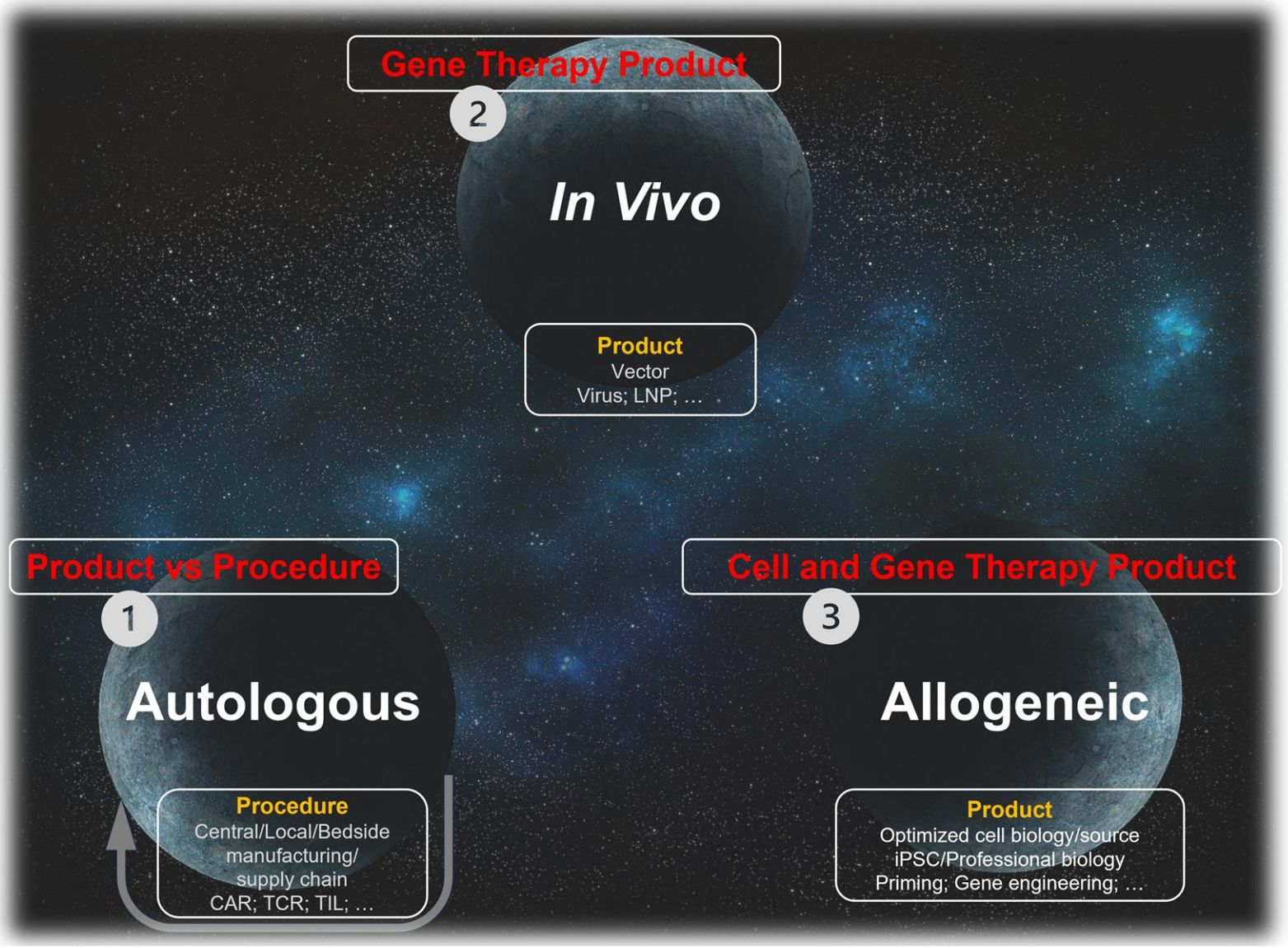
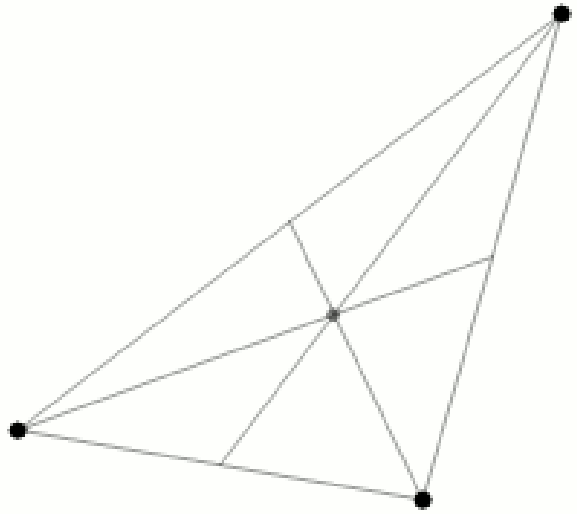
KIJI
Therapeutics

April 2026

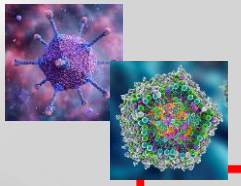
The 3 body problem: The future of C&GT





The future of C&GT: Three modalities !!

Kiji Therapeutics



Gene Therapy Product

In vivo

Product: Vector (virus; LNP; …)

Off-the-Shelf Supply Chain

Product vs Procedure

Autologous

Procedure
Local/bedside manufacturing/ supply chain (CAR; TCR; TIL; …)

1

2

3

BIOLOGY
Potency; Efficacy

C&GT

QUALITY
CMC; Capacity

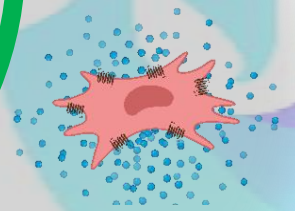
Feasibility
CoG; Clinical

Cell and Gene Therapy Product

Allogeneic

Product: Optimized cell biology
Cell source (**iPSC**, …)
Professional biology
(Priming; **Gene engineering**; …)

iMSC



eNK

iMφ

Kiji Therapeutics - highlights

Value Proposition	Biology and manufacturing optimized gene engineered, off-the-shelf iPSC derived cell therapy with differentiated MoA aiming to compete with biologics, on efficacy and business model, for the management of autoimmune diseases.
Kiji Therapeutics	Proprietary multi-gene engineered cell therapy for unmet medical needs in autoimmune diseases - **large indication:** IBD, Psoriasis, via IIT trial (GvHD) as clinical proof-of-concept • Seasoned mgmt. team with leaders in cell and gene therapy • Full package pre-clinical MoA data (multiple animal models) • Followed by early PoC IIT trial (GvHD) entering FIH (1H2026)
Gene engineered i/MSC platform	CXCR4 and IL10 gene engineered MSCs for improved therapeutic benefit: - Proof-of-concept showcase: Non-dilutive Grant Fully funded Ad-MSC PoC clinical study in 3rd line in GvHD (1H2026) with a Best-in-Class approach superior to FDA approved Ryoncil™ - **Lead pipeline:** iPSC derived iMSC platform with unique MoA for IBD and Psoriasis

The story of the MSCs – US and Global

Country/Region	Product(s)	Indication(s)
USA	**Ryoncil (remestemcel-L)**	**Pediatric SR-aGVHD**
Canada, New Zealand	Prochymal	Steroid-resistant GvHD in children
Europe	Holoclar; Alofisel (withdrawn Dec 2024)	Limbal stem cell deficiency (ocular burns) Complex perianal fistulas in Crohn's disease
South Korea	Queencell, Cellgram-AMI, Cupistem, Cartistem, NeuroNataR	Cellgram-AMI, Cupistem, Cartistem, NeuroNataR
Japan	Temcell HS, Stemirac	GvHD, spinal cord injury
India	Stempeucel	Critical limb ischemia
Iran	MesestroCell	Osteoarthritis
China	**Ruibosheng (amimestrocel)**	**Umbilical cord-derived MSC, conditional approval for GvHD**

Ryoncil® (remestemcel-L-rknd) Suspension for IV infusion

铂生生物 PLATINUM LIFE

Year	Event
1993	Osiris founded; MSCs characterized by Dr. Caplan1
2009	Osiris completes phase III trial of Prochymal in GVHD
2012	Prochymal receives conditional approval in Canada for pediatric GVHD
2013	Mesoblast acquires Osiris' MSC business, including Prochymal
2024	**FDA approves Ryoncil** (remestemcel-L) for pediatric steroid-refractory GVHD
2025	**Ryoncil becomes available to patients in the U.S.**

From left: Dr. Arnold Caplan (the "father of MSCs") and Kevin Kimberlin, co-founders of Osiris Therapeutics, Inc.

Mesoblast Ryoncil ® has a target price of 1.5 M USD and **US sales of 46.4 M USD** in the first 9 months since launch

MSCs for GvHD in Europe – Conditional approval under HE

PHARMAZEUTISCHE ZEITUNG

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Start / Pharmazie

Mesenchymale Stromazellen

Obnitix ist kein unbeugsamer Gallier

Asterix, Obelix und Obnitix®? Nein, hinter dem letzten Namen verbirgt sich kein unbeugsamer Gallier, sondern eine neue Therapie zur Behandlung schwer erkrankter Patienten nach einer allogenen Stammzelltransplantation.

Mesenchymal stromal cells

Obnitix is not an indomitable Gaul

Asterix, Obelix, and Obnitix®? No, the last name doesn't refer to an indomitable Gaul, but rather to a new therapy for treating seriously ill patients following an allogeneic stem cell transplant.

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journal homepage: www.isct-cytotherapy.org

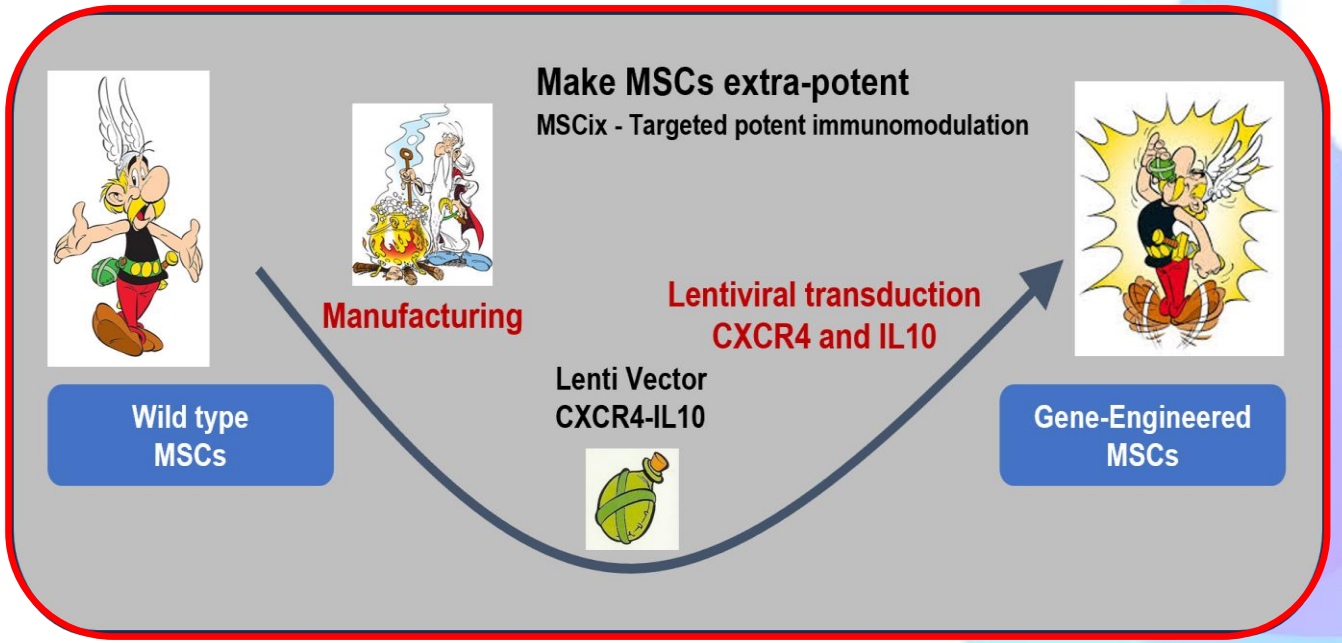
ELSEVIER

International Society ISCT Cell & Gene Therapy®

ISCT Committee Paper

Patient access to and ethical considerations of the application of the European Union hospital exemption rule for advanced therapy medicinal products

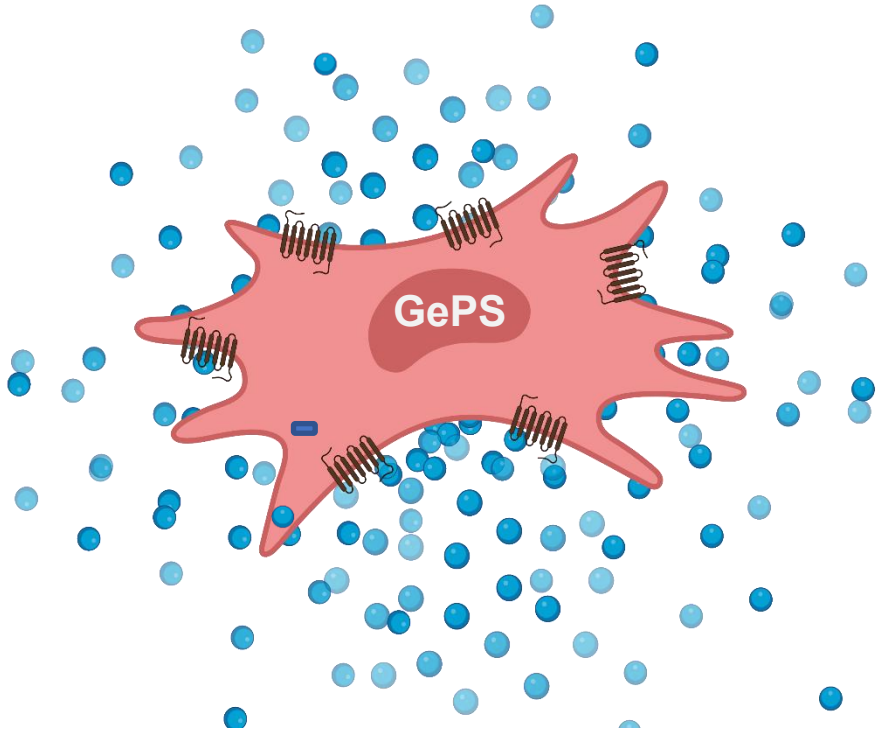
Natividad Cuende[1,*], Rachele Ciccocioppo[2,*,**], Miguel Forte[3,4], Jacques Galipeau[5], Laertis Ikonomou[6], Bruce L. Levine[7], Alok Srivastava[8,9], Patricia J. Zettler[10,11,12]



OBNITIX: MSC under **Hospital Exemption** in Germany and compassionate use elsewhere (Spain and other)

Targeted potent immunomodulation

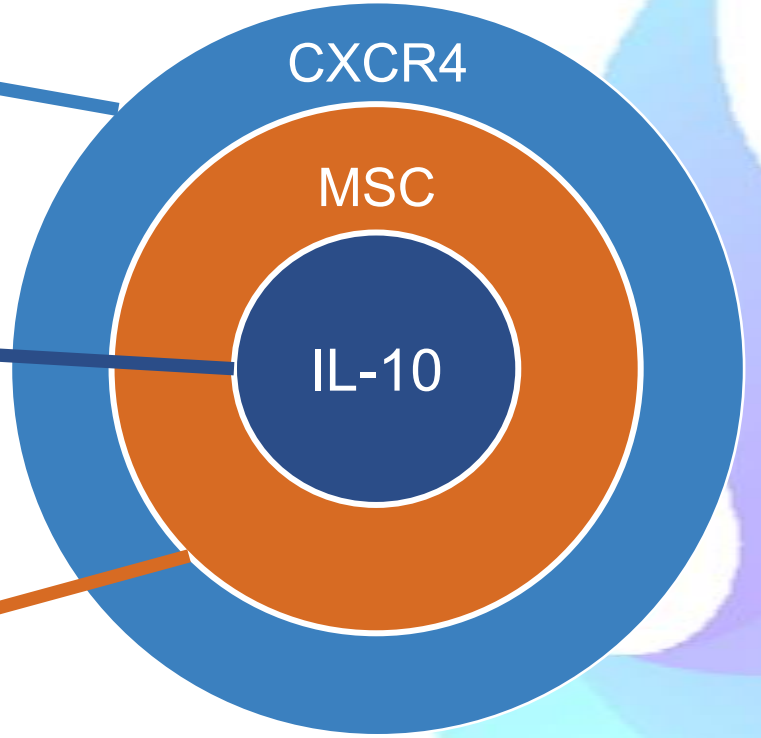
Gene engineering MSC (iMSC) to upgrade targeting & efficacy



- Targeting inflammation (SDF-1)
- Critical chemokine receptor

- Immune Master Switch (IL-10)
- Promotes Th2 (regulatory)

- Potent immunomodulation
- Clinically validated



IL-10

CXCR4

CXCR4 provides homing to SDF-1, which is present at site(s) of inflammation, (i)MSC secretes immunomodulatory IL-10 for enhanced therapeutic effect

KJ02 3rd gen MSC competitive to Biologics in price & efficacy

Kiji Therapeutics

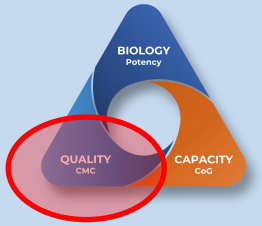
1st Generation MSC Tx

Ryoncil
Wild-type MSC
mesoblast

GvHD FDA approved
1,5 M USD/patient

Ryoncil (remestemcel-L-rknd) Suspension for IV infusion

FDA validated wild-type efficacy in GvHD

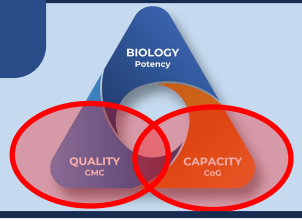


2nd Generation MSC Tx

Cymerus
iPSC derived MSC
cynata therapeutics

GvHD Ph III
Target: 300 k USD/patient

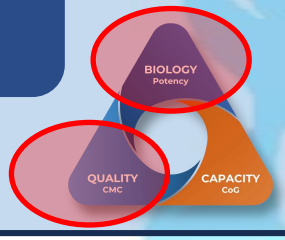
Scalability; Consistency
Lower cost



KJ01
Gene engineered MSC
Kiji Therapeutics

GvHD IND ready

Increased Efficacy

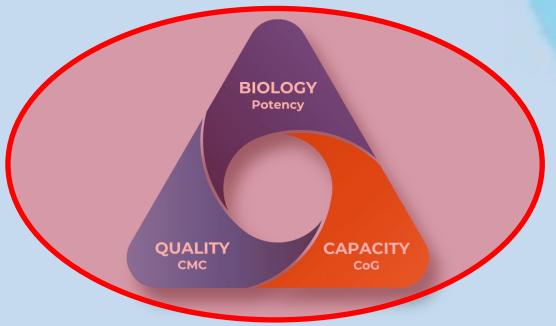


3rd Generation MSC Tx

KJ02
Gene engineered and iPSC derived
Kiji Therapeutics

IBD, Psoriasis, CNS – R&D

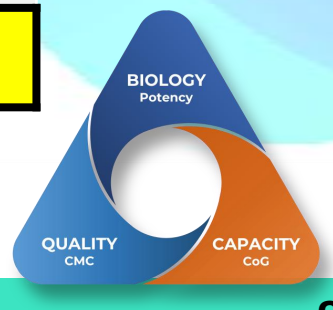
Increased Efficacy
Scalability; Consistency
Lower cost



Actual and projected Cost of Goods (COGS) KJ01 and KJ02

	KJ-01			KJ-02
	Small batch 2D	Full batch 2D	Full batch 3D	Full batch 3D iPSC
Source	Donor adipose tissue (lipoaspirate)			iPSC Master Cell Bank (MCB)
Number of doses	24	84	800+	1,500-2,000
Number of patient treatments (4 doses for GvHD*)	6	21	200+	400-500
Use	FIH Clinical trials	Clinical trials	Clinical trials / Commercial	Commercial/large indication
Cost per Treatment	€65k	€50k	€5-10k	€1-5k

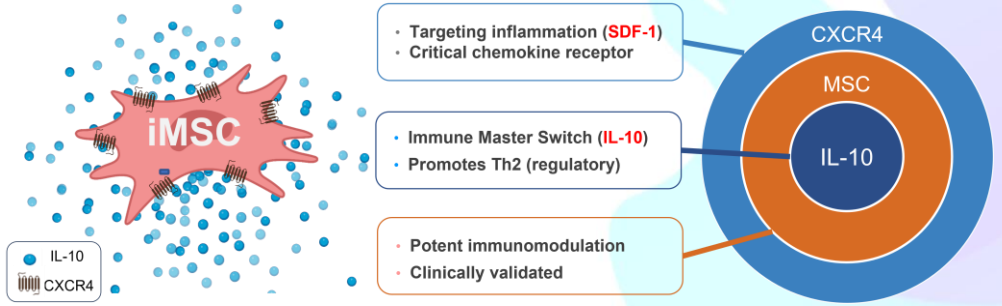
*Number of doses per treatment may vary per indication



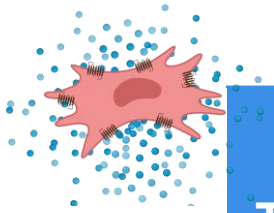
Resetting autoimmune diseases with gene engineered iMSCs - Tregs and Macrophage

- **Reset the innate immune system through Tregs and Macrophage re-polarization** for long-term clinical benefit with extensive pre-clinical MoA data in multiple positive animal models

- **Milestones:**
  - **Clinical data** in GvHD in **2026/27**
  - iPSC derived **iMSC** platform in **2027**
  - Clinical development for IBD, Psoriasis in **2028/2029**



Pipeline



	Technology (Allogeneic; Off-the Shelf)	Indication	Stage				
			Discovery/ Pre-clinical	IND/CTA enabling studies	Clinical		
					IND/CTA	FPFV	Data
KJ02	iPSC - iMSC (IL10/CXCR4) R&D grade	**IBD**	IND package in development		1H27	1H28	2029
		Psoriasis	R&D; Pre-clinical				
KJ01	Donor Ad-MSC (IL10/CXCR4) GMP grade	**SR-aGvHD**	IND package available		2H25	1H26	1H27

Multiple clinical indications within the next 3 to 5 years

Kiji Therapeutics value proposition

KJ-02

Lead asset – Large indication – Large commercial value
R&D grade iPSC derived; Lentiviral transduced; HLA KO
Fully characterized *in vitro* and equivalent to donor derived transduced MSCs
New type of construct with strong differentiated potential in IBD and other autoimmune conditions

Targets IBD with a differentiated long term/innate immune system resetting MoA (peer review published) for induction and maintenance of remission in Crohn's Disease

Positioned to compete with biologics as off-the-shelf; low CoG; IV clinically convenient; long spaced administration product;

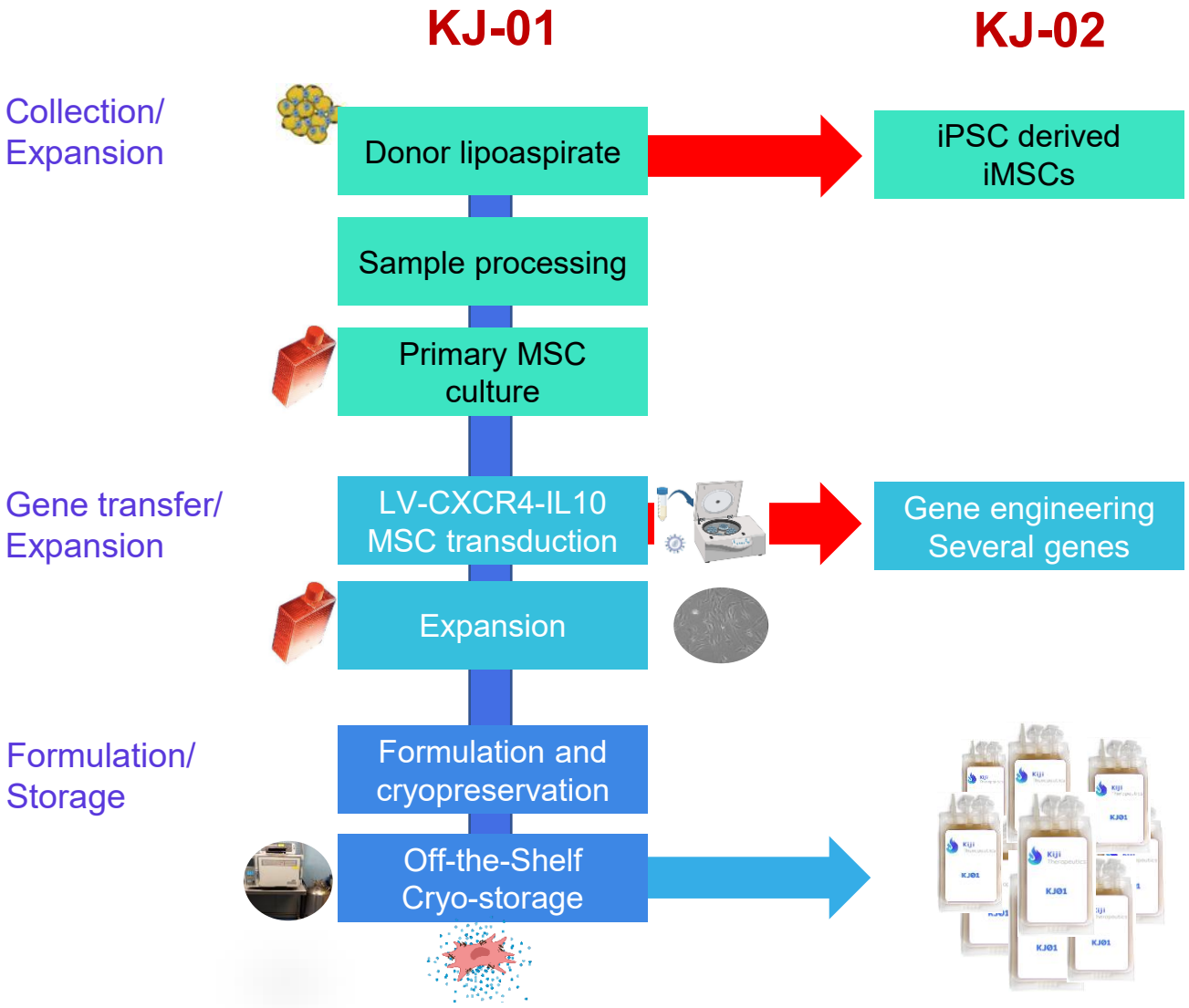
KJ-01

Proof-of-concept showcase (fully grant funded)
Full GMP grade ready for clinic trial; Donor derived MSC lentiviral transduced
Extensive pre-clinical MoA data in vitro and multiple animal models
Will deliver PoC data supportive of differentiated gene engineered MoA in Human
Targets refractory acute GvHD with CTA being submitted; Clinical trial fully funded

Best-in-class fast follower of Mesoblast Ryoncil®
Model: Low patient number (Orphan indication) and higher cost / willingness to pay (low volumes)

Product/Platform overview

Allogeneic, cryopreserved, off-the-shelf; iPSC derived engineered cell product

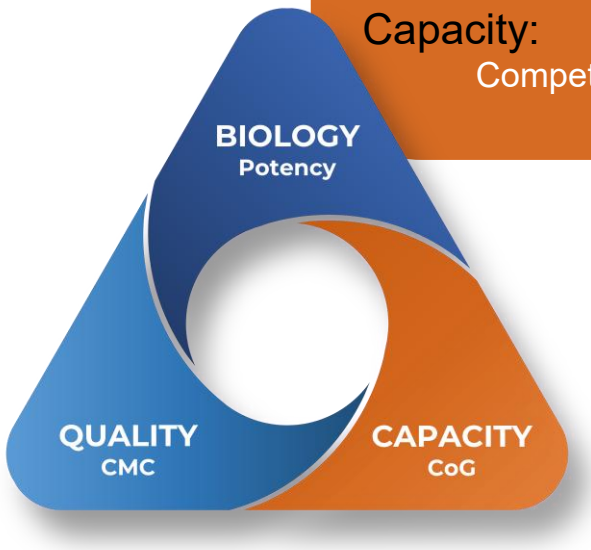


Kiji strong differentiated positioning

Biology:
- Increased therapeutic efficacy through gene engineering

Quality:
- Consistency and Clinically convenient; Potency

Capacity:
- Competitive CoG

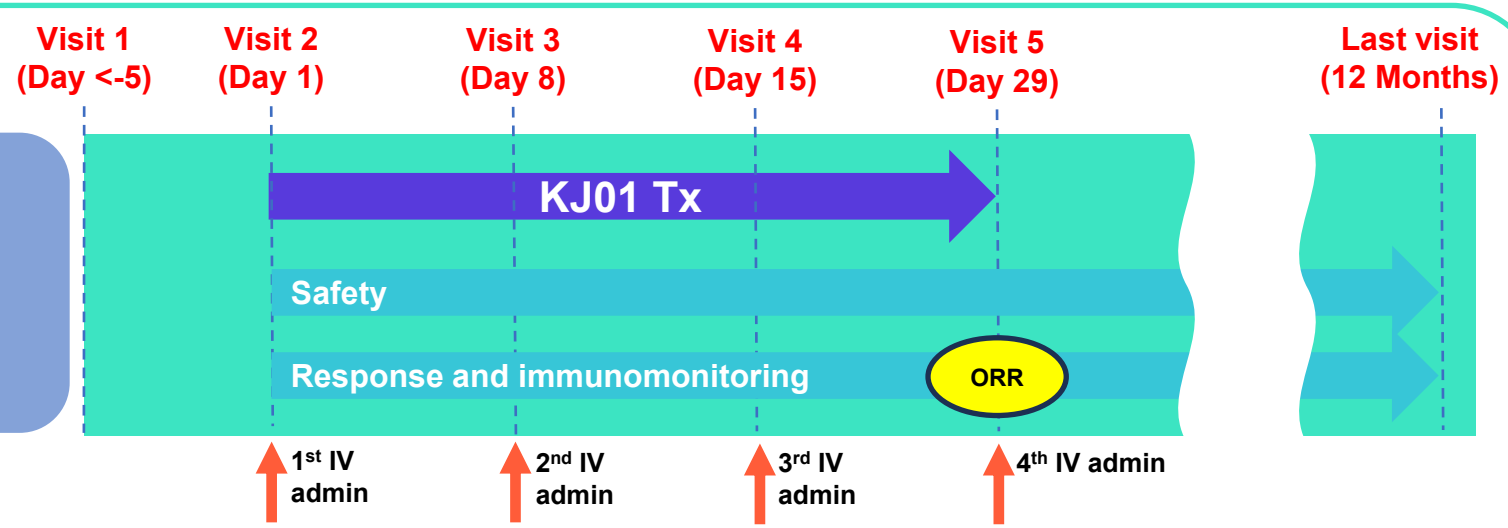


KJ-01 Phase I/IIa – 3rd line aGvHD IIT (PoC) -1H2026

Kiji Therapeutics

Inclusion:
N = 18 Patients (18-75 years)
Grade II – IV aGvHD
Steroid refractory,
Ruxolitinib intolerant or refractory

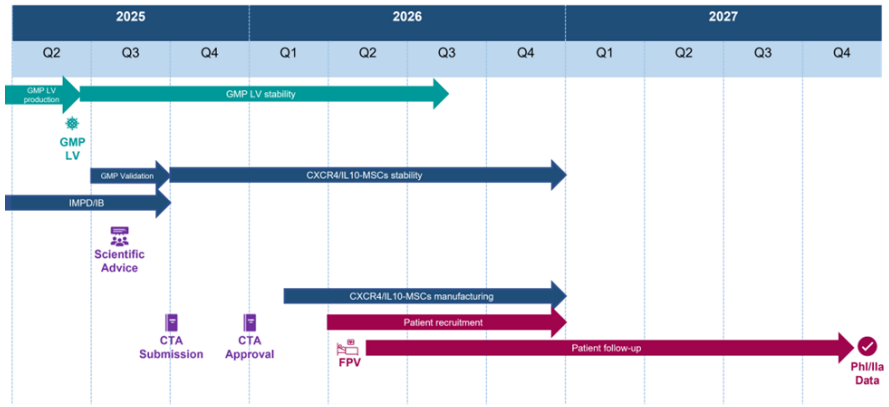
Target dose:
1.8-2 x 10^6 cells/Kg 4 times



Low dose cohort pts 1 to 3	0.9-1 x 10^6	0.9-1 x 10^6	1.8-2 x 10^6	1.8-2 x 10^6	KJ01 cells/Kg
Target dose cohort pts 4 to 15	1.8-2 x 10^6	1.8-2 x 10^6	1.8-2 x 10^6	1.8-2 x 10^6	KJ01 cells/Kg

Primary endpoint:
Safety

Secondary endpoints:
Overall Response Rate (ORR) at 28 days
Response status (CR; PR; OR) at 28, 100 days & 12 months
Overall Survival (OS) at day 100 and 12 months
Immune biomarkers



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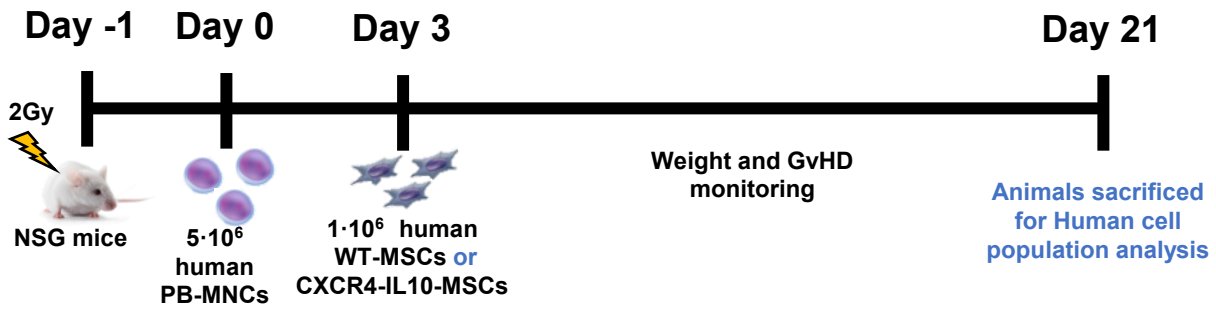
Ciemat Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas

KJ01 superior to MSC against GvHD *in vivo*

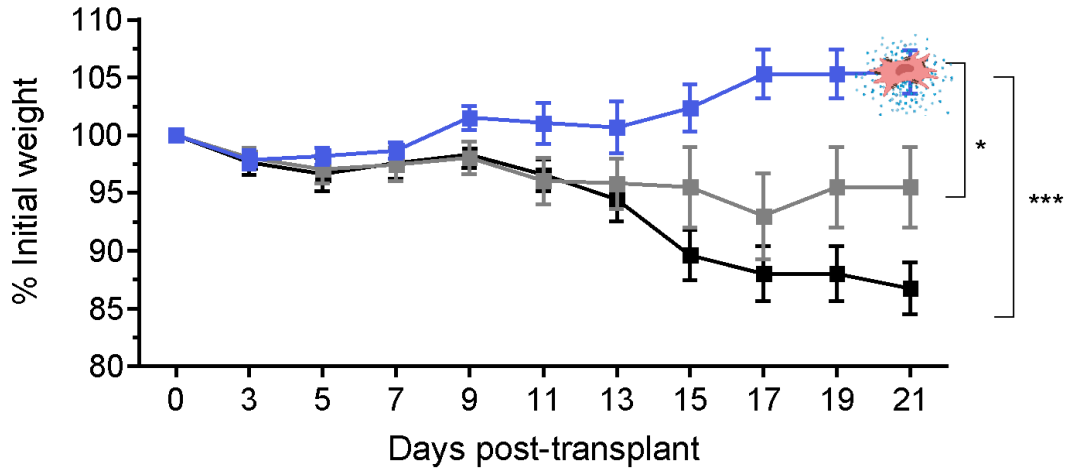
Kiji Therapeutics

Humanized GvHD mouse model

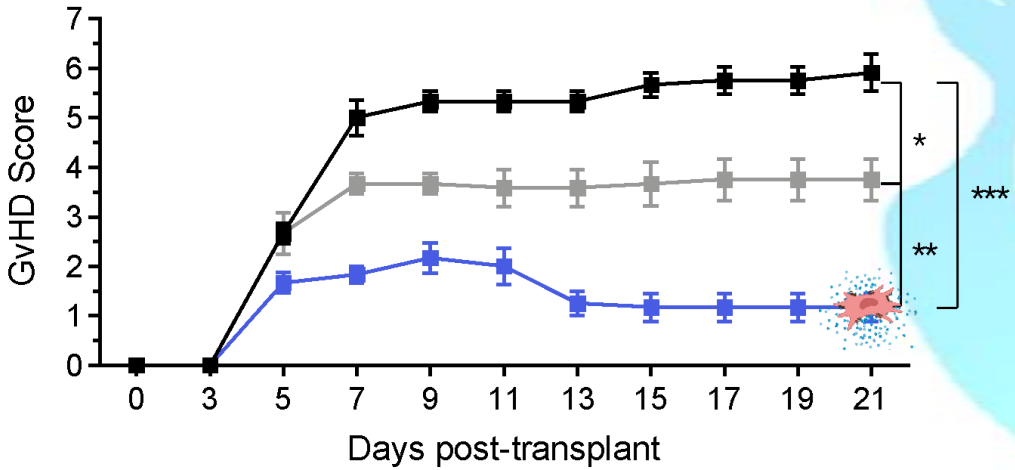


IL10/CXCR4 MSCs therapy superior to wild type MSC

Weight evolution



GvHD clinical score



NO WEIGHT LOSS

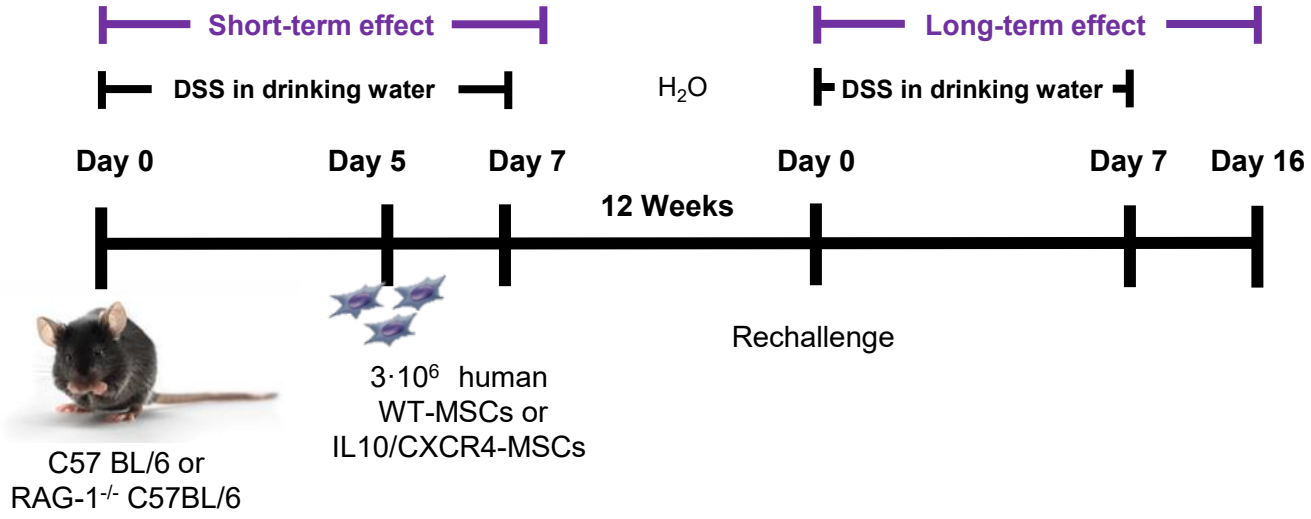
LESS SEVERE SIGNS OF ILLNESS

*p < 0.05; **p < 0.01; ***p < 0.001

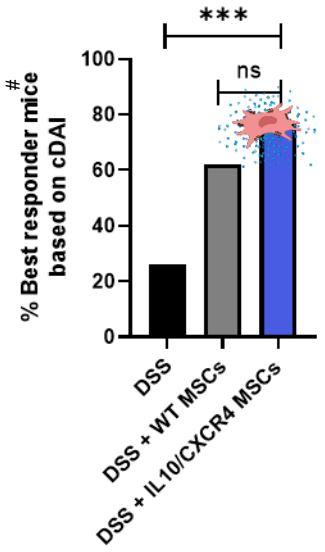
Improved efficacy in IBD with MSCs

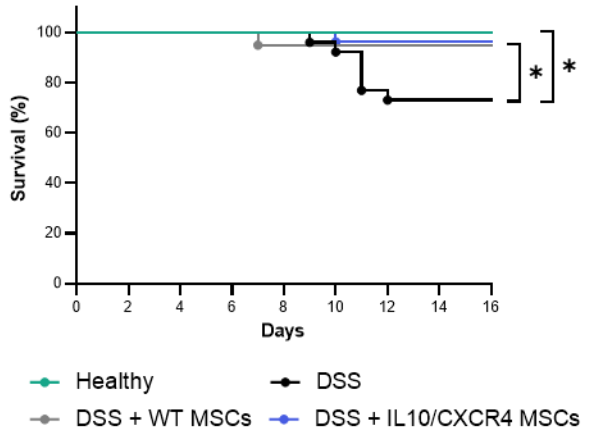
Kiji Therapeutics

DSS-induced colitis mouse model

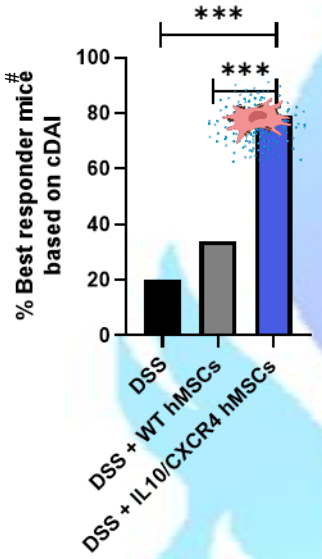


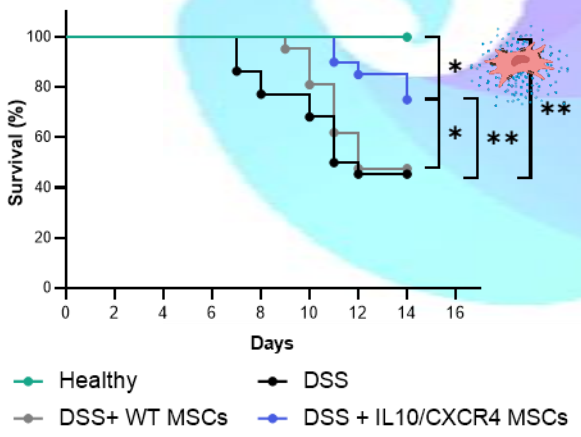
Short-term effect





Long-term effect





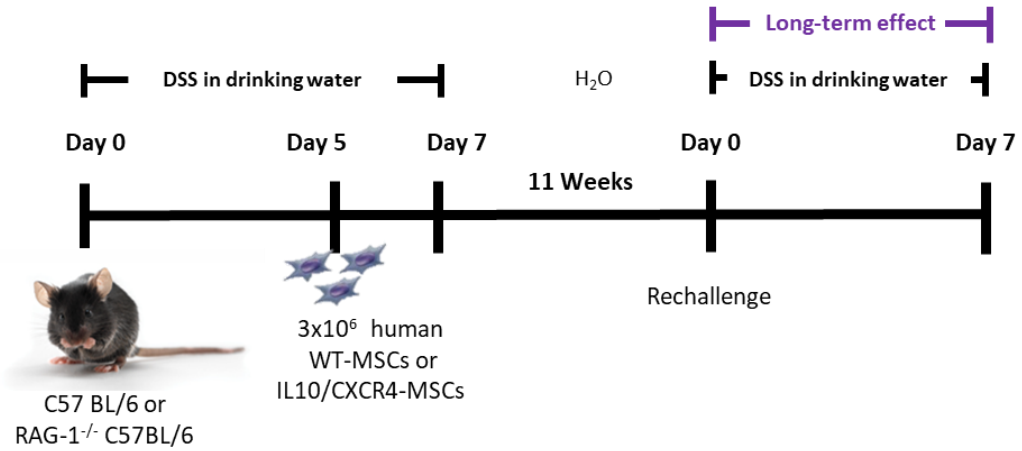
Improved efficacy with IL10-CXCR4
Long-term effect
Potential effect on innate immune system

Cumulative Disease Activity Index; Best reponders (mice at top 25th percentile of CDAI)

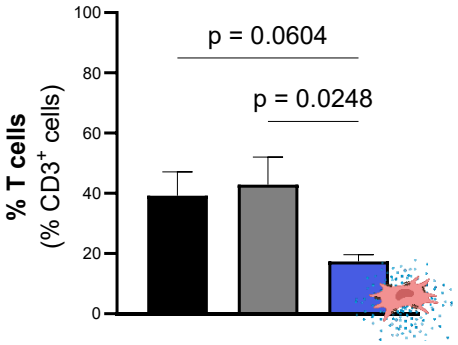
IL10/CXCR4 MSCs Long-term Efficacy in IBD

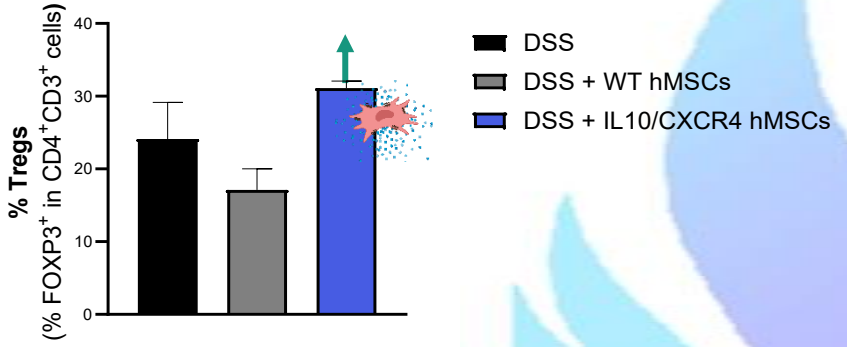
Kiji Therapeutics

DSS-induced colitis mouse model

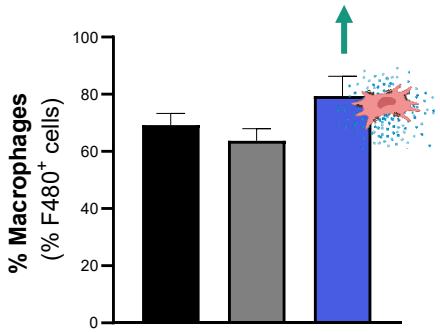


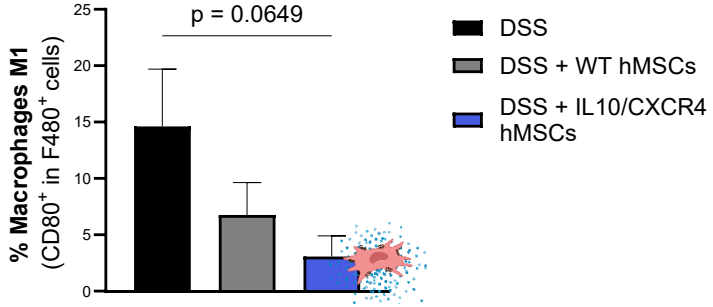
T Cells



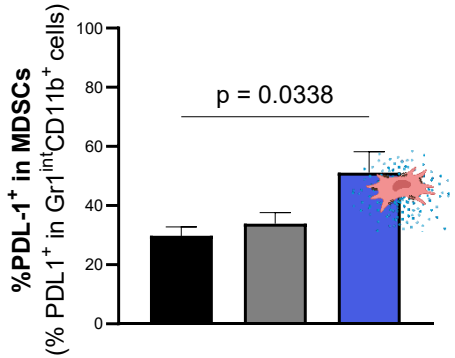


Macrophages



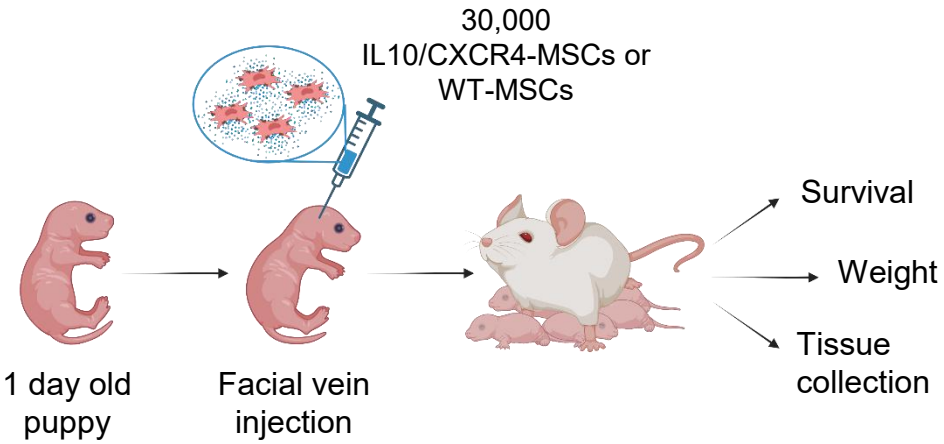


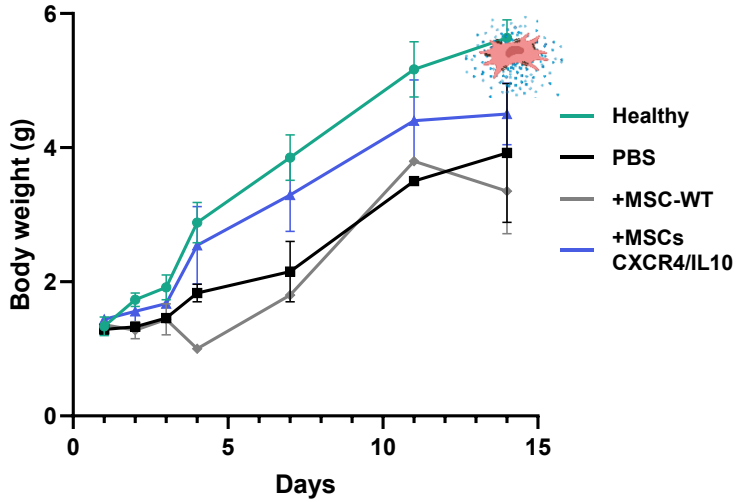
Myeloid-derived suppressor cells

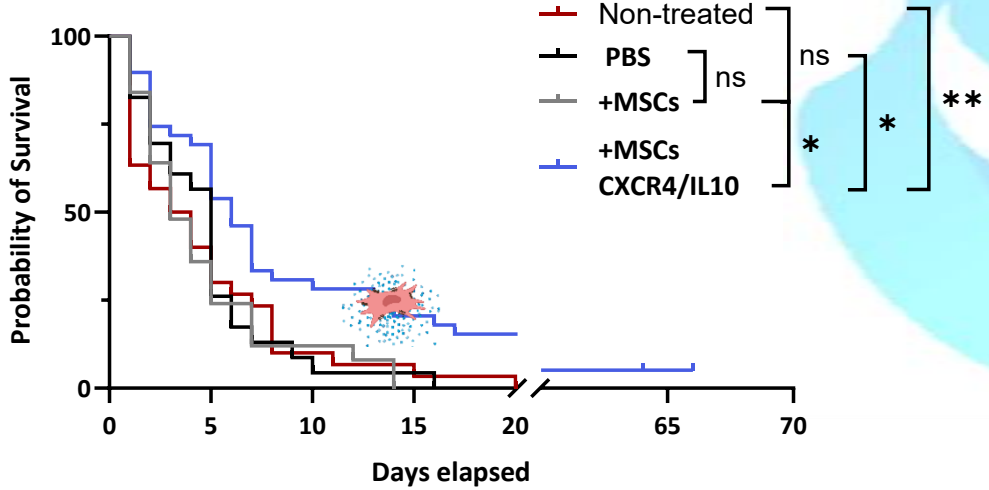


IL10/CXCR4 MSCs therapy imprints an immune memory-like response that confers long term sustained protection

Improved efficacy in skin inflammation MSCs

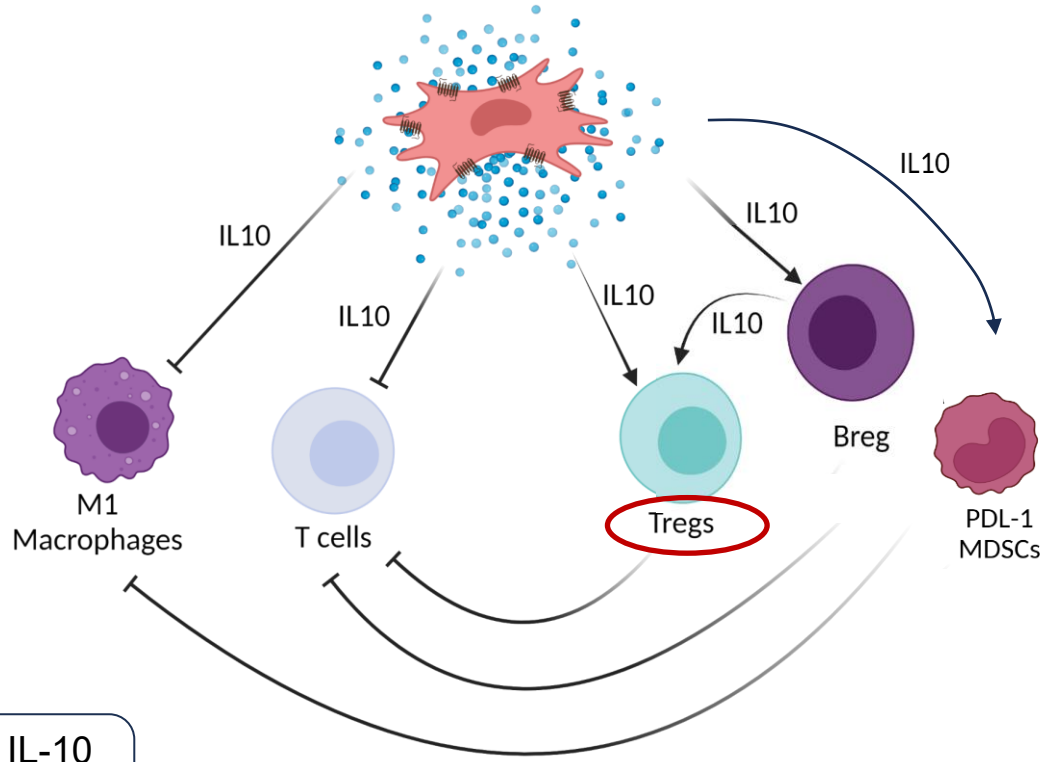






Increased survival in Col7a1 Hypomorphic mice treated with IL10/CXCR4-AdMSCs versus wild type MSCs

KJ01 and KJ02 immunomodulatory MoA
Cytokines; T_{reg}; B_{reg}; Macrophages; MDSC
Kiji Therapeutics data



Serum anti-Inflammatory cytokine profile

- Reduced (↓) levels of pro-inflammatory cytokines:
  - IFNγ; IL17α; IL1β; IL8; IL12; TNF
- Increased (↑) levels of anti–inflammatory cytokines:
  - IL10; TGFβ; IL6

Blood anti-inflammatory lymphocyte profile

Human CD4 and CD8 Lymphocytes

- ↑T central memory vs ↓T effector memory
- ↓T effector vs ↑T naïve cells

Regulatory T and B profile in spleen

T Lymphocytes showing a lower TH1/Th2 ratio

- ↑IL10 Treg and ↓IFN γ Inflammatory T cells

Spleen human Breg Lymphocytes

- ↑IL10 transitional Breg and ↑memory Breg

Macrophages and MDSC

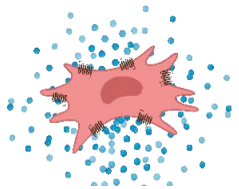
Impact on innate immunity

- Macrophages (↓M1 and ↑M2)
- ↑Myeloid Derived Suppressor Cells

KJ01 and KJ02 Benefits : Evidence summary

Kiji Therapeutics

KJ01/2



CXCR4

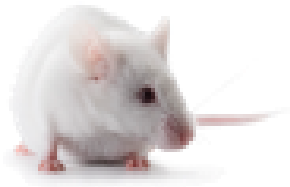
IL10

Preclinical *in vitro* evidence:

- *In vitro* migration
- *In vitro* immunomodulation

Clinical animal models evidence:

- GvHD; IBD; Epidermolysis Bullosa



In vivo distribution and MoA:

- Engineered MSC biodistribution
- Tissue target IL10 identification
- Cytokine switch from pro-inflammatory to anti-inflammatory
- Effector T-cell CD4+ & CD8+ subpopulations
- Other cell populations (macrophage and MDSC)

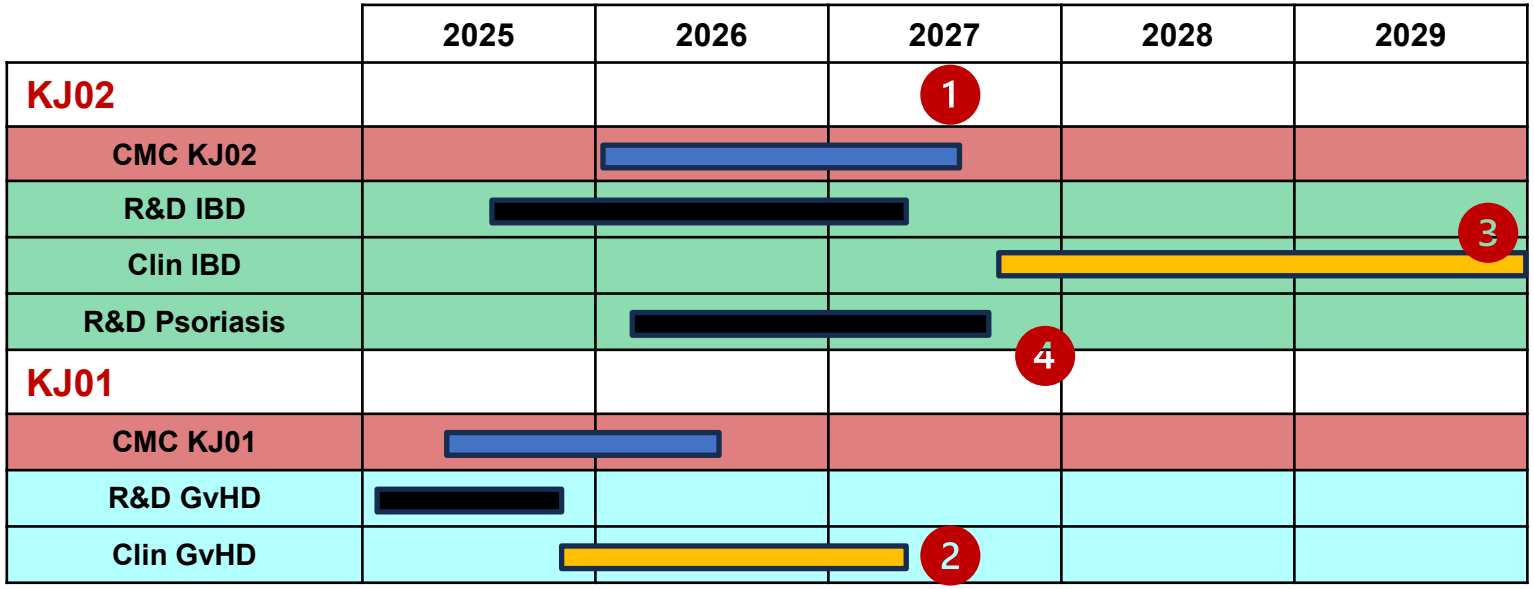
Short-term benefit:

↑ T_{reg} and ↑ B_{reg}
↑ Immunomodulation via Th1 ➔ Th2
↓ Inflammation via Th1 ➔ Th2

Long-term benefit:

Impact on innate immunity
Macrophages (↓ M1 and ↑ M2)
↑ Myeloid Derived Suppressor Cells

Use of proceeds/operational plan and milestones
Base scenario for 2025-2030



Milestones

1. iMSC platform **(2027)**
2. Clinical data GvHD **(2027)**
3. Clinical data in IBD **(2029/30)**
4. Pre-clinical Psoriasis **(2028)**

Led by an experienced and impactful management team

Kiji Therapeutics

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CEO

Anthony Ting, PhD
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Yen Wu, PhD
CBO

Maria Fernandez Garcia, PhD
Dir R&D / PM

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Bone Therapeutics
Bristol Myers Squibb
zelluna IMMUNOTHERAPY
Alliance for Regenerative Medicine
International Society ISCT Cell & Gene Therapy

Athersys
Takeda
Bone Therapeutics
Alliance for Regenerative Medicine
International Society ISCT Cell & Gene Therapy

Orphalan Innovation for Orphan Diseases
hemerion
Ribonexus

CHARITÉ Universitätsmedizin Berlin
AdBio partners
MAX DELBRÜCK CENTER
SurBio+
Humboldt-Universität zu Berlin
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Ciemat
cnio stop cancer
UAM Universidad Autónoma de Madrid
Hospital Universitario Fundación Jiménez Díaz Grupo quirónsalud

Expert Scientific and Strategy Advisory Board

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Director of Center and The Research Leona M. and Harry B.Helmsley IBD

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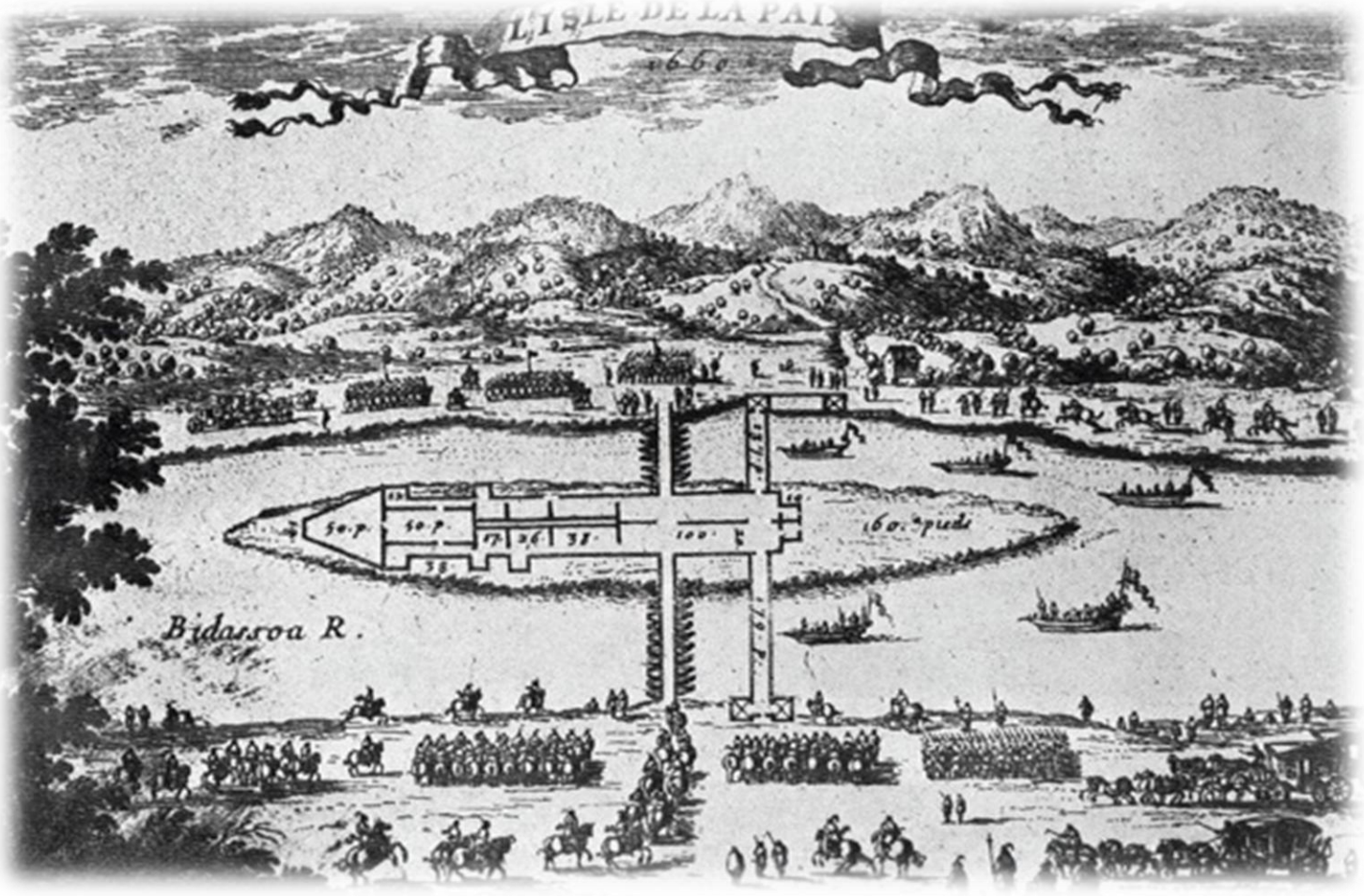
International Scientific/Clinical and Strategy Advisory Board with Opinion Leaders in Cell and Gene Therapy (C&GT), MSC product development, iPSC technology and clinical indications for GvHD, IBD and Psoriasis

Kiji to be the leading company in iPSC-MSC multi-gene engineered cell therapies



Cell and Gene Therapy Product

3

Allogeneic

Product
Optimized cell biology/source
iPSC/Professional biology
Priming; Gene engineering; ...


KIJI
Therapeutics
April 2026