymphomas (TCLs). The therapy demonstrated sustained tumor regression in xenograft models with minimal off-target activity, providing a potential alternative to pan-T-cell-directed strategies.

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 20/25 | MATURITY 2/5 | DECISION LOCUS: TECH / PROCESS

P08 Zugo-cel: CRISPR Therapeutics' Phase 1 Autoimmune Trial Data

A07 | Quality control | Joint evaluation

2026-11-08

FACT

CRISPR Therapeutics reported encouraging preliminary data for zugocabtagene geleucel (zugo-cel), an allogeneic CD19-targeting CAR-T therapy, in refractory rheumatologic autoimmune diseases. In systemic sclerosis (SSc) patients, 83% achieved a clinical response at Month 3, while all nine patients with idiopathic inflammatory myopathy (IIM) showed moderate or major improvement.

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 3/5 | DECISION LOCUS: SYSTEM / ADOPTION

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

P09 ANGPTL3 Base Editing: Durable Lipid Lowering via GalNAc-LNP

A01 | Clinical use | Joint evaluation

Prior release 2026-11-06

FACT

A single-course CRISPR adenine base editing treatment targeting ANGPTL3 achieved a > 90% reduction in circulating protein in nonhuman primates, lasting up to 3 years. The therapy reduced LDL-C by 40-50% and triglycerides by 50-70% in hyperlipidemia models. These results provide the foundation for a first-in-human clinical trial for refractory hyperlipidemia syndromes.

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 3/5 | DECISION LOCUS: SYSTEM / ADOPTION

P10 Rese-cel Phase 1/2 Data: Cabaletta Bio's Autoimmune Results 2026

A09 | Access & scale | Research

2026-11-08

FACT

Cabaletta Bio reported Phase 1/2 data for rese-cel, showing durable, immunomodulator-free responses across dermatomyositis, lupus nephritis, and systemic sclerosis. The therapy demonstrated a favorable benefit-risk profile in 71 patients, suggesting a potential "immune reset" for severe autoimmune diseases.

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 1/5 | DECISION LOCUS: SYSTEM / ADOPTION

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 01-08

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	ANGPTL3 Base Editing: Durable Lipid Lowering via GalNAc-LNP	https://doi.org/10.1038/s41586-026-11233-9
A02	Fate Therapeutics FT819: Lupus Clinical Data and ACR 2026 Results	https://www.biospace.com/press-releases/fate-therapeutics-presents-new-ft819-data-at-acr-convergence-2026-highlighting-meaningful-clinical-responses-in-lupus-without-the-use-of-conditioning-chemotherapy
A03	ACE-engineered iPSC-derived neutrophils: MDR infection defense	https://doi.org/10.64898/2026.11.09.765250
A04	Intrasplenic Cell Transplantation: Feasibility in OIDP Pig Models	https://doi.org/10.1097/txd.0000000000002025
A05	Lonvo-z: Intellia's in vivo CRISPR therapy and FDA timeline	https://www.nature.com/articles/d41586-026-03705-9
A06	CTX310: CRISPR Therapeutics Phase 1b Data for Severe Hypertriglyceridemia	https://markets.businessinsider.com/news/stocks/crispr-therapeutics-presents-phase-1b-data-for-ctx310-demonstrating-reductions-in-angptl3-and-triglycerides-in-patients-with-severe-hypertriglyceridemia-at-the-american-heart-association-aha-1036737204
A07	Zugo-cel: CRISPR Therapeutics' Phase 1 Autoimmune Trial Data	https://markets.businessinsider.com/news/stocks/crispr-therapeutics-provides-update-on-zugocabtagene-geleucel-zugo-cel-in-refractory-rheumatologic-autoimmune-diseases-1036733484
A08	NKX019 Safety Data: Nkarta's CAR NK Therapy for Autoimmune Diseases	https://finance.yahoo.com/healthcare/articles/nkarta-presents-initial-safety-data-120000700.html

ANALYZED ARTICLES | ITEMS 09-16

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
A09	Rese-cel Phase 1/2 Data: Cabaletta Bio's Autoimmune Results 2026	https://www.manilatimes.net/2026/11/08/tmt-newswire/globenewswire/cabaletta-bio-presents-new-rese-cel-phase-12-clinical-data-at-acr-convergence-2026-featuring-immunomodulator-free-durability-and-favorable-benefit-risk-profile-across-autoimmune-diseases/2464767
A10	Human iPS-derived Hypothalamic-Pituitary Organoids: Efficient Induction Method via Feeder Cell Optimization	https://www.cira.kyoto-u.ac.jp/j/pressrelease/news/261106-010000.html
A11	KIR3DL2-directed CAR T-cells: Preclinical efficacy for TCLs	https://doi.org/10.64898/2026.11.05.764385
A12	Cancer Cachexia: Cardiac Wasting Signaling in hiPSC-CMs	https://doi.org/10.1172/jci.insight.203276
A13	Human iPSC-derived nociceptors: Impact of surface coatings on phenotype	https://doi.org/10.21203/rs.3.rs-11207616/v1
A14	iPSC-CMs: Adult-like Thick-Filament Protein Stoichiometry	https://doi.org/10.64898/2026.11.09.761920
A15	Kamau Therapeutics: Nula-cel RMAT designation and SCD trial data	https://markets.businessinsider.com/news/stocks/kamau-therapeutics-receives-fda-regenerative-medicine-advanced-therapy-rmat-designation-for-nula-cel-a-gene-corrected-stem-cell-therapy-for-sickle-cell-disease-1036755594
A16	FDA Gene Therapy Guidance: Personalized Platform Approval Roadmap	https://www.inquirer.com/health/fda-guidelines-personalized-gene-therapy-rare-diseases-philadelphia-scientists-20261108.html

iPS_RegenerativeMedicine — Selected Articles

Date: 2026-11-15

Articles: 16

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#15 Kamau Therapeutics: Nula-cel RMAT designation and SCD trial data

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#01 ANGPTL3 Base Editing: Durable Lipid Lowering via GalNAc-LNP

Published November 06, 2026 Nature USA



OVERVIEW

A single-course CRISPR adenine base editing treatment targeting ANGPTL3 achieved a > 90% reduction in circulating protein in nonhuman primates, lasting up to 3 years. The therapy reduced LDL-C by 40–50% and triglycerides by 50–70% in hyperlipidemia models. These results provide the foundation for a first-in-human clinical trial for refractory hyperlipidemia syndromes.

IN DEPTH

Key Findings

The administration of a CRISPR adenine base editor resulted in durable, liver-selective inactivation of angiopoietin-like 3 (ANGPTL3). In nonhuman primates, a single dose led to a mean reduction in circulating ANGPTL3 protein of > 90%, with effects persisting for 3 years. In both mouse and nonhuman primate models of hyperlipidemia, this gene inactivation was associated with reductions of approximately 40 to 50% in low-density lipoprotein cholesterol (LDL-C) and 50 to 70% in triglycerides.

Technical Details

The treatment utilizes a GalNAc–lipid nanoparticle (LNP) to encapsulate guide RNA and mRNA encoding the CRISPR adenine base editor. This delivery system enables liver-selective targeting. In vitro analyses using human liver cells confirmed that the base editor introduces a DNA base-pair change at the target ANGPTL3 locus. Safety assessments showed no detectable off-target editing across approximately 3000 candidate sites, nor were any treatment-related structural variants detected.

Background & Context

Circulating concentrations of LDL-C and triglyceride-rich lipoproteins are primary risk factors for atherosclerotic cardiovascular disease. While various lipid-lowering medications are approved, the chronic care treatment model often fails to help most patients achieve target lipid levels. ANGPTL3 is identified as a known driver that increases both LDL-C and triglyceride concentrations, making it a strategic target for permanent or durable inactivation.

Significance & Outlook

By shifting from chronic medication to a potential single-course treatment, this approach addresses the limitations of current lipid-management models. The durability and precision demonstrated in nonhuman primates and human liver cells have led to the initiation of an ongoing first-in-human clinical trial. This trial focuses on patients suffering from refractory hyperlipidemia syndromes.

Source: https://doi.org/10.1038/s41586-026-11233-9

Collected: November 14, 2026 | Automated Research System (AI)

#02 Fate Therapeutics FT819: Lupus Clinical Data and ACR 2026 Results

Published November 10, 2026　BioSpace（Fate Therapeutics プレスリリース）　USA



OVERVIEW

Fate Therapeutics reported that 100% of extrarenal lupus patients treated with FT819 without conditioning chemotherapy achieved measurable clinical responses. Safety data from 36 patients across four autoimmune indications showed no dose-limiting toxicities, supporting a shift toward outpatient administration and reduced chemotherapy reliance.

IN DEPTH

Key Findings

Clinical data presented at ACR Convergence 2026 indicate that FT819 can induce meaningful responses in lupus patients without the use of conditioning chemotherapy. In a cohort of three extrarenal lupus patients treated under this regimen, two achieved the Definition of Remission in Systemic Lupus Erythematosus (DORIS) and one achieved Lupus Low Disease Activity State (LLDAS). Additionally, a patient treated with a two-dose regimen saw a reduction in the Systemic Lupus Erythematosus Disease Activity Index (SLEDAI-2K) by 8 points at Month 1, reaching LLDAS by Month 2 and sustaining it through Month 3.

Technical Details

The FT819 Phase 1 study evaluates three distinct regimens:

- **Regimen A:** Single dose following fludarabine-free conditioning with cyclophosphamide or bendamustine.
- **Regimen B:** Single dose without conditioning chemotherapy, added to stable background therapy.
- **Regimen C:** Two doses (Day 1 and Day 15) following fludarabine-free conditioning.

As of August 18, 2026, 36 patients were treated, with 30 completing at least three months of follow-up. Outpatient administration was utilized for 12 of 36 patients (33%), with eight discharged the same day and four the following day. Safety data for outpatients was comparable to inpatient settings, with grade ≥3 treatment-emergent adverse events occurring in 16.7% of both groups and serious adverse events in 8.3% of both groups.

Background and Context

FT819 is an iPSC-derived cellular immunotherapy. Fate Therapeutics is also advancing FT839, a dual-CAR T cell targeting CD19 and CD38. Preclinical data for FT839 demonstrate the elimination of a broad spectrum of activated immune cells in rheumatoid arthritis models, supporting the goal of immune cell reset without conditioning chemotherapy.

Significance and Outlook

The ability to deliver FT819 as an outpatient therapy without intensive conditioning reduces barriers for autoimmune disease patients. These results inform the design of the RECLAIM-LN Phase 2 trial for lupus nephritis and the COMPLETE Phase 1/2 basket trial for FT839. The company's scalable manufacturing process is intended to enable on-demand dosing and lower costs of goods.

Source: https://www.biospace.com/press-releases/fate-therapeutics-presents-new-ft819-data-at-acr-convergence-2026-highlighting-meaningful-clinical-responses-in-lupus-without-the-use-of-conditioning-chemotherapy

Collected: November 14, 2026 | Automated Research System (AI)

#03 ACE-engineered iPSC-derived neutrophils: MDR infection defense

Published November 11, 2026 bioRxiv



OVERVIEW

Researchers have engineered human induced pluripotent stem cells (iPSCs) to express angiotensin-converting enzyme (ACE), creating neutrophils with enhanced antimicrobial capabilities. These ACE-iNeu cells demonstrated superior bactericidal activity against multidrug-resistant bacteria and reduced bacterial burdens in neutropenic mice.

IN DEPTH

Key Findings

ACE-engineered human iPSC-derived neutrophils (ACE-iNeu) significantly enhance host defense against multidrug-resistant (MDR) bacterial infections. Specifically, these engineered cells exhibited increased bactericidal activity against methicillin-resistant Staphylococcus aureus and multidrug-resistant Pseudomonas aeruginosa. In vivo testing via adoptive transfer into neutropenic mice showed that ACE-iNeu significantly reduced bacterial burdens in both circulating blood and tissues.

Technical Details

The researchers engineered human iPSCs to express angiotensin-converting enzyme (ACE) using either constitutive or inducible expression methods. The resulting ACE-iNeu cells showed improvements across several functional markers:

- Enhanced granulocytic maturation
- Increased reactive oxygen species (ROS) generation
- Higher inflammatory cytokine production
- Strengthened antimicrobial signaling

To understand the underlying mechanisms, the team performed integrated metabolomic and transcriptomic analyses. These results revealed that ACE expression is associated with alterations in adenine nucleotide abundance, as well as changes in developmental and inflammatory pathways.

Background & Context

Severe neutropenia leaves patients highly susceptible to life-threatening infections. While granulocyte transfusions are a current treatment option, they are limited by the availability of donors and the variable quality of the cells provided. This study explores the use of iPSC technology to create a standardized, engineered cell source to overcome these limitations.

Significance & Outlook

The study establishes ACE engineering as a viable strategy to improve both the production efficiency and the antimicrobial potency of human iPSC-derived neutrophils. These findings provide a foundation for the further preclinical development of engineered granulocyte therapies designed to treat severe drug-resistant infections.

Source: https://doi.org/10.64898/2026.11.09.765250

Collected: November 14, 2026 | Automated Research System (AI)

#04 Intrasplenic Cell Transplantation: Feasibility in OIDP Pig Models

Published November 06, 2026 Transplantation Direct Japan



OVERVIEW

Researchers demonstrated that the spleen can serve as an ectopic transplantation site for human stem cell-derived hepatocyte-like cells (iPSC-HLCs) and induced tissue progenitor cell-derived islets (iTPC-derived islets) in operational immunodeficient pigs (OIDP). The study confirmed the production of human albumin and C-peptide in the serum, suggesting the spleen's viability as a clinically relevant alternative to the liver.

IN DEPTH

Key Findings

The study established the feasibility of intrasplenic cell transplantation via the splenic artery using an operational immunodeficient pig (OIDP) model. Following the transplantation of human-induced pluripotent stem cell-derived hepatocyte-like cells (iPSC-HLCs), human albumin was detected in pig serum 2 weeks post-transplantation. Histological analysis at 4 weeks identified colonies of cells positive for human albumin and vimentin. Additionally, transplantation of quantum-dot-labeled human iTPC-derived islets resulted in the detection of human C-peptide in the serum after a glucose tolerance test, with histological confirmation of insulin-producing cells in the spleen.

Technical Details

The OIDP model involved thymectomy and the administration of tacrolimus, mycophenolate mofetil, and prednisolone via a gastric tube. To prevent rejection without removing the organ, splenic immune cells were inactivated by perfusing mitomycin C through a temporal extracorporeal circuit that isolated the spleen from systemic circulation. This circuit also served as the route for cell delivery. The researchers used adult microminiature pigs (27- to 29-mo-old, 20–24 kg) for iPSC-HLC transplantation and domestic piglets (5-wk-old, approximately 10 kg) for iTPC-derived islet transplantation.

Background and Context

While intrahepatic transplantation (such as the Edmonton protocol) is a standard for islets, researchers sought an alternative ectopic site. The spleen was selected due to its sinusoidal vascular structure, which mimics that of the liver. The study utilized iPSC-HLCs derived from hepatic fibroblasts of pediatric drug-induced fulminant hepatitis patients and iTPC-derived islets produced from human pancreatic cells containing 80% or more islets.

Significance and Outlook

The spleen offers several advantages as a transplantation site: it typically remains healthy during liver disease or diabetes, it can be surgically removed if tumors form without risking the patient's life, and it allows for preconditioning via extracorporeal circulation. This proof-of-concept study provides a preclinical basis for assessing the therapeutic potential of progenitor-derived cells via a vascular approach that closely mimics clinical procedures.

Source: https://doi.org/10.1097/txd.0000000000002025

Collected: November 14, 2026 | Automated Research System (AI)

#05 Lonvo-z: Intellia's in vivo CRISPR therapy and FDA timeline

Published November 12, 2026 Nature（News） USA



OVERVIEW

Intellia Therapeutics has applied for FDA approval of lonvoguran ziclumeran (lonvo-z), a CRISPR-Cas9 therapy that edits genes directly inside the body. Data shows the treatment remains effective four years post-administration, potentially eliminating the need for lifelong medication for hereditary angioedema.

IN DEPTH

Key Findings

Intellia Therapeutics has submitted an application to the US Food and Drug Administration (FDA) for lonvoguran ziclumeran (lonvo-z). If approved, lonvo-z will be the first CRISPR-based medicine delivered *in vivo* (directly inside the body). A decision from the FDA is expected by 10 March. Recent data released by the developer indicates that the therapy continues to be effective four years after treatment.

Technical Details

Lonvo-z is designed to treat a form of hereditary angioedema characterized by painful swelling in the throat and other body parts. The therapy utilizes the CRISPR–Cas9 system to disable the gene responsible for producing prekallikrein, a liver-produced protein that promotes blood-vessel leakage. To achieve this, the therapy employs fatty particles that encase mRNA encoding the CRISPR–Cas9 components. These particles are infused into the blood and taken up by the liver. The entire procedure takes one day.

Background & Context

Currently, the only FDA-approved CRISPR therapy is Casgevy, used for sickle-cell disease and β-thalassaemia. Unlike lonvo-z, Casgevy is an *ex vivo* treatment: blood stem cells are removed from the patient, edited externally, and then re-infused. This process requires harsh chemotherapy to prepare the patient's body and involves higher costs and complexity because each dose must be custom-made for the individual.

Significance & Outlook

The transition to *in vivo* editing aims to sidestep the complexities of cell removal and chemotherapy. However, this approach introduces specific safety risks, including the inability to screen for unwanted off-target edits and the potential for harmful immune responses. Intellia previously faced a clinical hold on two other *in vivo* trials after a participant with additional health conditions died from liver damage; the FDA lifted this hold this year following the implementation of extra safety measures.

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Source: https://www.nature.com/articles/d41586-026-03705-9

Collected: November 14, 2026 | Automated Research System (AI)

#06 CTX310: CRISPR Therapeutics Phase 1b Data for Severe Hypertriglyceridemia

Published November 09, 2026　Markets Insider（GlobeNewswire プレスリリース）　USA



OVERVIEW

A single-course treatment of CTX310 reduced circulating ANGPTL3 by 65% and triglycerides (TG) by 63% at Day 30 in patients with severe hypertriglyceridemia. The in vivo CRISPR/Cas9 therapy was well tolerated with no treatment-related serious adverse events, suggesting a potential one-time treatment for high-risk lipid disorders.

IN DEPTH

Key Findings

In a Phase 1b clinical trial, CTX310 demonstrated a mean reduction of 63% in triglycerides (TG) and 65% in circulating ANGPTL3 protein at Day 30. These results were observed in a cohort of 10 participants with severe hypertriglyceridemia (sHTG) who had a median baseline TG level above 1,000 mg/dL. Additionally, the treatment resulted in mean reductions of 51% in non-HDL-C, 23% in apolipoprotein B (ApoB), and 16% in LDL-C (from a mean baseline of 60 mg/dL). Reductions in ANGPTL3 and TG were maintained in participants followed for at least 90 days.

Technical Details

CTX310 is an investigational in vivo CRISPR/Cas9 gene editing therapy targeting angiopoietin-like protein 3 (ANGPTL3). The Phase 1b multicenter, open-label trial utilized a weight-based flat dosing approach: 40 mg for participants below 85 kg and 50 mg for those 85 kg or above, approximating the 0.8 mg/kg dose from Phase 1a. One participant received a dose of 56.5 mg.

The sHTG cohort consisted of 10 participants with fasting serum TG above 500 mg/dL. All participants had diabetes; 30% had a history of pancreatitis, and 20% had clinical atherosclerotic cardiovascular disease. Safety data indicated no treatment-related serious adverse events and no clinically relevant elevations in liver transaminases (AST or ALT). One participant experienced a Grade 2 infusion-related reaction that resolved.

Background & Context

The data were presented at the AHA Scientific Sessions 2026, building upon Phase 1a results and one-year durability data published in The New England Journal of Medicine. The U.S. FDA has granted Regenerative Medicine Advanced Therapy (RMAT) designations to CTX310 for the treatment of sHTG and for patients with elevated low-density lipoprotein cholesterol (LDL-C).

Significance & Outlook

Severe hypertriglyceridemia is associated with increased risks of acute pancreatitis and cardiovascular disease, often remaining uncontrolled despite current therapies. No events of acute pancreatitis were reported during this study. CRISPR Therapeutics is continuing the Phase 1b trial across multiple lipid disorders with ongoing sites in the United States, United Kingdom, Australia, and New Zealand.

Source: https://markets.businessinsider.com/news/stocks/crispr-therapeutics-presents-phase-1b-data-for-ctx310-demonstrating-reductions-in-angptl3-and-triglycerides-in-patients-with-severe-hypertriglyceridemia-at-the-american-heart-association-aha-1036737204

Collected: November 14, 2026 | Automated Research System (AI)

#07 Zugo-cel: CRISPR Therapeutics' Phase 1 Autoimmune Trial Data

Published November 08, 2026　Markets Insider（GlobeNewswire プレスリリース）　USA



OVERVIEW

CRISPR Therapeutics reported encouraging preliminary data for zugocabtagene geleucel (zugo-cel), an allogeneic CD19-targeting CAR-T therapy, in refractory rheumatologic autoimmune diseases. In systemic sclerosis (SSc) patients, 83% achieved a clinical response at Month 3, while all nine patients with idiopathic inflammatory myopathy (IIM) showed moderate or major improvement.

IN DEPTH

Key Findings

Preliminary data from a Phase 1 basket trial indicate that zugo-cel is well tolerated and effective across multiple refractory rheumatologic indications. As of October 15, 2026, 21 patients have been dosed. In the systemic sclerosis (SSc) cohort, 83% (5/6) of patients receiving standard lymphodepletion achieved a revised Composite Response Index in Systemic Sclerosis (rCRISS)-25 response at Month 3, with all five responders maintaining their response. Additionally, all nine patients with idiopathic inflammatory myopathy (IIM)—including those with antisynthetase syndrome, dermatomyositis, and immune-mediated necrotizing myopathy—achieved a moderate or major Total Improvement Score (TIS) response.

Technical Details

Zugo-cel is an off-the-shelf allogeneic CAR-T cell therapy targeting CD19. It utilizes CRISPR/Cas9 for targeted gene knockout and CAR insertion to enable immune evasion and enhance T effector cell potency, removing the need for HLA matching. The trial is currently evaluating dose escalation; six patients dosed at the 200 million CAR+ cell level demonstrated higher CAR-T cell expansion compared to those at the 100 million level.

Safety data across all 21 patients showed no dose-limiting toxicities, no ICANS, no IEC-HS, and no cytokine release syndrome (CRS) above Grade 2. There were no reported deaths, severe infections, or prolonged cytopenias.

Background and Context

The rheumatology basket trial focuses on systemic lupus erythematosus (SLE), SSc, and IIM. In the SLE cohort, two of three evaluable patients maintained drug-free DORIS remission through at least Month 9. Beyond rheumatology, CRISPR Therapeutics is evaluating zugo-cel in Phase 1 trials for autoimmune hematologic diseases (including ITP and wAIHA), autoimmune neurologic diseases (including progressive multiple sclerosis and various autoimmune encephalitides), and relapsed or refractory B-cell malignancies.

Significance and Outlook

The results suggest that an allogeneic, "off-the-shelf" approach may provide a meaningful treatment option for patients with refractory autoimmune diseases who typically face a lifelong burden of immunosuppressive therapy. The company continues to advance the therapy across its various autoimmune and immuno-oncology pipelines.

Source: https://markets.businessinsider.com/news/stocks/crispr-therapeutics-provides-update-on-zugocabtagene-geleucel-zugo-cel-in-refractory-rheumatologic-autoimmune-diseases-1036733484

Collected: November 14, 2026 | Automated Research System (AI)

#08 NKX019 Safety Data: Nkarta's CAR NK Therapy for Autoimmune Diseases

Published November 11, 2026　Yahoo Finance（GlobeNewswire プレスリリース）　USA



OVERVIEW

Nkarta reported no cases of CRS, ICANS, IEC-HS, or GvHD across 40 patients treated with NKX019 for five autoimmune diseases. The data indicates a favorable safety profile with no deaths or treatment discontinuations due to adverse events. This suggests CAR NK cells may provide a safer alternative to CAR T cells by limiting in vivo expansion.

IN DEPTH

Key Safety Findings

Integrated safety analysis of 40 patients in the Ntrust program showed no reported cases of cytokine release syndrome (CRS), immune effector cell-associated neurotoxicity syndrome (ICANS), immune effector cell-associated hemophagocytic lymphohistiocytosis-like syndrome (IEC-HS), or graft-versus-host disease (GvHD). There were no deaths and no treatment discontinuations resulting from adverse events. Additionally, no cases of fludarabine-associated leukoencephalopathy were reported.

Technical Details and Patient Cohort

The analysis included patients with systemic sclerosis, myositis, lupus nephritis, rheumatoid arthritis, and antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis. The cohort consisted of patients with active or progressive disease; 75% (30 patients) had received four or more prior treatments, with a median disease duration of 4.7 years. Patients underwent outpatient lymphodepleting preconditioning using fludarabine and cyclophosphamide before receiving a three-dose cycle of NKX019.

- **Dosing Distribution:** 70% (28 patients) received 4 billion cells, 17.5% (7 patients) received 2 billion cells, and 12.5% (5 patients) received 1 billion cells.
- **Adverse Events:** Treatment-emergent adverse events occurred in 85% (34 patients). Grade 3 or higher events occurred in 35% (14 patients), primarily hematologic issues attributed to lymphodepletion.
- **Infusion Reactions:** 12.5% (5 patients) experienced infusion-related reactions associated with NKX019 (four grade 1, one grade 3), all of which resolved within 24-48 hours without the use of tocilizumab.

Background and Biological Context

NKX019 is an allogeneic, off-the-shelf, CD19-directed CAR NK cell therapy. According to Nkarta CEO Paul J. Hastings, CAR NK cells differ from CAR T cells because they are designed for targeted B-cell depletion with limited in vivo expansion and more predictable cellular exposure. This biological difference is intended to avoid the toxicities typically associated with autologous T cell-based modalities.

Significance and Outlook

The data, presented at ACR Convergence 2026 with a cutoff date of September 18, 2026, suggests that the safety profile of NKX019 remains consistent regardless of dose, as no increase in adverse events was observed at higher doses.

Source: https://finance.yahoo.com/healthcare/articles/nkarta-presents-initial-safety-data-120000700.html

Collected: November 14, 2026 | Automated Research System (AI)

#09 Rese-cel Phase 1/2 Data: Cabaletta Bio's Autoimmune Results 2026

Published November 08, 2026　The Manila Times（GlobeNewswire プレスリリース）　USA



OVERVIEW

Cabaletta Bio reported Phase 1/2 data for rese-cel, showing durable, immunomodulator-free responses across dermatomyositis, lupus nephritis, and systemic sclerosis. The therapy demonstrated a favorable benefit-risk profile in 71 patients, suggesting a potential "immune reset" for severe autoimmune diseases.

IN DEPTH

Key Clinical Findings

Cabaletta Bio presented Phase 1/2 data for resecabtagene autoleucel (rese-cel) showing high rates of immunomodulator-free durability. In dermatomyositis patients, 100% (6 of 6) maintained moderate-to-major Total Improvement Score (TIS) responses through one year, with 83% achieving this by Week 16. For lupus nephritis, 100% (5 of 5) of patients with follow-up between six months and two years achieved DORIS remission and showed histologic response or remission via repeat kidney biopsies.

In systemic sclerosis, 71% (5 of 7) of patients achieved rCRISS-50 and 86% (6 of 7) achieved rCRISS-25 at one year. Additionally, five patients with interstitial lung disease showed a 9% mean improvement in forced vital capacity (FVC) at the one-year mark.

Technical Details and Safety Profile

Rese-cel is an investigational chimeric antigen receptor (CAR) T cell therapy administered as a single, weight-based intravenous infusion following preconditioning and the discontinuation of immunomodulators. Clinical experience across 71 patients spanning myositis, systemic sclerosis, lupus, and myasthenia gravis indicates a favorable benefit-risk profile:

- **Cytokine Release Syndrome (CRS):** 94% (67/71) experienced no or Grade 1 CRS; Grade 2 CRS occurred in 6% (4/71).
- **Neurotoxicity:** 96% (68/71) experienced no immune effector cell-associated neurotoxicity syndrome (ICANS).
- **Other:** No immune effector cell-associated hemophagocytic lymphohistiocytosis-like syndrome (IEC-HS) was reported.

Background and Program Context

The data, with a cut-off date of September 4, 2026, are part of the REstoring SElf-Tolerance (RESET™) clinical development program. This program consists of single-arm, company-sponsored trials in dermatology, neurology, and rheumatology. The cohorts are designed to transition directly into registrational studies.

Significance and Outlook

The results suggest that a single dose of rese-cel can induce a durable immune reset, allowing patients to discontinue immunomodulators and reduce or eliminate steroid use. This is particularly significant for patients at risk of severe disability and end-organ damage who do not respond sufficiently to current therapies.

Source: https://www.manilatimes.net/2026/11/08/tmt-newswire/globenewswire/cabaletta-bio-presents-new-rese-cel-phase-12-clinical-data-at-acr-convergence-2026-featuring-immunomodulator-free-durability-and-favorable-benefit-risk-profile-across-autoimmune-diseases/2464767

Collected: November 14, 2026 | Automated Research System (AI)

#10 Human iPS-derived Hypothalamic-Pituitary Organoids: Efficient Induction Method via Feeder Cell Optimization

Published November 06, 2026　京都大学 iPS細胞研究所（CiRA）　Japan



OVERVIEW

Researchers at CiRA have developed a method to improve the differentiation efficiency of hypothalamic-pituitary organoids by optimizing the ratio of two types of feeder cells. The team also established a "Weekend-free" induction protocol using a stable bFGF mutant to reduce the frequency of medium exchanges. These advancements reduce researcher workload and enhance the production of functional pituitary hormone-producing cells for disease research and regenerative medicine.

IN DEPTH

Key Findings

The research group led by Ryusaku Matsumoto and Takuya Yamamoto has developed a high-efficiency method for creating hypothalamic-pituitary organoids from human iPS cells. By adjusting the composition of feeder cells used during the maintenance culture of iPS cells, the team successfully optimized the ratio of hypothalamic cells to oral ectoderm cells, thereby increasing the differentiation efficiency into LHX3+ pituitary progenitor cells.

Technical Details

The study identified that the type of feeder cell influences differentiation preference: iPS cells cultured with SNL feeder cells showed a preference for non-neural ectoderm (resulting in more oral ectoderm cells), while those cultured with mouse embryonic fibroblasts (MEF) preferred neural ectoderm (resulting in more hypothalamic cells). To balance these populations, the researchers tested a mixture of the two. They found that using a mixed feeder cell layer with an SNL:MEF ratio between 3:1 and 1:1 significantly improved the induction of pituitary progenitor cells.

Additionally, the team addressed the labor-intensive nature of the process, which typically requires daily medium exchanges over 2–3 months. By replacing basic fibroblast growth factor (bFGF) with a stable mutant (FGF2-G3), they reduced medium exchange frequency to three times per week. This led to the establishment of a "Weekend-free" protocol that eliminates the need for weekend work while maintaining differentiation efficiency and showing a trend of increased ACTH secretion.

Background & Context

The pituitary gland develops from the oral ectoderm through critical cell-cell interactions with the adjacent hypothalamus. While iPS-derived organoids can reproduce these interactions and produce functional hormone-secreting cells, previous methods suffered from inconsistent efficiency across different cell lines and high operational burdens on researchers.

Significance & Outlook

This optimized induction method simplifies the production of three-dimensional tissue models that mimic the development and interaction of the hypothalamus and pituitary gland. These results, published in Stem Cell Reports on November 5, 2026, are expected to expand applications in the study of pituitary diseases and the development of regenerative medicine.

Source: https://www.cira.kyoto-u.ac.jp/j/pressrelease/news/261106-010000.html

Collected: November 14, 2026 | Automated Research System (AI)

#11 KIR3DL2-directed CAR T-cells: Preclinical efficacy for TCLs

Published November 06, 2026 bioRxiv USA



OVERVIEW

Researchers have developed a novel CAR T-cell therapy targeting KIR3DL2 (CD158k) to treat cutaneous and systemic T-cell lymphomas (TCLs). The therapy demonstrated sustained tumor regression in xenograft models with minimal off-target activity, providing a potential alternative to pan-T-cell-directed strategies.

IN DEPTH

Key Findings

The study establishes the preclinical efficacy and safety of 3DL2CART cells in treating various subtypes of cutaneous and systemic T-cell lymphomas. In vivo testing showed sustained tumor regression and prolonged survival across two cell line-derived xenograft models of cutaneous TCL and one patient-derived xenograft model of hepatosplenic TCL. The therapy exhibited minimal off-target activity against bystander immune cells and no appreciable fratricide.

Technical Details

The researchers identified KIR3DL2 (CD158k) as a viable target using single-cell RNA-seq data analysis and flow cytometric phenotyping. Findings indicated that KIR3DL2 expression is restricted to a subset of mature NK cells and a minor fraction of $CD8^+$ T cells, with negligible expression in other healthy adult tissues. Anti-KIR3DL2 CAR constructs (3DL2CARs) selectively redirected T cells against antigen-positive cell lines, triggering antigen-specific signaling, cytotoxicity, proliferation, and cytokine production.

To address the risk of tumor cell contamination in autologous CAR T-cell products, a two-tiered safety strategy was implemented:

- Immunomagnetic depletion of KIR3DL2-positive tumor cells.
- In vivo activation of a safety switch to prevent immune escape if malignant cells are inadvertently transduced.

Background & Context

While CAR T-cell therapy has transformed the treatment of multiple myeloma and B-cell malignancies, its application to T-cell lymphomas has been limited by a lack of safe target antigens. Targeting pan-T antigens is possible but carries a substantial risk of infection and immunosuppression.

Significance & Outlook

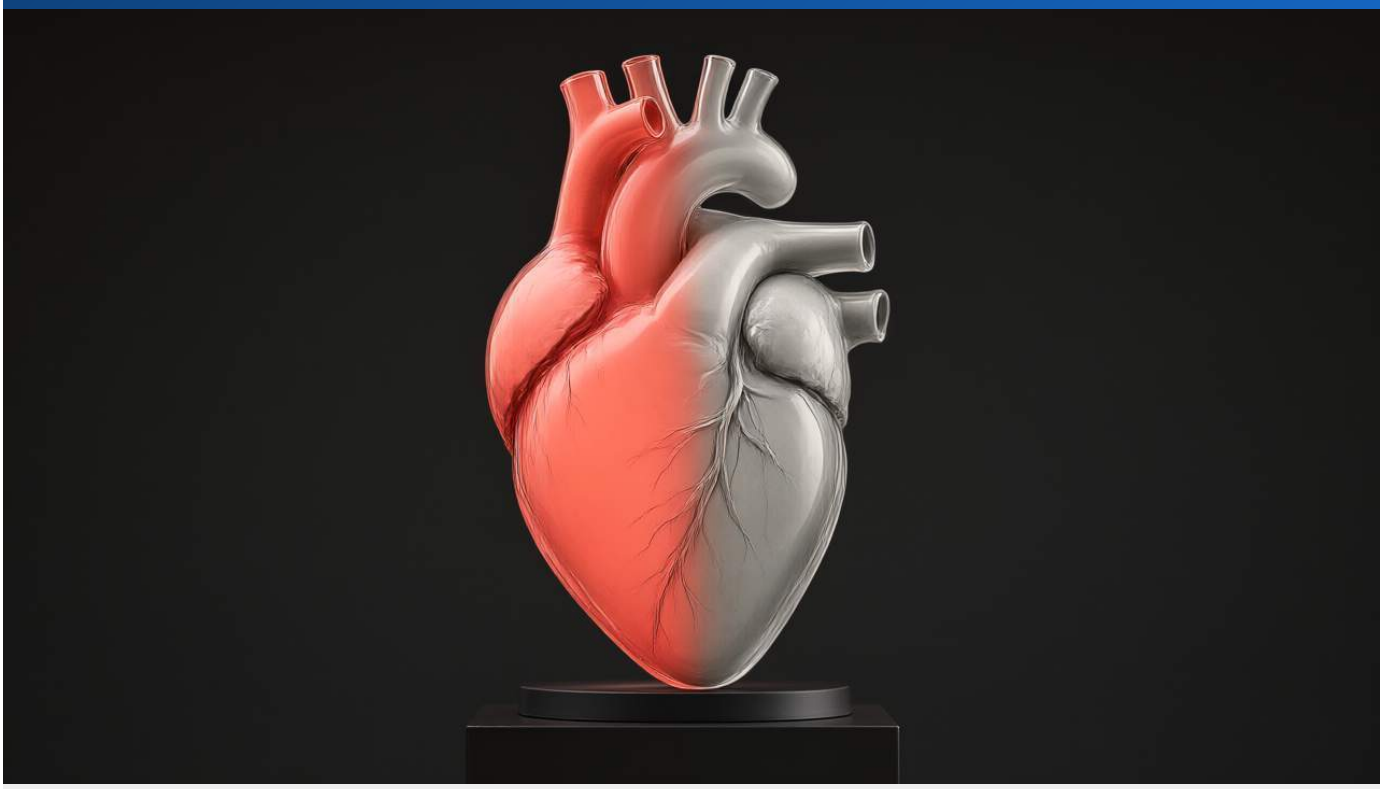
By targeting KIR3DL2, this approach offers a more precise alternative to pan-T-cell-directed strategies. The results support the clinical translation of 3DL2CART-cell therapy to address the critical unmet clinical need for patients with relapsed/refractory cutaneous and systemic TCLs.

Source: https://doi.org/10.64898/2026.11.05.764385

Collected: November 14, 2026 | Automated Research System (AI)

#12 Cancer Cachexia: Cardiac Wasting Signaling in hiPSC-CMs

Published November 09, 2026 JCI Insight USA



OVERVIEW

Researchers have developed a human induced pluripotent stem cell-derived cardiac myocyte (hiPSC-CM) platform to model cancer cachexia. The study identifies that tumor-induced cardiac wasting is driven by the suppression of the AKT/mTORC1/S6 signaling axis, leading to contractile dysfunction and metabolic dysregulation.

IN DEPTH

Key Findings

The study demonstrates that cancer cachexia induces significant wasting and dysfunction in human iPSC-derived cardiac muscle. Using a specialized modeling platform, researchers observed that cachectic hiPSC-CMs exhibit cellular atrophy, reduced contraction amplitude, and prolonged relaxation time. Additionally, these cells show an elevated oxygen consumption rate, indicating a state of metabolic dysregulation.

Technical Details

To simulate the effects of cancer cachexia, the research team utilized a human induced pluripotent stem cell–derived cardiac myocyte (hiPSC-CM) platform. The model was implemented using two primary methods:

- Conditioned media from tumor cells.
- Transwell coculture with C26 or HCT116 tumor cells.

Mechanistically, the wasting process is linked to the suppression of the AKT/mTORC1/S6 signaling axis. Specifically, the researchers noted a reduction in total and p-AKT levels, as well as a decrease in the p-S6/S6 ratio.

Background & Context

Cancer cachexia is a debilitating syndrome characterized by involuntary body mass loss and profound skeletal muscle wasting, which is associated with high mortality rates. While skeletal muscle is a primary focus of cachexia research, cardiac muscle wasting is also prominent in both cancer patients and cancer survivors.

Significance & Outlook

By elucidating the aberrant signaling pathways—specifically the AKT/mTORC1/S6 axis—this research provides a mechanistic understanding of how tumor cells trigger cardiac wasting. The development of the hiPSC-CM platform allows for the study of human-specific cardiac responses to cancer-induced wasting, offering a foundation for understanding the metabolic and contractile failures associated with the syndrome.

Source: https://doi.org/10.1172/jci.insight.203276

Collected: November 14, 2026 | Automated Research System (AI)

#13 Human iPSC-derived nociceptors: Impact of surface coatings on phenotype

Published November 09, 2026 Research Square



OVERVIEW

Surface coating materials significantly alter the morphology, transcriptomic identity, and functional activity of human induced pluripotent stem cell (iPSC)-derived nociceptors (iNocs). The study identifies specific effects of laminin, poly-L-lysine/laminin, and Matrigel on cellular maturation and firing rates. These findings suggest that optimizing extracellular matrix compositions is critical for improving the physiological relevance of human pain models.

IN DEPTH

Key Findings

The choice of surface coating material acts as a critical regulator of human iPSC-derived nociceptor (iNoc) phenotypes. Researchers observed significant differences in cellular morphology, the size of cellular clusters, and transcriptomic signatures depending on the substrate used. Specifically, iNocs cultured on poly-L-lysine/laminin exhibited the highest spontaneous discharge activity and firing rates.

Technical Details

The study utilized a combination of indirect immune fluorescence microscopy, RNA sequencing, multi-electrode array recordings, and single-cell patch-clamp techniques to compare three coatings: laminin, poly-L-lysine/laminin, and Matrigel. The results revealed distinct biological profiles:

- **Matrigel:** Promoted biological gene ontologies associated with mesenchymal pathways and extracellular matrix remodelling.
- **Laminin:** Enhanced proliferative gene sets as well as synaptic and neuronal processes.
- **Poly-L-lysine/laminin:** Increased spontaneous discharge activity.

Conversely, single-cell patch-clamp recordings indicated that coating materials had only minor effects on the basic electrophysiological parameters and passive membrane properties of individual iNocs.

Background & Context

Human iNocs are essential tools for modelling pain and sensory neurobiology. However, achieving reproducibility and full maturation remains a technical challenge. While existing differentiation protocols primarily focus on the application of soluble factors, the influence of the extracellular matrix on these cells has remained insufficiently understood until this investigation.

Significance & Outlook

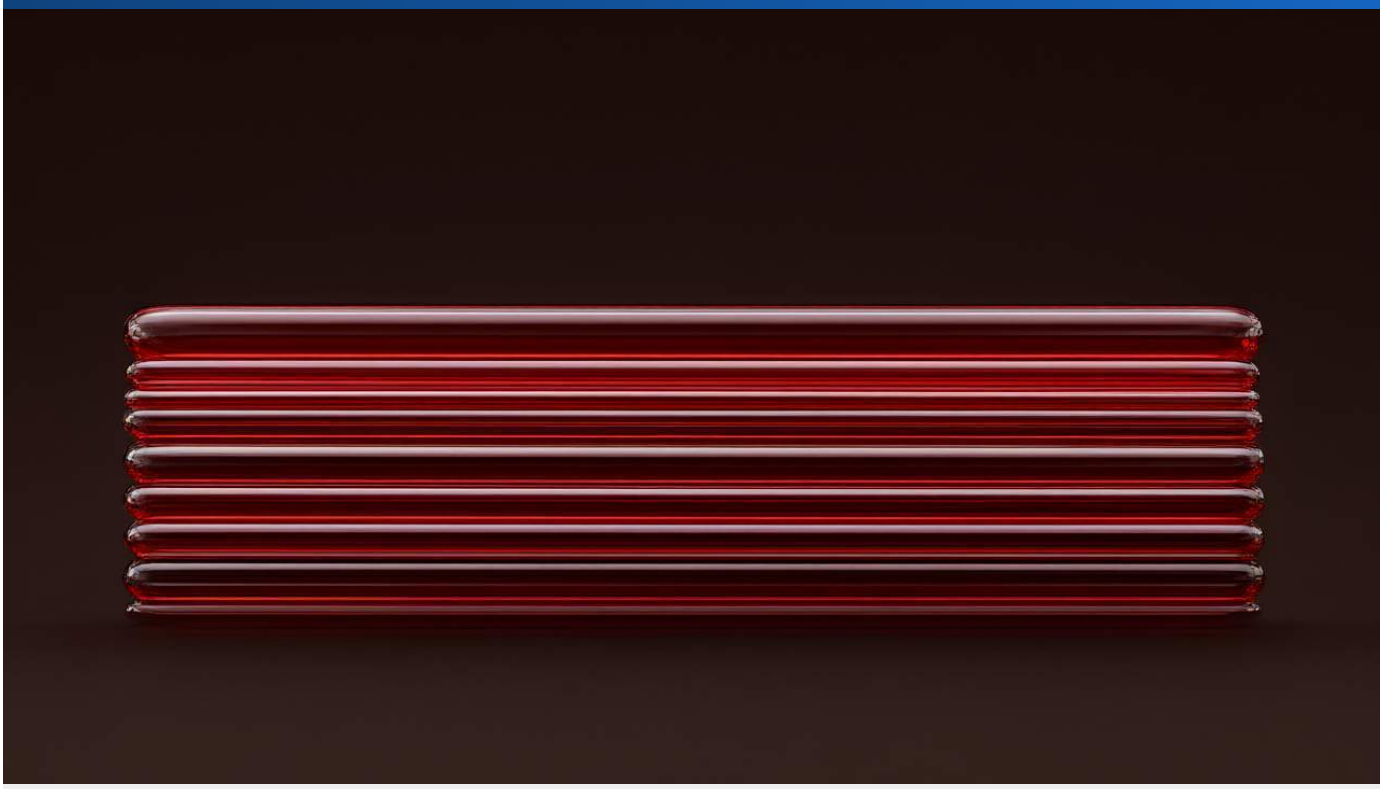
These results demonstrate that the extracellular matrix composition is a primary driver of iNoc identity and function. By optimizing surface coating substrates, researchers can better control the maturation and physiological relevance of translational human pain models, providing a more reliable platform for sensory neurobiology research.

Source: https://doi.org/10.21203/rs.3.rs-11207616/v1

Collected: November 14, 2026 | Automated Research System (AI)

#14 iPSC-CMs: Adult-like Thick-Filament Protein Stoichiometry

Published November 11, 2026 bioRxiv



OVERVIEW

Human induced pluripotent stem cell-derived cardiomyocytes (iPSC-CMs) exhibit thick-filament protein proportions that closely match adult human myocardium despite overall developmental immaturity. This preservation of stoichiometry supports the use of iPSC-CMs for studying sarcomeric diseases linked to protein dosage, such as MYBPC3-associated hypertrophic cardiomyopathy.

IN DEPTH

Key Findings

Research indicates that human iPSC-derived cardiomyocytes (iPSC-CMs) maintain adult-like stoichiometry of thick-filament proteins. While these cells generally cluster with fetal hearts in terms of their global shared proteome and retain fetal troponin I expression, specific protein ratios within the thick filament remain comparable to those found in adult cardiac regions.

Technical Details

The study utilized an integration of two independent quantitative proteomic datasets of iPSC-CMs, comparing them against published proteomes from fetal and region-resolved adult human hearts. The analysis focused on sarcomeric protein abundance and revealed the following:

- Myosin heavy-to-light chain ratios approached the expected 2:4 stoichiometry.
- The abundance of myosin-binding protein relative to myosin heavy chains was comparable across both iPSC-CMs and adult cardiac regions.

Background & Context

iPSC-CMs are known to retain immature sarcomeric isoforms, which has previously raised questions regarding their ability to accurately recapitulate the protein stoichiometry of the adult human myocardium. This study clarifies that developmental immaturity in the expression of sarcomeric isoforms does not necessarily result in altered proportions of thick-filament proteins.

Significance & Outlook

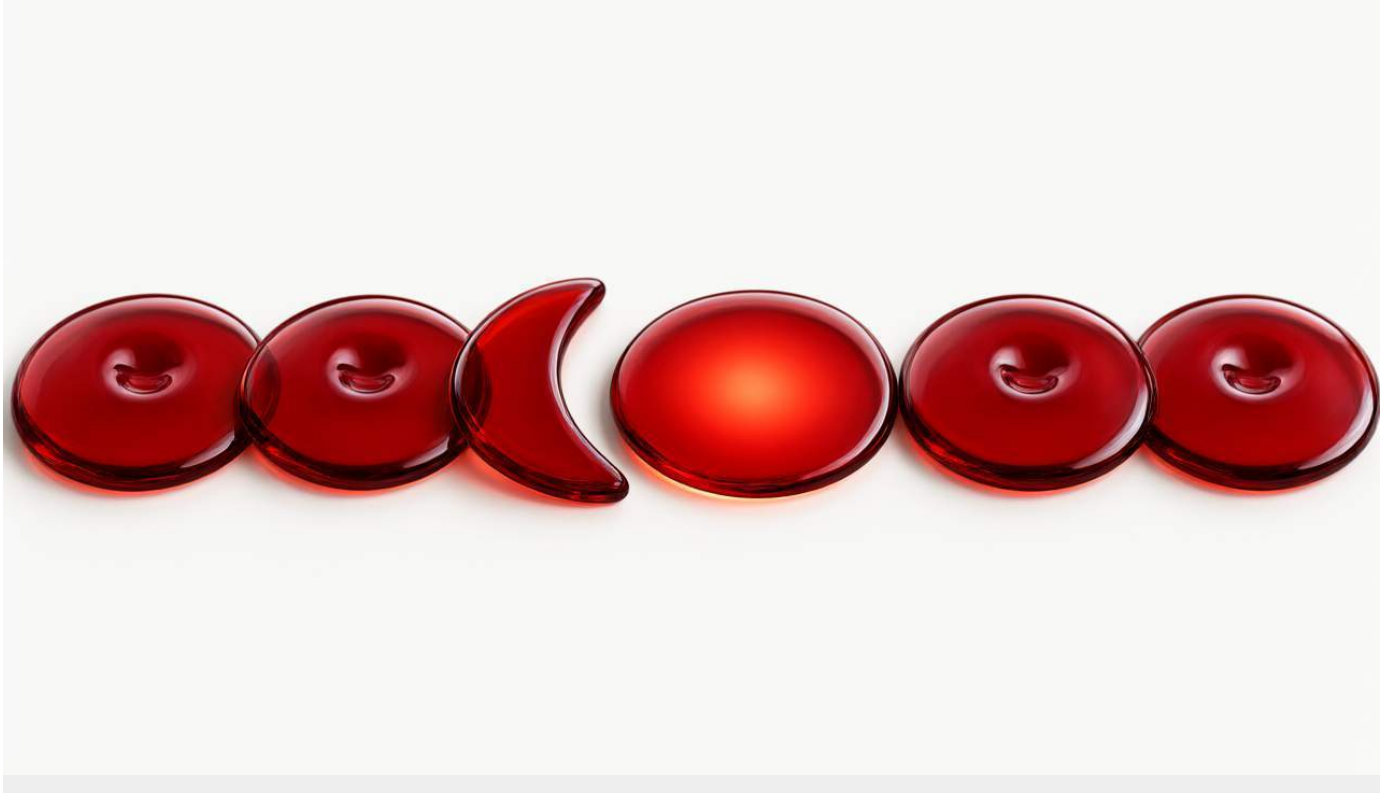
The finding that adult-like myosin-binding protein stoichiometry is preserved in iPSC-CMs provides a technical validation for their use in disease modeling. Specifically, these cells are suitable for investigating sarcomeric diseases associated with altered protein dosage, with a particular application in the study of MYBPC3-associated hypertrophic cardiomyopathy.

Source: https://doi.org/10.64898/2026.11.09.761920

Collected: November 14, 2026 | Automated Research System (AI)

#15 Kamau Therapeutics: Nula-cel RMAT designation and SCD trial data

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OVERVIEW

Kamau Therapeutics has received FDA Regenerative Medicine Advanced Therapy (RMAT) designation for nula-cel, a gene-corrected stem cell therapy for sickle cell disease. Preliminary Phase 1/2 RESTORE trial data shows all three patients treated with the optimized process achieved transfusion independence and zero vaso-occlusive events. This therapy aims to provide a lifelong cure by correcting the root mutation rather than compensating with fetal hemoglobin.

IN DEPTH

Key Findings

The U.S. Food and Drug Administration (FDA) has granted RMAT designation to nula-cel, an investigational stem cell medicine for sickle cell disease (SCD). This designation is based on preliminary clinical evidence from the Phase 1/2 RESTORE trial. In this trial, all three patients treated via the optimized manufacturing process achieved transfusion independence and experienced no vaso-occlusive events. Furthermore, two patients with over six months of follow-up demonstrated sickle hemoglobin levels below 20%, which is under the 30% threshold targeted for stroke prevention, showing a predominantly adult hemoglobin profile.

Technical Details

Nula-cel utilizes ex vivo homology-directed repair (HDR) to precisely engineer a patient's own long-lived blood stem cells outside the body. Unlike other therapies that induce fetal hemoglobin to compensate for the disease, nula-cel is designed to correct the sickle mutation itself, converting it into the normal adult β-globin sequence. This ex vivo approach allows Kamau Therapeutics to verify the quality, identity, potency, and safety of each cell product before it is returned to the patient. Because these engineered cells self-renew, the treatment is designed as a one-time intervention to provide durable, potentially lifelong benefits.

Background & Context

Sickle cell disease is an inherited disorder affecting approximately 100,000 people in the U.S. and millions globally. A mutation in the β-globin gene produces sickle hemoglobin (HgbS), leading to chronic hemolysis, pain crises, and organ damage. While existing therapies reduce pain crises, they do not restore native adult hemoglobin (HgbA) or consistently maintain HgbS levels below 30%, a level associated with stroke prevention in ASH guidelines.

Significance & Outlook

The RMAT designation allows Kamau Therapeutics to engage in more frequent interactions with the FDA to align on the path toward a registrational dataset. The company plans to request a multidisciplinary RMAT meeting to discuss endpoints for accelerated approval and the potential for priority review. Nula-cel has also previously received Fast Track and Orphan Drug designations from the FDA.

Source: https://markets.businessinsider.com/news/stocks/kamau-therapeutics-receives-fda-regenerative-medicine-advanced-therapy-rmat-designation-for-nula-cel-a-gene-corrected-stem-cell-therapy-for-sickle-cell-disease-1036755594

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#16 FDA Gene Therapy Guidance: Personalized Platform Approval Roadmap

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OVERVIEW

The FDA has proposed draft guidance to accelerate personalized gene therapies for ultrarare diseases by allowing the approval of a core platform rather than individual drug versions. This framework enables the customization of treatments for specific genetic mutations without requiring separate clinical trials for every variation.

IN DEPTH

Key Findings

The FDA has released proposed draft guidance that establishes a regulatory pathway for individualized gene-editing therapies. The core of this proposal is the "individualized feature space," which defines acceptable variations between versions of a drug. If a new version of a therapy falls within these parameters and maintains the same core features, it can be customized for new patients after approval without the need for an entirely new clinical trial.

Technical Details

The proposed framework introduces the concept of an "umbrella clinical trial," allowing different versions of a gene-editing drug to be tested under a single trial. Under this system, all variations of the therapy would share the same name on the drug label. This approach addresses the technical challenge where a single disease is caused by multiple different genetic mutations—described as "single-letter typos" in a genetic sequence—requiring a "GPS" change to the drug's search-and-find function to target different mutations while the underlying drug platform remains the same.

Background and Context

The guidance was shaped by collaborations between the FDA and scientists including Kiran Musunuru (Penn Medicine) and Rebecca Ahrens-Nicklas (Children’s Hospital of Philadelphia). The need for this shift was highlighted by the development of a custom gene-editing therapy for an infant, Baby KJ, which corrected a mutation driving a rare metabolic disease and improved liver function. Previously, the regulatory system required separate clinical trials for every version of a therapy, a process deemed effectively impossible for diseases with hundreds of possible mutations. Additionally, Musunuru and Ahrens-Nicklas worked on therapies for phenylketonuria (PKU), a condition that currently requires lifelong low-protein diets and injections.

Significance and Outlook

This regulatory shift is expected to make the delivery of personalized medicine feasible for ultrarare diseases. To support this scalability, a trio of researchers—Musunuru, Ahrens-Nicklas, and Lindsey George—received a $39 million federal award last summer to develop a scalable platform for treating rare genetic liver-related diseases in children. Future focus remains on defining requirements for postmarketing surveillance to monitor long-term safety and effects.

Source: https://www.inquirer.com/health/fda-guidelines-personalized-gene-therapy-rare-diseases-philadelphia-scientists-20261108.html

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