Orchard therapeutics™

HSC Gene Therapy: Differentiated Profile and Potential Beyond Rare

September 14, 2021

We aspire to end the devastation caused by genetic and other severe diseases through the curative potential of HSC gene therapy

Forward-looking Statements

Certain information set forth in this presentation and in statements made orally during this presentation contains “forward-looking statements”. Except for statements of historical fact, information contained herein constitutes forward-looking statements and may include, but is not limited to, the Company’s expectations regarding: (I) the safety and efficacy of its product candidates; (II) the expected development of the Company’s business and product candidates; (III) the timing of regulatory submissions for approval of its product candidates; (IV) the timing of interactions with regulators and regulatory submissions related to ongoing and new clinical trials for its product candidates; (V) the timing of announcement of preclinical data for its product candidates and the likelihood that such data will be positive and support further development and regulatory approval of these product candidates; (VI) the timing and likelihood of approval of such product candidates by the applicable regulatory authorities; (VII) the adequacy of the Company’s manufacturing capacity and plans for future investment and commercialization; (VIII) execution of the Company’s vision and growth strategy, including with respect to global growth; (IX) the size and value of potential markets for the Company’s product candidates; and (X) projected financial performance and financial condition. The words “may,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts," “potential,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements are provided to allow investors the opportunity to understand management’s beliefs and opinions in respect of the future so that they may use such beliefs and opinions as one factor in evaluating an investment.

These statements are neither promises nor guarantees of future performance. Such forward-looking statements necessarily involve known and unknown risks and uncertainties, which include, without limitation, the severity of the impact of the COVID-19 pandemic on the Company’s business, including on preclinical and clinical development and commercial programs, which may cause actual performance and financial results in future periods to differ materially from any projections of future performance or results expressed or implied by such forward-looking statements. You are cautioned not to place undue reliance on forward-looking statements. These statements are subject to a variety of risks and uncertainties, many of which are beyond the Company’s control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. For additional disclosure regarding these and other risks faced by the Company, see the disclosure contained in the Company’s public filings with the U.S. Securities and Exchange Commission (the “SEC”), including in the Company’s quarterly report on Form 10-Q for the quarter ended June 30, 2021, as well as subsequent filings and reports filed with the SEC. These forward-looking statements speak only as of the date of this presentation. The Company undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

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HSC Gene Therapy: Differentiated Profile and Potential Beyond Rare

TIME	AGENDA TOPIC	SPEAKER
7:00 – 7:10am	Differentiated Profile of Orchard's HSC Gene Therapy	**Bobby Gaspar**
7:10 – 7:25am	Potential in Larger Indications: Update on OTL-104 Program for NOD2 Crohn's Disease	**Piv Sagoo**
7:25 – 7:40am	Future Applications: Antigen-specific Treg cells & Vectorized Antibodies	**Bobby Gaspar**
7:40 – 7:50am	Leveraging the HSC Platform through Business Development	**Bobby Gaspar**
7:50 – 8:00am	Q&A	

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Differentiated Profile of Orchard's HSC Gene Therapy

Bobby Gaspar, M.D., Ph.D.

Chief executive officer

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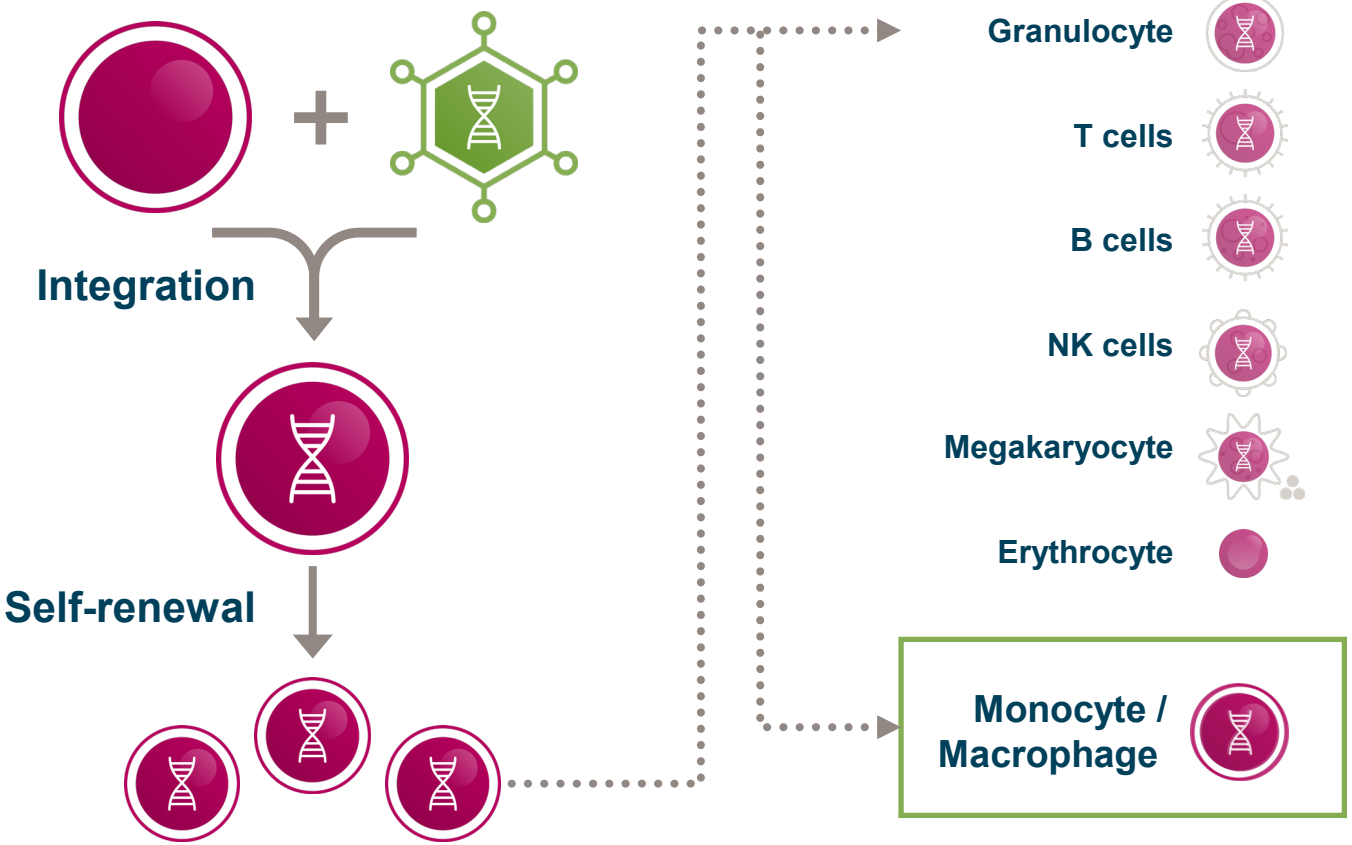
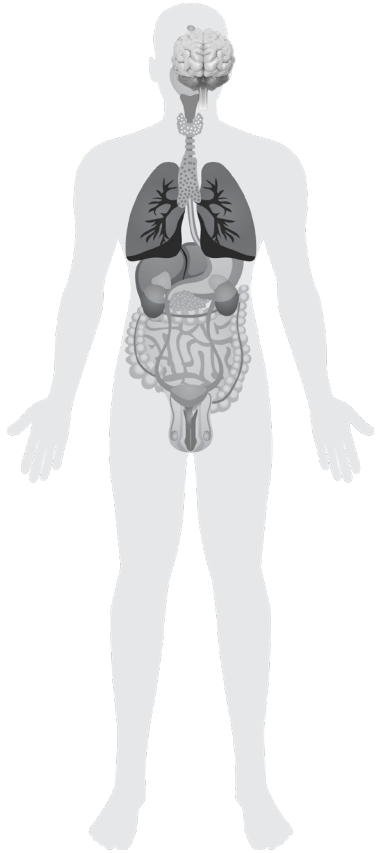
HSC Gene Therapy Offers a Highly Differentiated Approach





Brain

GI

High area of focus

Other Organ Systems

Areas for potential future expansion

Literature references: Alessia Capotondo, Rita Milazzo, Letterio Salvatore Politi, Angelo Quattrini, Alessio Palini, Tiziana Plati, Stefania Merella, Alessandro Nonis, Clelia di Serio, Eugenio Montini, Luigi Naldini, and Alessandra Biffi, PNAS September 11, 2012 109 (37) 15018-15023; https://doi.org/10.1073/pnas.1205858109; Tissue macrophages: heterogeneity and functions, Siamon Gordon and Annette Plüddemann, BMC Biology 2017 15:53, 29 June 2017

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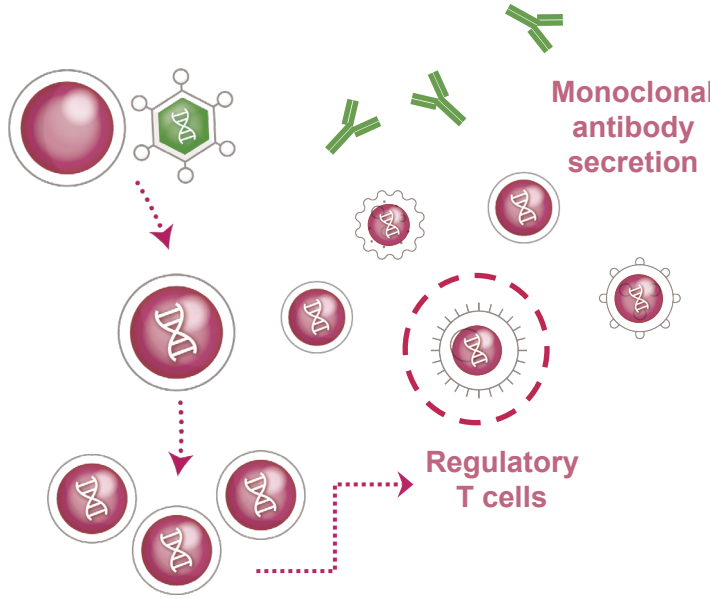
Orchard's HSC Gene Therapy Offers a Highly Differentiated, Validated Approach with Opportunities for Expansion

Validation in Rare Diseases	Larger Indications	Future Applications
libmeldy™ (atidarsagene autotemcel) *Approved for early-onset MLD in the EU (Dec 2020)* *Clinical POC in 5 additional indications*	**OTL-104** **NOD2-Crohn's** **OTL-204 / OTL-205** **FTD / ALS** **OTL-105** **HAE**	

HSC Gene Therapy Platform Approach

1H 2020 → **2H 2020** → **Future**

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Advantages of Orchard's HSC Gene Therapy Approach vs AAV Technologies

Challenge	AAV gene therapy	Orchard's HSC lentiviral gene therapy
Safety	Multiple safety concerns including liver failure, thrombotic microangiopathy and neurotoxicity, **particularly at high doses**	Orchard's programs have not, to date, seen similar immune reactions and systemic related to IMP infusion
Durability	Increasing evidence of **reduction in efficacy** in liver-directed AAV approaches over time	Long term durability following HSC transplant
Immunomodulation	Commonly use steroids +/- other agents, but **no consistent approach to management**	Use well-known conditioning regimens Reduced-toxicity agents in development
Applicability	Neutralizing antibodies to capsid means 30-80% of patients are unable to receive therapy	Autologous nature of HSC GT means no patients are automatically excluded from receiving treatment

Seen across AAV programs regardless of therapeutic target

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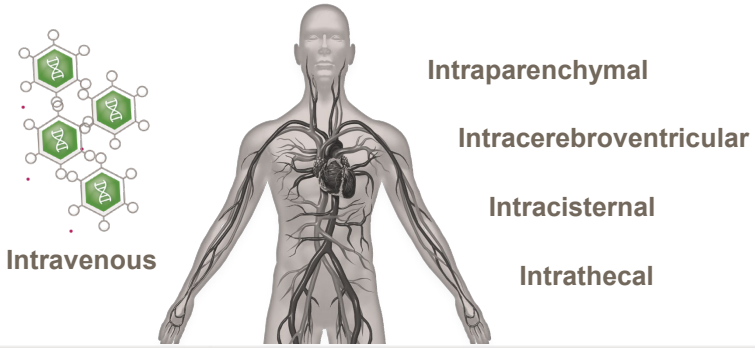
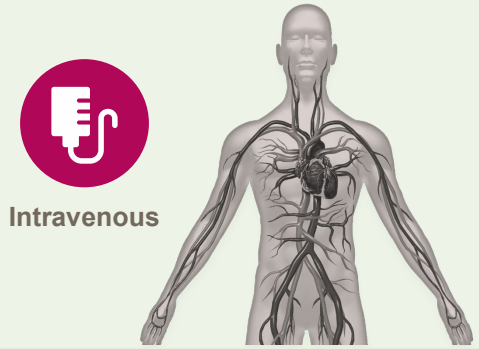
Safety Considerations for AAV Vectors vs HSC Gene Therapy

AAV gene therapy



What the body sees	Safety concerns for IV route
Foreign naked viral vector entering the blood stream or CNS	• Hepatotoxicity • Thrombotic microangiopathy • Dorsal root ganglion toxicity at high doses • Use of steroids and other immunosuppressive drugs
Immune Response	**Safety concerns for other routes**
IV triggers adaptive (anti-capsid antibodies and CTL) and innate immune responses	• MRI abnormalities • Dorsal root ganglion histopathology • Potentially mediated by vector or by damage from administration

Ex vivo HSC lentiviral gene therapy



What the body sees

Its own blood cells

Immune response

No immune response triggered against gene modified cells

Safety

Well controlled and studied conditioning protocol limits safety issues

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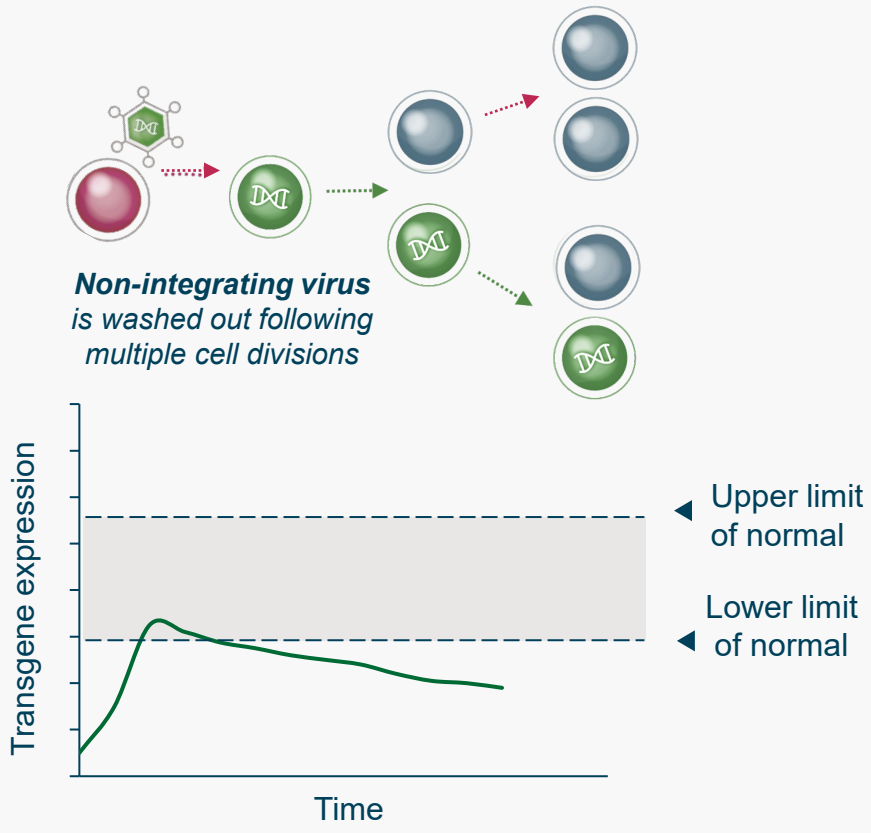
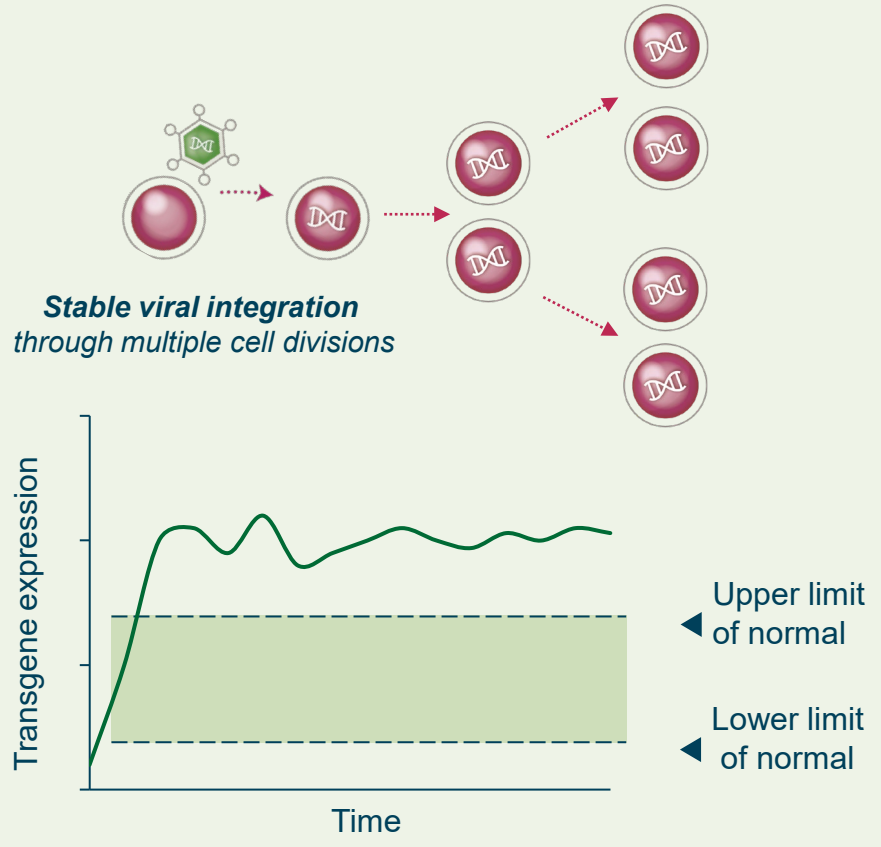
HSC Gene Therapy Offers Superior Durability Compared to Liver-directed AAV Approaches Which Show Tapering Transgene Expression

Illustrative waning of liver-directed AAV gene therapy



Illustrative lentiviral HSC gene therapy durability



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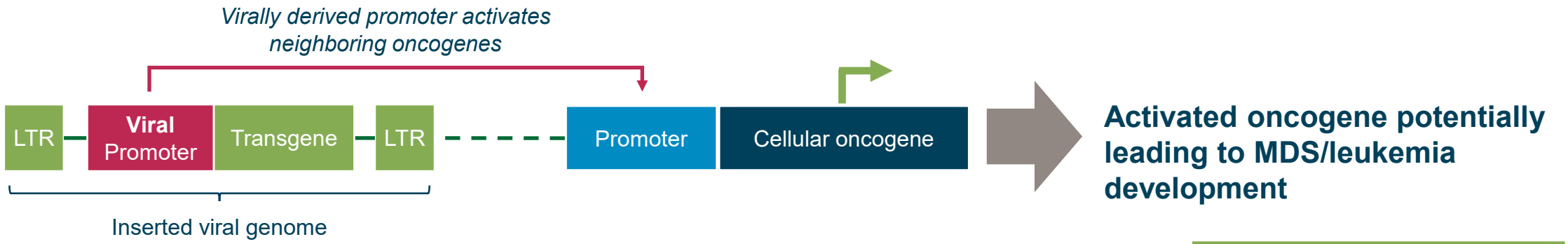
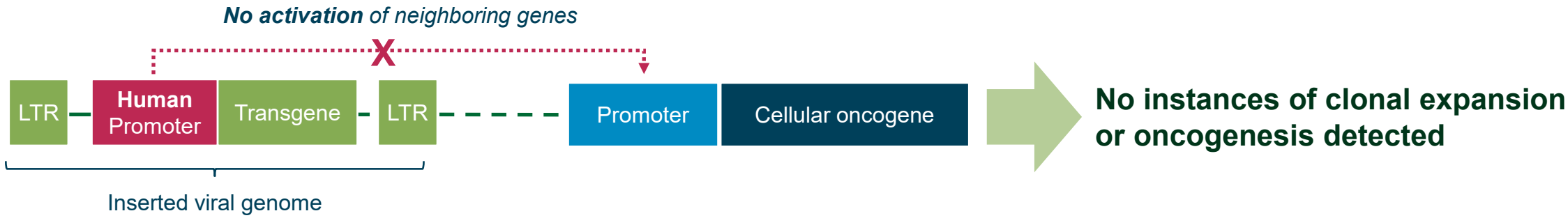
No Oncogenesis Has Been Observed in Orchard's Lentiviral HSC Gene Therapy Using Human Promoters

Viral-derived promoter with strong enhancer activity in the gene therapy vector:



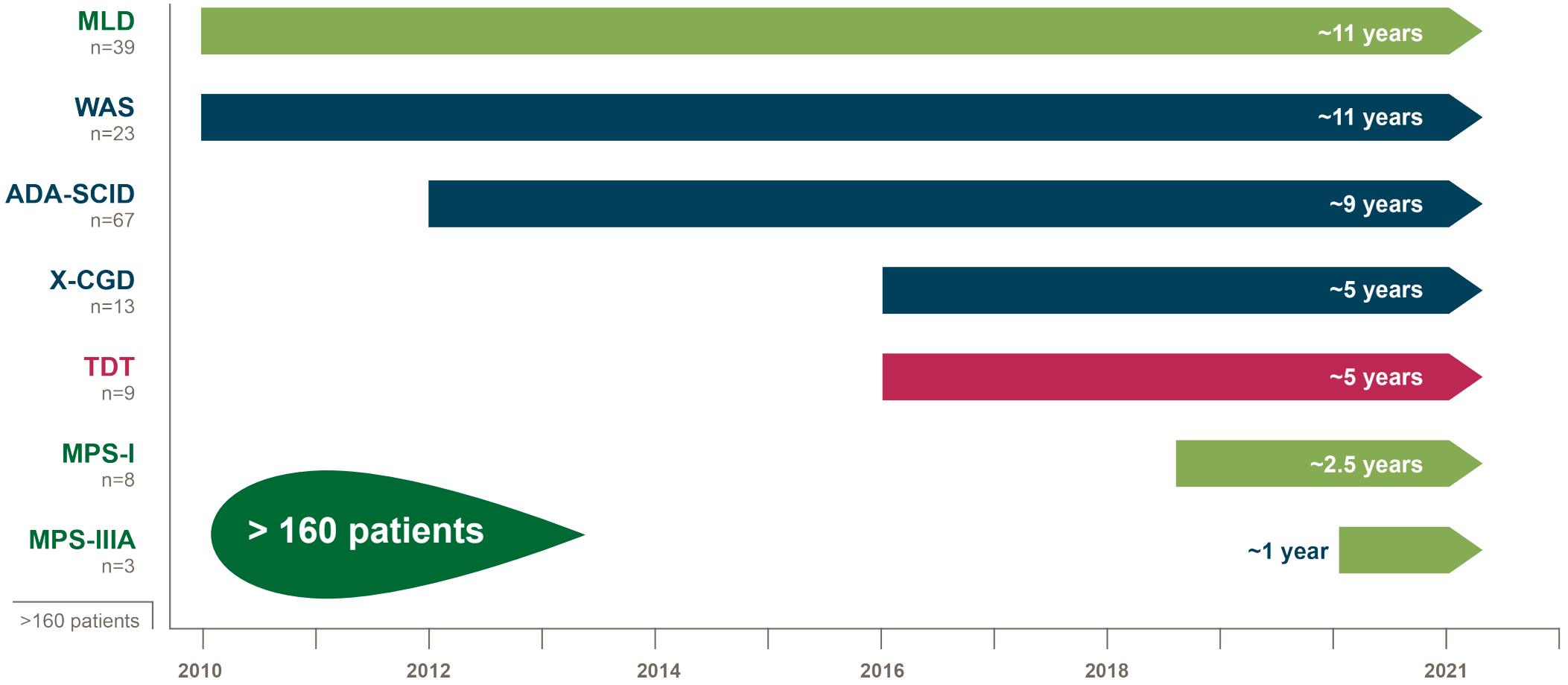
★ All Orchard's programs

Orchard's vectors use human promoters with limited enhancer activity:



Not all lentiviral vectors are the same

Durability of Response and Safety Demonstrated via Longest Patient Follow-up with Orchard's HSC Gene Therapy



- Patients treated in the development phase, including in clinical trials and under pre-approval access (defined as any form of pre-approval treatment outside of a company-sponsored clinical trial, including, but not limited to, compassionate use, early access, hospital exemption or special license).
- Data based on in-house data as of February 2021 and comprises all patients treated with CD34+ hematopoietic stem cells transduced *ex vivo* with vector of interest, inclusive of current and former programs.

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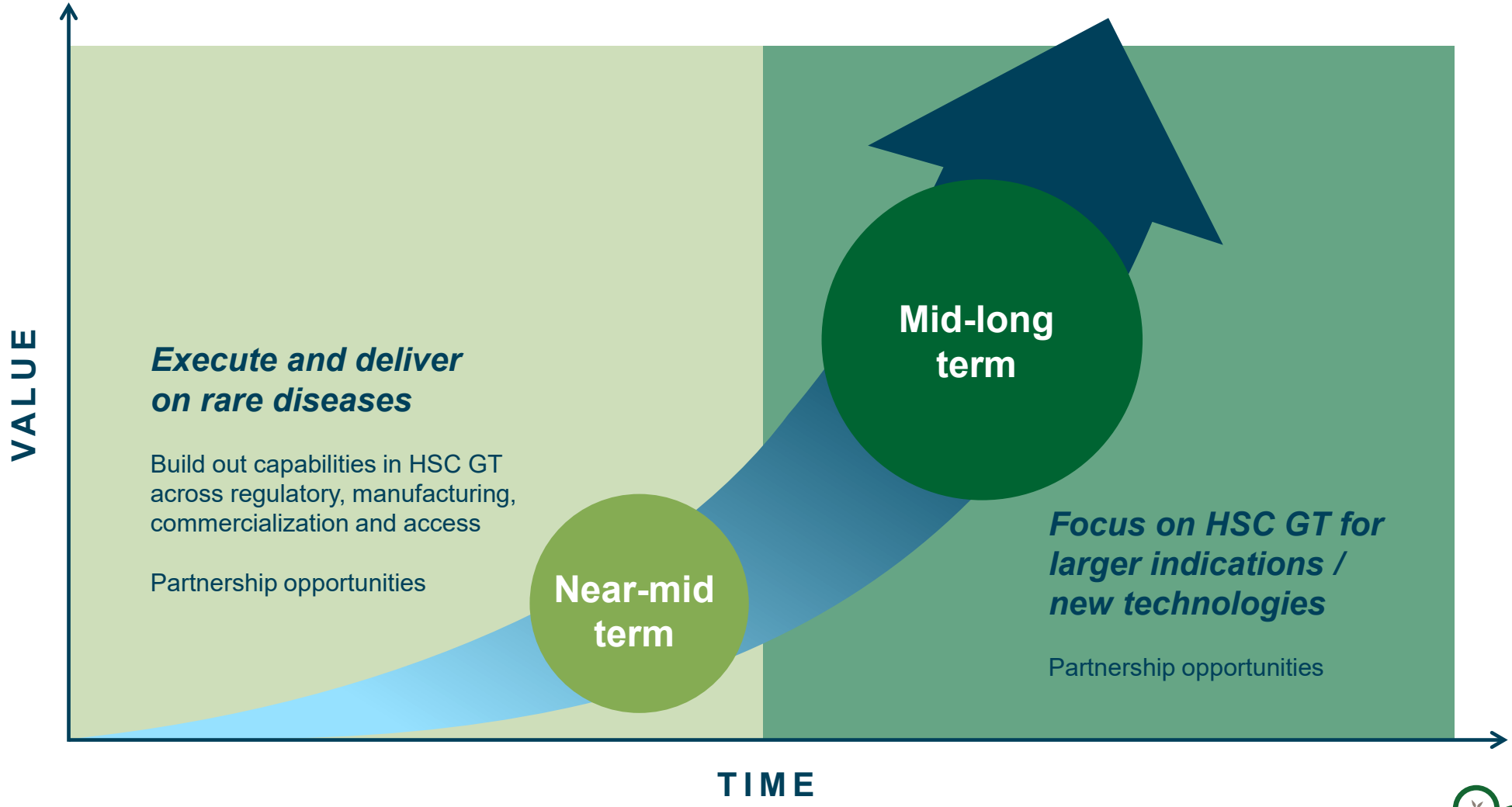
Accelerating Long-term Growth and Value Creation By Expanding into Larger Indications



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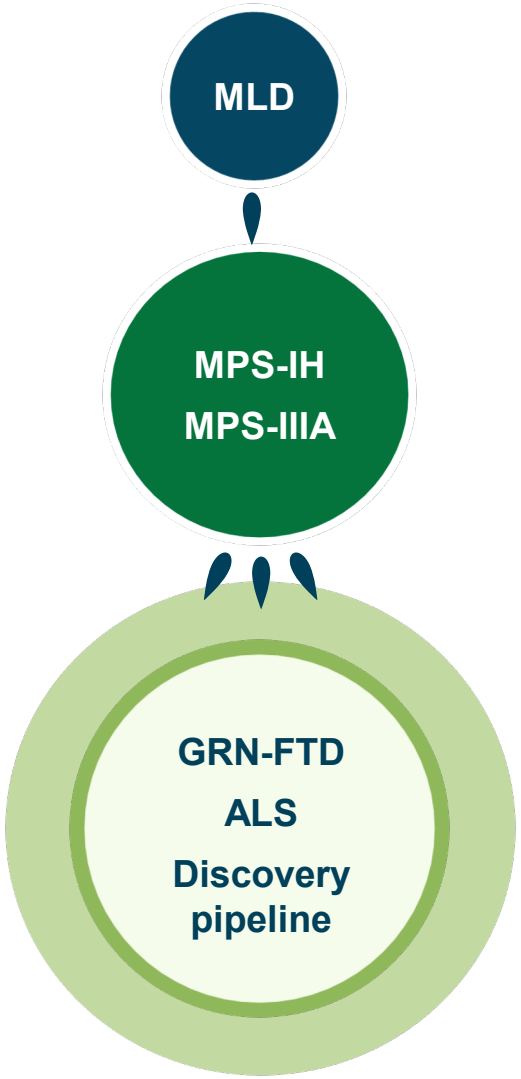
Upcoming Talks at ESGCT Covering Orchard's Neurometabolic Programs



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HSC Gene Therapy: Differentiators and Applications Beyond Rare

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Update on OTL-104 Program for NOD2 Crohn's Disease

Piv Sagoo, Ph.D.

Director, gene and cell therapy research

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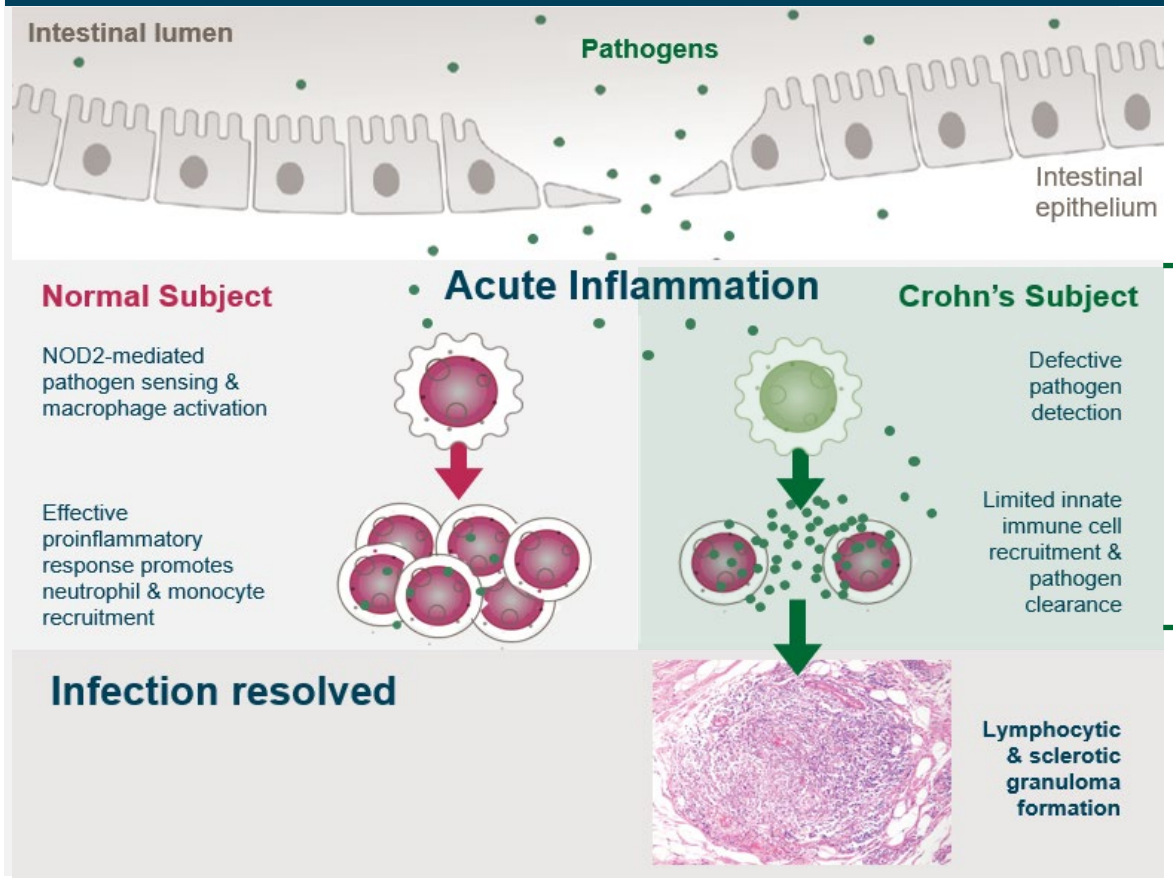
HSC Gene Therapy for NOD2 Crohn's Disease: OTL-104 Program Update

NOD2 Crohn's Disease is Driven by Impaired Intestinal Pathogen Sensing and Clearance



Emerging research supporting our HSC-gene therapy approach for NOD2 Crohn's Disease

- NOD2 defect strongly associated with clinical severity in Crohn's disease patients
  - Genes 2021: Gene polymorphisms of NOD2, IL23R, PTPN2 and ATG16L1 in patients with Crohn's disease: on the way to personalized medicine?; Hoffmann et al.
- Clear link between dysfunctional immune cells & defective NOD2 gene expression in Crohn's Disease
  - Nature 2021: A molecular connection hints at how a genetic risk factor drives Crohn's disease; Plevy S.
- Unmet clinical need
  - Gastroenterology 2021: Predicting outcomes in pediatric Crohn's disease for management optimization: Ricciuto et al.

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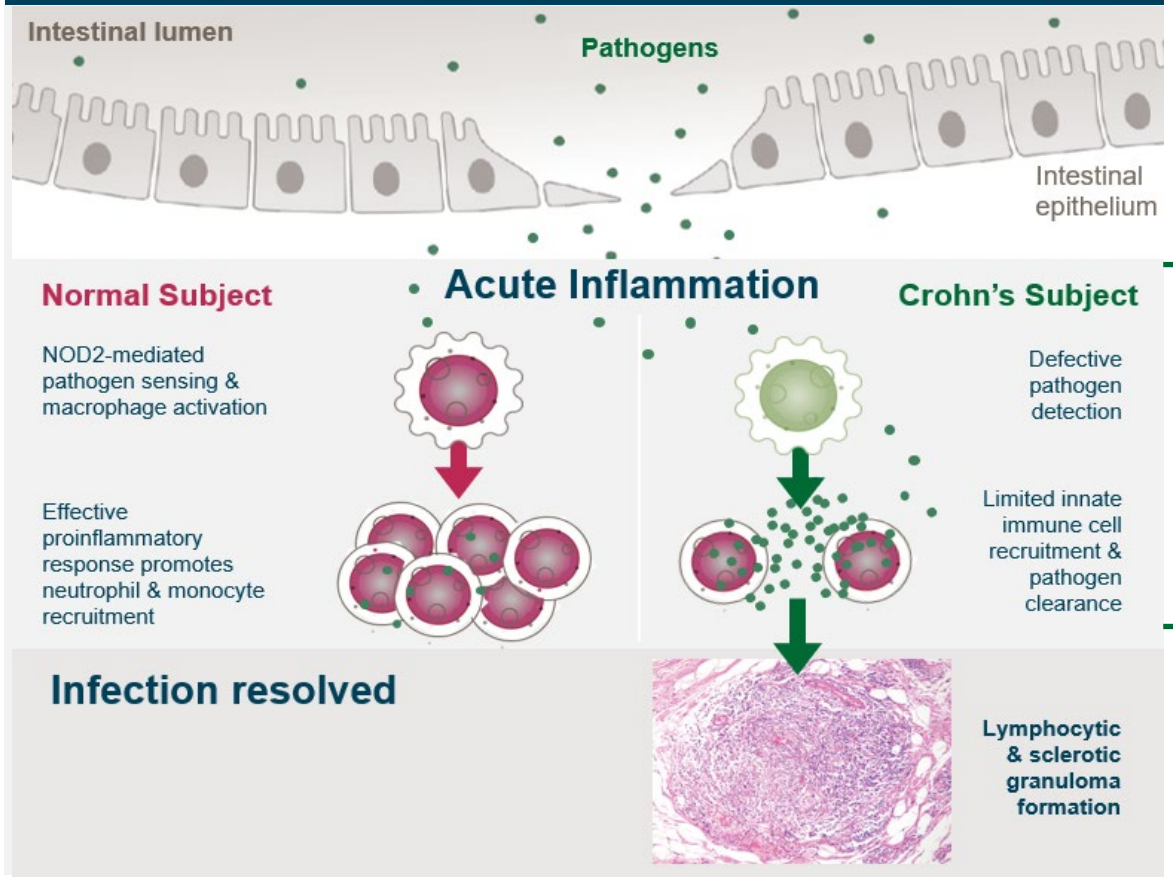
HSC Gene Therapy for NOD2 Crohn's Disease: OTL-104 Program Update

NOD2 Crohn's Disease is Driven by Impaired Intestinal Pathogen Sensing and Clearance



Early Discovery > Preclinical Stage Program

Objective 1	✓	Validate NOD2 as a target to correct abnormal innate immune cell functions
Objective 2	✓	Development of gene modification approach & therapeutic candidates
Objective 3	✓	Safety profiling of NOD2-LV gene modification of human CD34+ stem cells
Objective 4	In progress	NOD2 HSC GT can reconstitute key cellular functional compartments in the gastrointestinal tract
Objective 5	In progress	Proof of Concept in an experimental colitis disease model demonstrating *in vivo* efficacy

Program development plan leading to IND-enabling toxicology/ biodistribution studies

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NOD2-LV Transduction of KO Human CD34+ Stem Cells Restores NOD2 Dependent Monocyte Immune Responses

Objectives
1-2

Experimental Schema

Mobilised blood CD34+ stem cells

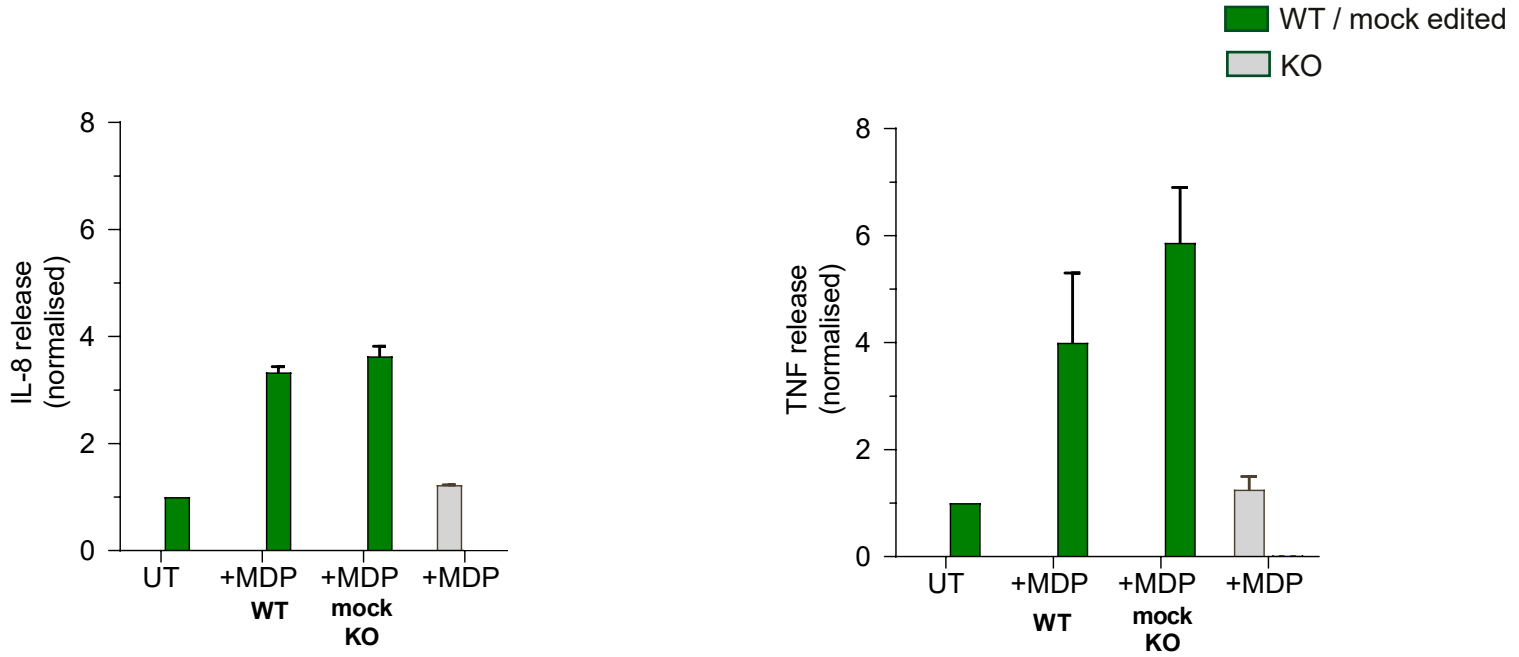
CRISPR-Cas9 targeted NOD2 disruption

KO

Myeloid differentiation

MDP response profiling

Highly Efficient LV Transduction of CD34+ Stem Cells, Restores Normal MDP sensing by Human Macrophages



- Gene editing to disrupt (KO) human NOD2 gene in healthy donor CD34+ cells mimics NOD2-Crohn's Disease donor monocyte function (~90% KO efficiency using CRISPR)
- LV delivery of functional NOD2 (codon optimised) to human CD34+ HSC restores MDP (muramyl dipeptide) sensing in differentiated macrophage cultures (90% TE; VCN~3)
- Highly efficient LV transduction of CD34+ cells, generate robust restoration of normal MDP sensing by human macrophages

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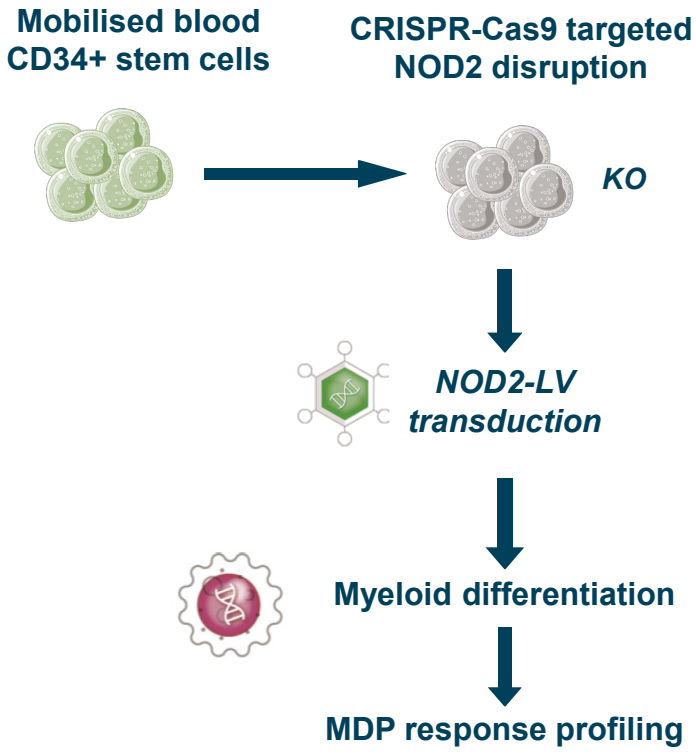
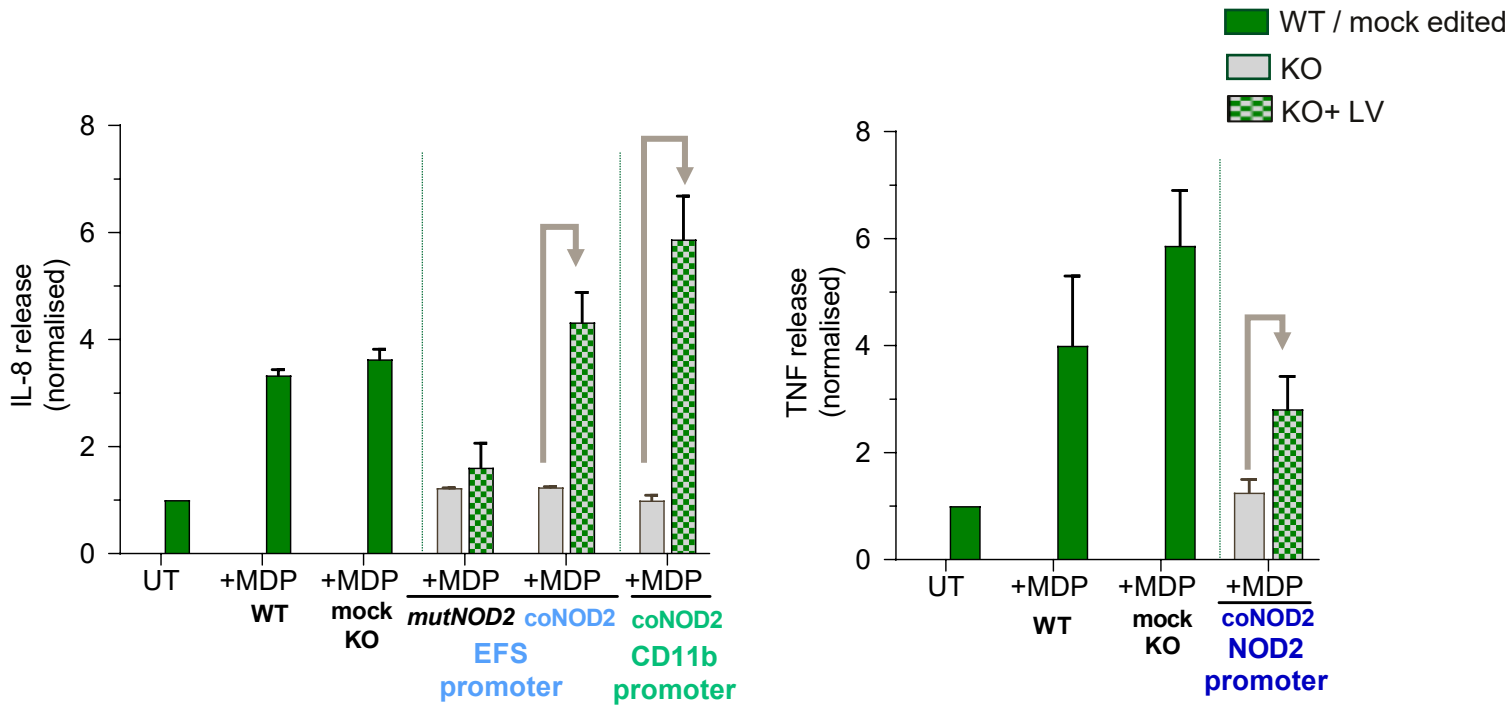
NOD2-LV Transduction of KO Human CD34+ Stem Cells Restores NOD2 Dependent Monocyte Immune Responses

Objectives
1-2

Experimental Schema



Highly Efficient LV Transduction of CD34+ Stem Cells, Restores Normal MDP Sensing by Human Macrophages



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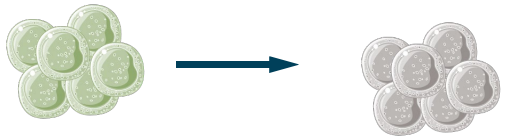
Gene Editing (HDR) Insertion of Functional NOD2 Restores NOD2 Dependent Monocyte Immune Responses in KO CD34+ Stem Cells

Objective 2

Experimental Schema

Mobilised blood CD34+ stem cells

CRISPR-Cas9 targeted NOD2 disruption (KO)

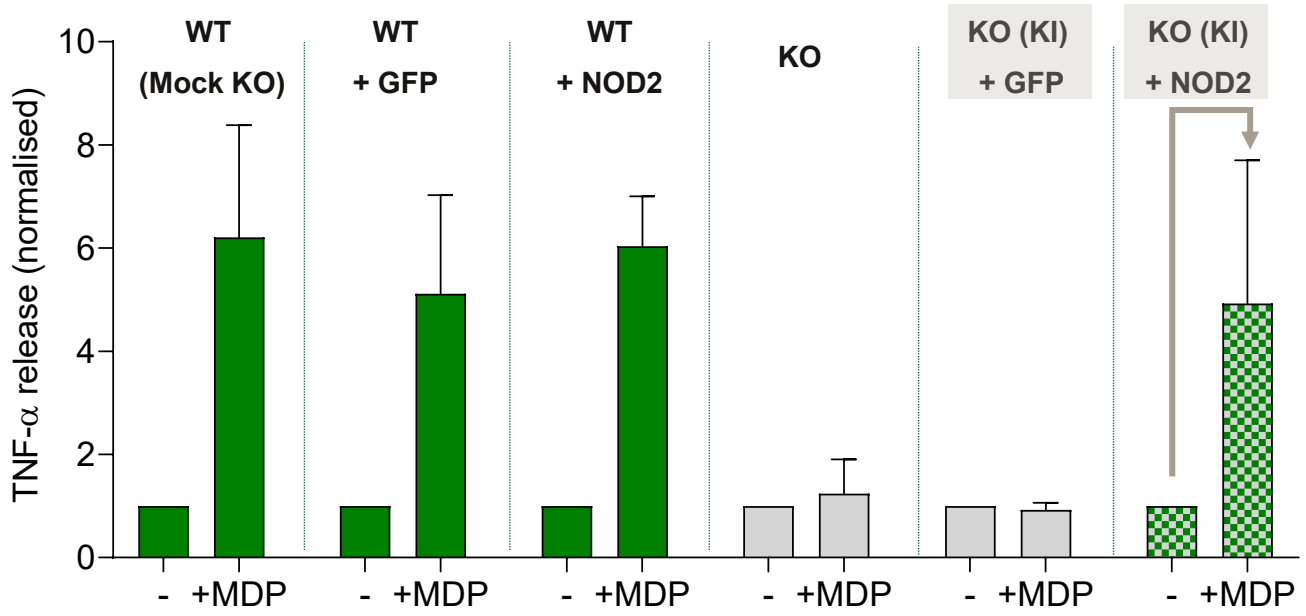


Homology Directed Repair to deliver functional NOD2 (KI)

Myeloid differentiation

MDP response profiling

Stem Cell Gene Editing of NOD2 Restores Equivalent Monocyte MDP Sensing Function as LV Gene Modification



- Gene editing strategy developed to insert functional NOD2 into exon 2 of the endogenous NOD2 locus (~60% HDR efficiency)
- KO (CRISPR) and subsequent Knock-In (KI) of NOD2 in CD34+ stem cells restores NOD2 activity
- Equivalent to results achieved using LV approach show both methods for gene modification are suitable for use

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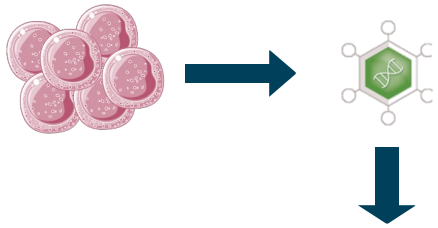
NOD2-LV Gene Modification Does Not Affect Human CD34+ Stem Cell Phenotype or Function

Objective 3

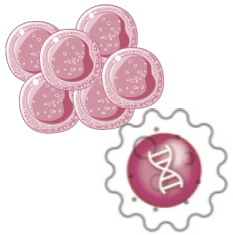
Experimental Schema

Mobilised blood CD34+ HSC

NOD2-LV or GFP-LV transduction



In vitro **hematopoietic characterization**



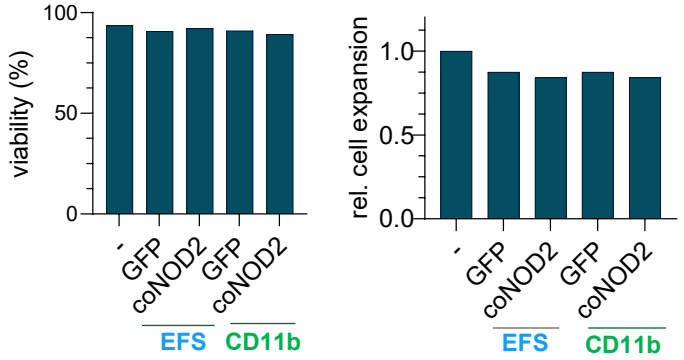
- CFU potential
- Myeloid differentiation
- macrophage polarization

In vitro Safety Profiling - NOD2-LV Gene Modification of CD34+ Stem Cells

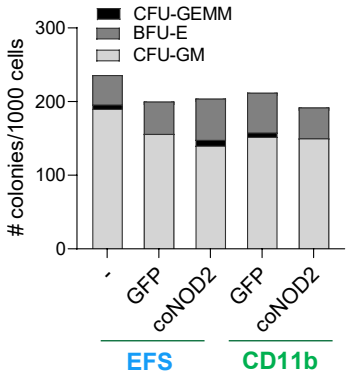
Gene modified CD34+ stem cells

Promoter	Transgene	VCN	% TE
EFS	GFP	1.8	67%
	coNOD2	3.4	68%
CD11b	GFP	5.3	83%
	coNOD2	2.2	65%

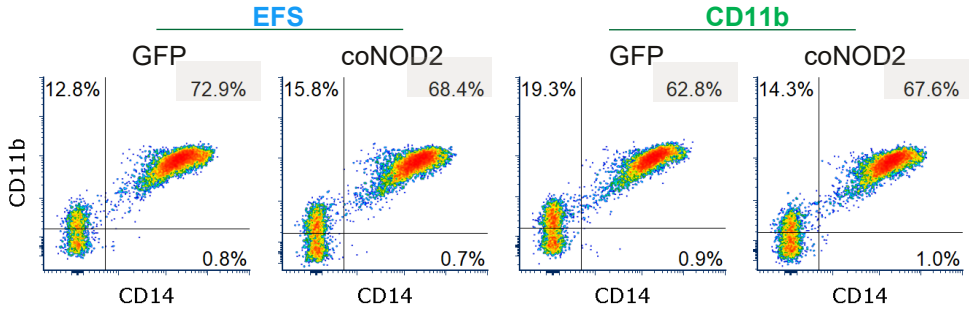
HSC viability & expansion



CFU enumeration



In vitro **differentiation into monocyte, macrophage subsets**



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NOD2-LV Gene Modification Does Not Affect CD34+ Stem Cell Differentiation *in vivo*

Objective **3**

Experimental Schema

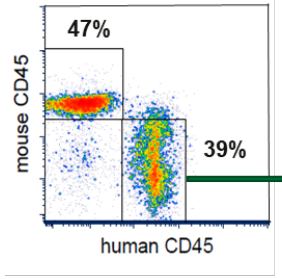
Mobilised blood CD34+ HSC

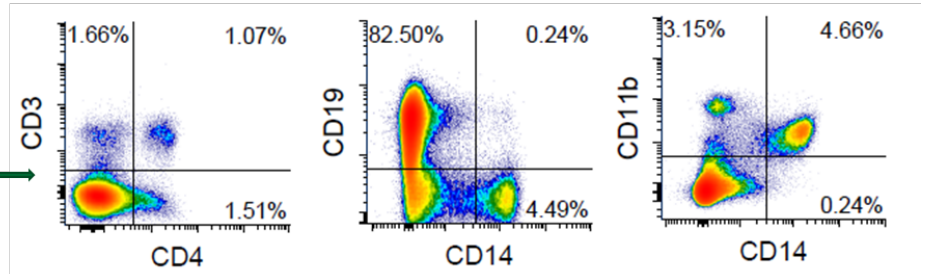
NOD2-LV or GFP-LV transduction

***in vivo* hematopoietic characterization**

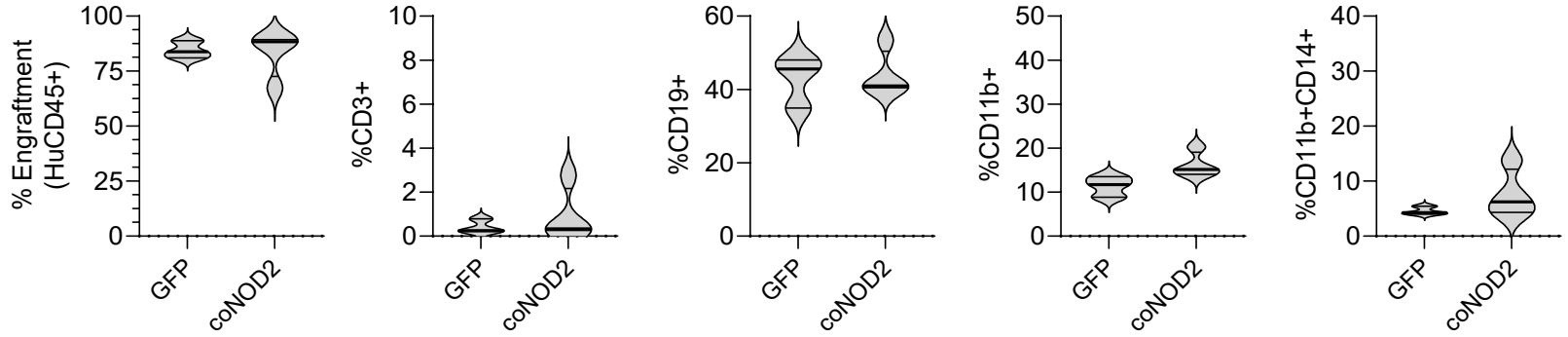
In vivo Safety Profiling – Analysis of NOD2-LV CD34+ Stem Cell Progeny

NSG-SGM3 engraftment study for broad haematopoietic stem cell differentiation





NOD2 transgene expression (CD11b promoter) has no significant impact on hematopoiesis in SGM3 mice



- Transduction of CD34+ stem cells for *in vivo* engraftment analysis confirms NOD2 overexpression does not impact myeloid & lymphoid development & differentiation in vivo
- NSG-SGM3 mouse (Jax) allow superior engraftment of diverse hematopoietic lineages

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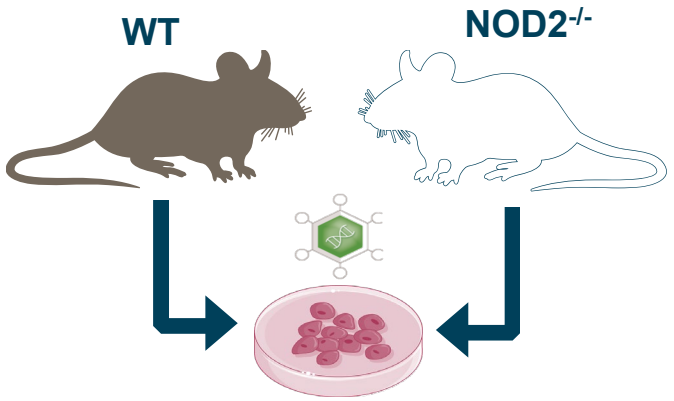
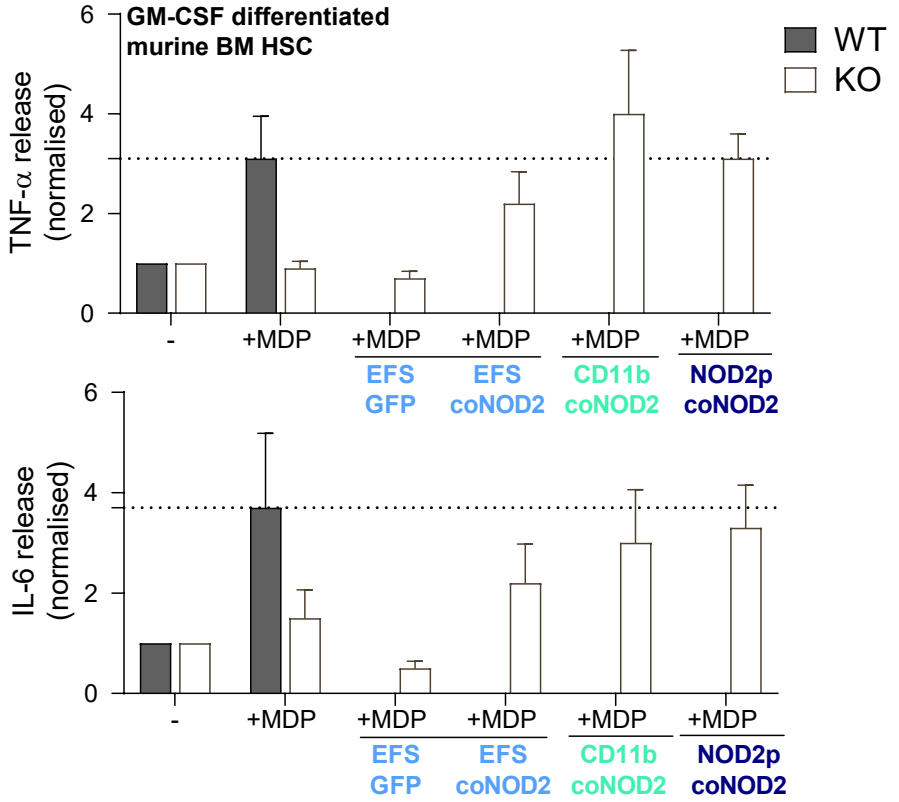
NOD2-LV Transduction of Murine HSC Can Restore NOD2 Dependent Immune Responses

Objective 4

Experimental Schema



LV Delivery of Functional NOD2 to Murine HSC Restores MDP Sensing in NOD2$^{-/-}$ BM-derived Macrophages & Monocytes



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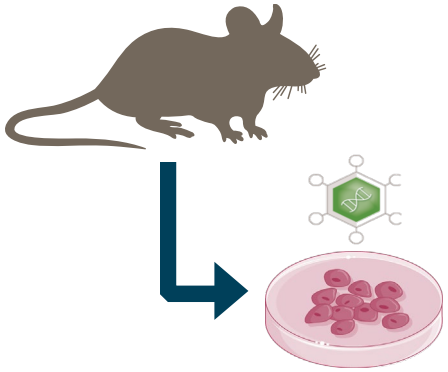
NOD2-LV HSC Gene Therapy Reconstitutes the Gut with a High Frequency of Gene Modified Hematopoietic Derived Cells

Objective 4

Experimental Schema

WT male / CD45.2



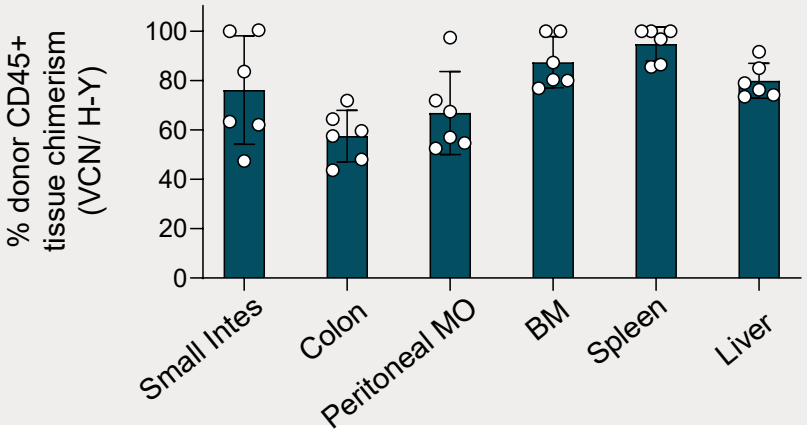
NOD2-LV, GFP-LV or untransduced lin⁻ BM HSC

Gene modified BM-Tx

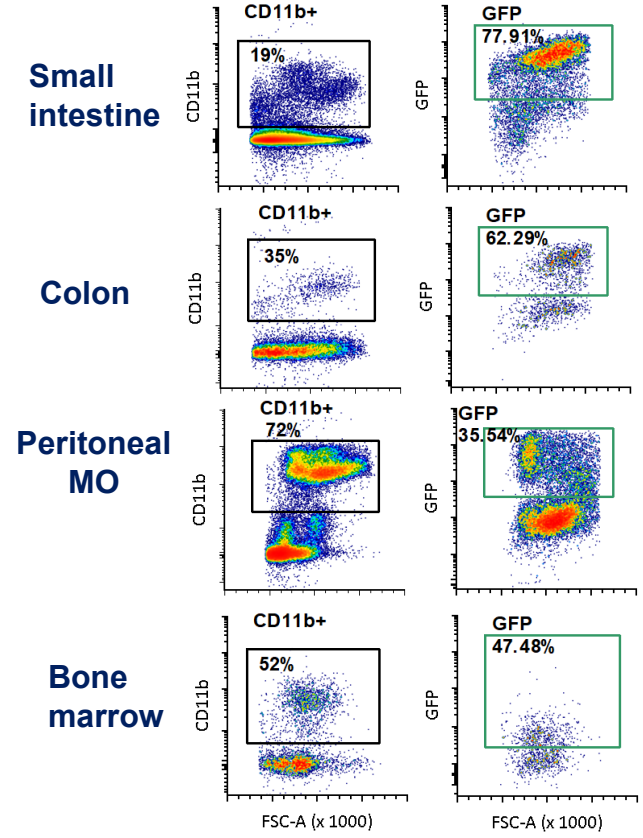
WT female / CD45.1

Intestinal Niche Environments are Efficiently Repopulated by Hematopoietic Derived Myeloid Progeny

- Transplant of lin- BM cells reconstitutes multiple tissue myeloid cell compartments



- Transplant of GFP-LV BM lin- cells (CD11b promoter) results in highly enriched myeloid derived gene modified compartments in gut



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Experimental Colitis Disease Model Development for PoC Studies Underway

Objective 5

Experimental Schema

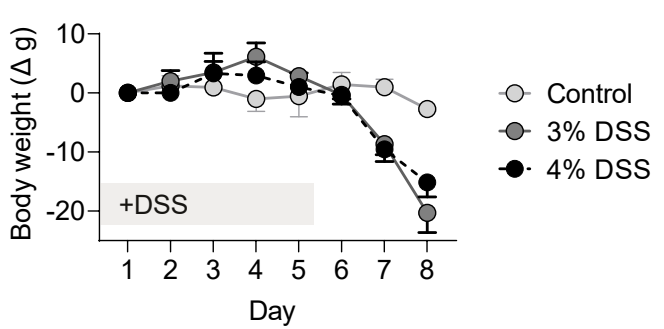
NOD2-LV Gene Modified HSC transplant

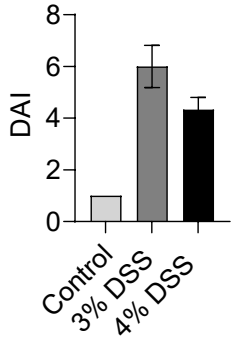
Chronic & Acute Colitis induction

Disease endpoints
Pathology score
Disease activity Index

Confirmation of Colitis Associated Intestinal Pathology in Acute Disease Induction Model

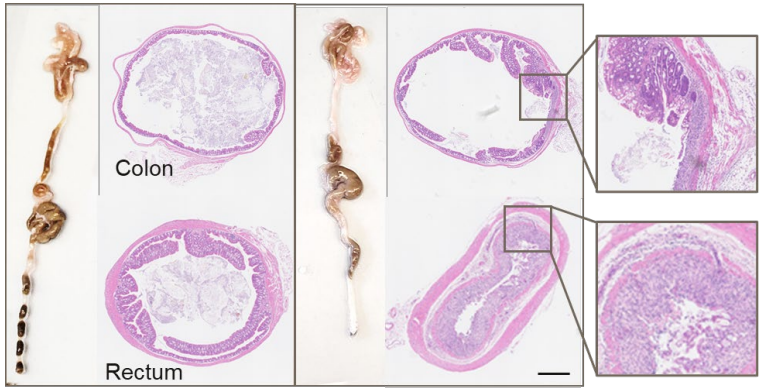
Dextran sulfate sodium (DSS)-induced colitis in WT mice





- Hemoccult positive
- Stool consistency
- Weight loss
- Behaviour

Control **4% DSS**



- Colon length shortening
- Epithelial mucosal degeneration, loss of crypts
- Inflammatory mononuclear cell infiltrate in mucosa, submucosa & muscle
- Submucosal oedema

- Model development & optimisation underway for selection of best translational model of Crohn's Disease IBD pathology

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Key Takeaways from OTL-104 for NOD2 Crohn's Disease Update

- Restoration of NOD2 protein expression in murine and human stem cells can rescue a defective myeloid immune response to microbial peptides
- Correction of NOD2 defective inflammatory functions is achievable by both NOD2-LV & NOD2-GE approaches
- NOD2-LV gene modification of CD34+ stem cells does not affect HSC engraftment or immune subset development and differentiation
- HSC transplantation can efficiently reconstitute intestinal tissue resident myeloid compartments
- Development of an experimental colitis model for PoC studies is in progress

Program development plan leading to IND-enabling toxicology / biodistribution studies

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HSC Gene Therapy: Differentiators and Applications Beyond Rare

TIME	AGENDA TOPIC	SPEAKER
7:00 – 7:10am	Differentiated Profile of Orchard's HSC Gene Therapy	**Bobby Gaspar**
7:10 – 7:25am	Potential in Larger Indications: Update on OTL-104 Program for NOD2 Crohn's Disease	**Piv Sagoo**
7:25 – 7:40am	Future Applications: Antigen-specific Treg cells & Vectorized Antibodies	**Bobby Gaspar**
7:40 – 7:50am	Leveraging the HSC Platform through Business Development	**Bobby Gaspar**
7:50 – 8:00am	Q&A	

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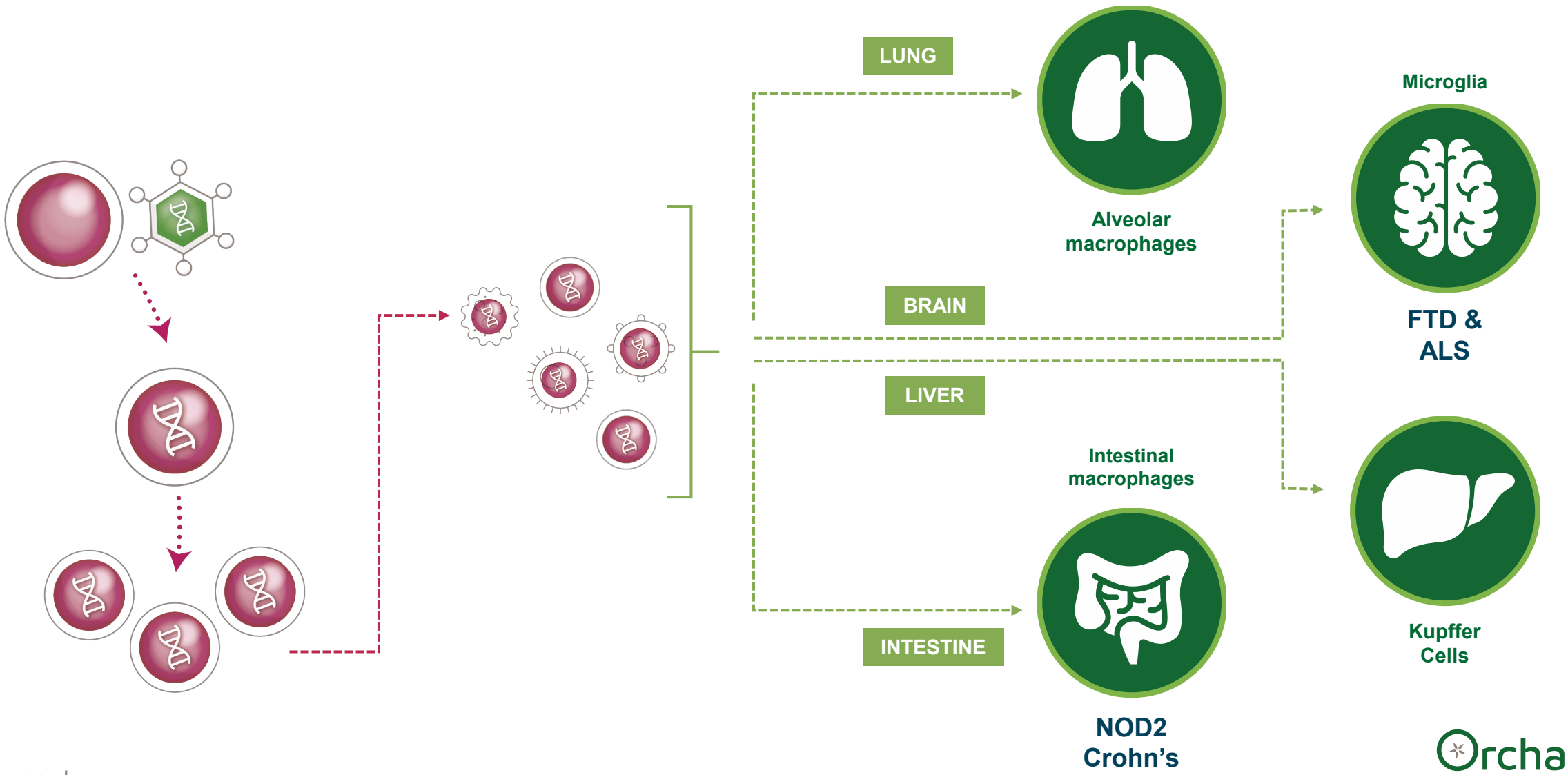
Future Applications for HSC Gene Therapy: Antigen-specific Treg Cells and Vectorized Antibodies

Bobby Gaspar, M.D., Ph.D.

Chief executive officer

Orchard therapeutics

The Power of HSC Gene Therapy: *Migration*



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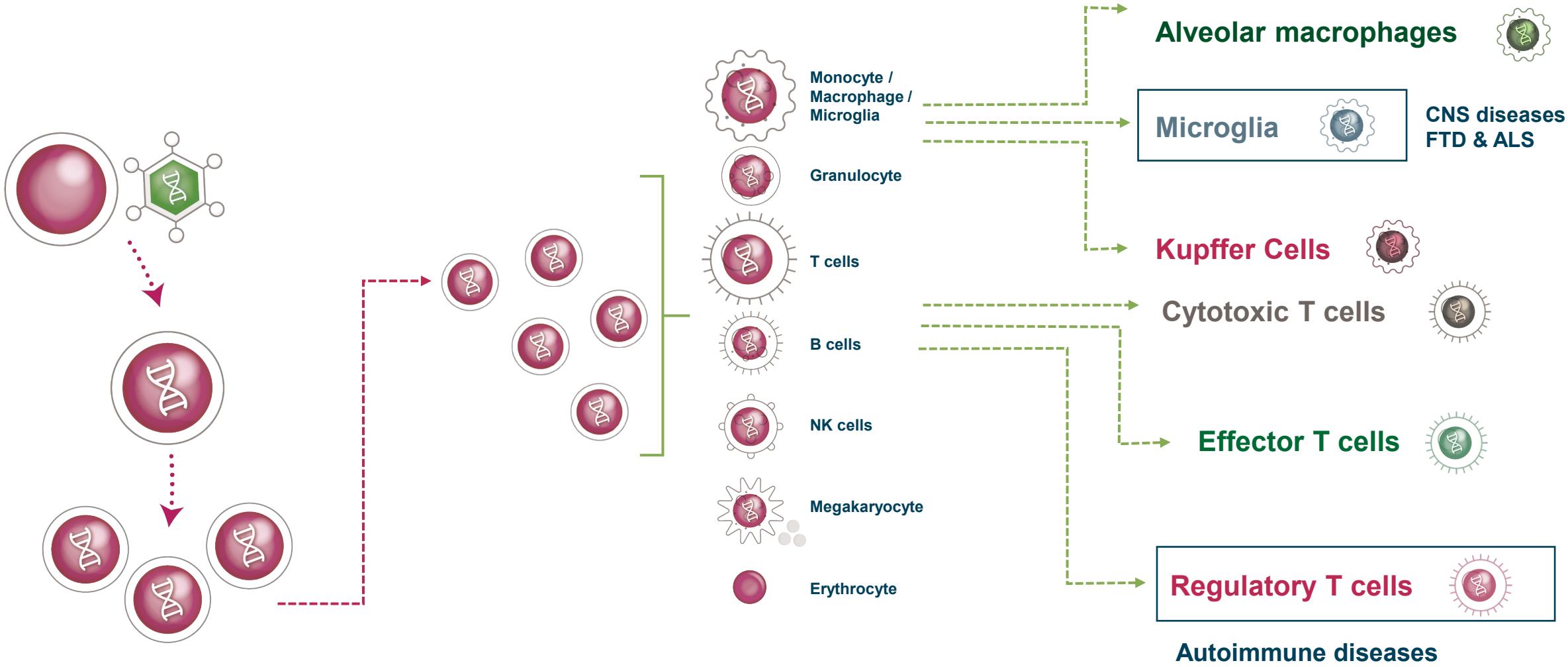
The Power of HSC Gene Therapy: *Differentiation*



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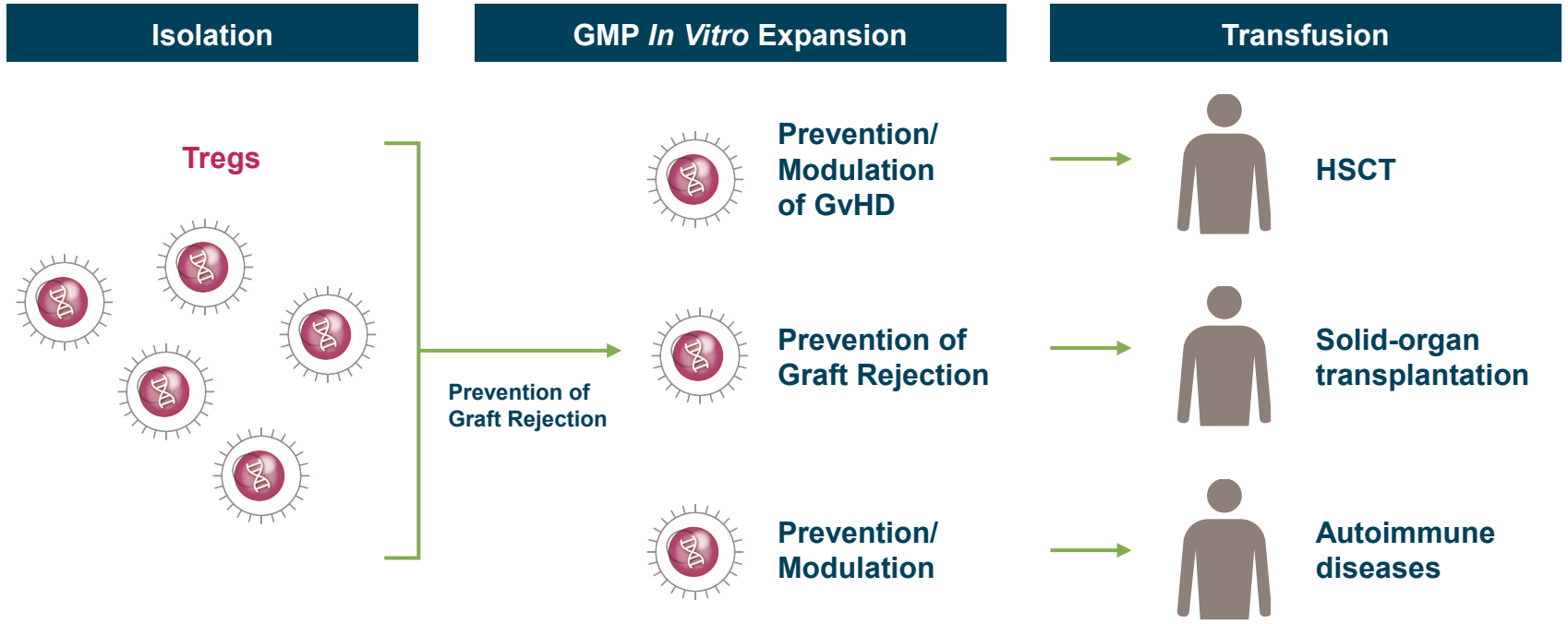
Tregs Are a Specialized Subset of Lymphocytes that Can Suppress Inflammation and Be Harnessed as a Cell Therapy Similar to CAR-Ts

- 1-2% of circulating lymphocytes
- Specialized suppressor cells
- Treg cells dominantly suppress immune activation
- Treg cells actively prevent inflammatory and autoimmune disease
- Autologous polyclonal Treg cell therapy in humans is safe but not effective
- **Can be targeted to specific auto-antigens to specifically suppress autoimmunity through CAR or TCR vector constructs**



Adapted from Sci Transl Med. 2015 Sept 9:7(304)

Orchard therapeutics

Orchard's Treg Approach Offers Potential Advantages Over Adoptive CAR-Treg Cell Therapy and Current Standard of Care

Current Standard of Care

Limitations of current therapies for autoimmune diseases

- Uncontrolled disease progression
- Life-long immunosuppression and associated severe infections
- Continuous chronic therapy adherence

Adoptive Treg Cell Therapy Approaches

Limitations of adoptive Treg cell therapy approaches

- Unclear durability and long-term efficacy
- Treg cells known to convert to effector phenotype under specific inflammatory conditions
- Unclear path to consistent GMP clinical scale manufacturing
- Unclear dose or dosing regimen due to limited lifespan of Treg cells

Companies exploring CAR-Tregs

- abata THERAPEUTICS — **$95M raise** MS cell therapy
- SONOMA BIOTHERAPEUTICS — **$265M raise** Arthritis, Diabetes cell therapy
- gentibio — **$157M raise** Type 1 Diabetes
- Sangamo THERAPEUTICS
- QuellTX

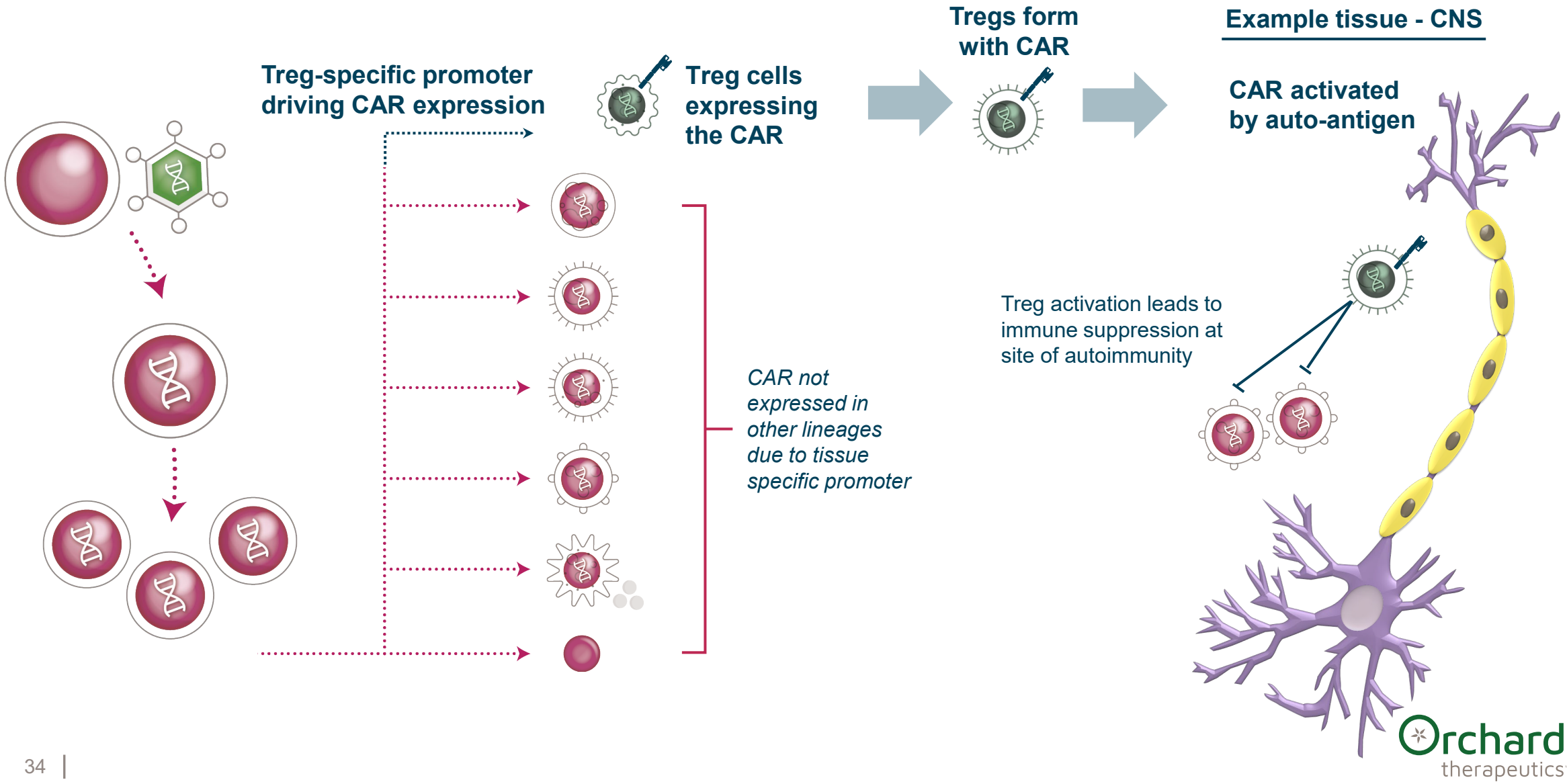
Orchard's HSC GT Approach

Orchard's HSC GT Approach

- Potential for long-term durability based on clinical evidence in genetic diseases
- Treg promotor designed to ensure CAR expression restricted to Treg cells
- Manufacturing protocols and GMP scale established
- Dosing regimen established for genetic diseases informs Orchard's HSC → Treg approach

Orchard therapeutics

Combining the Proven Durability of HSC Gene Therapy with the Specific Suppressive Potential of Tregs



Utilization of Tissue Specific Transcriptional Regulation, Antigen-specific Targeting Technology in the HSC Gene Therapy Platform

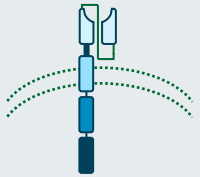
Existing HSC gene therapy platform

- Well validated platform
- Known manufacturing, dose and safety profile

Treg-specific transcriptional element

- Allows for activation of the antigen-specific cassette only in Treg cells

Antigen specific cassettes



- CARs, TCRs, BCRs
- Activate Treg suppression at site of autoimmunity

HSC CAR-Treg technology

- Transplant of transduced HSCs with an antigen specific cassette driven by Treg specific regulatory elements
- Generates a durable population of antigen-specific Treg cells
- Antigen specificity targeted to drivers of auto-immunity; Treg cells suppress autoimmune pathology
- Orchard has a proprietary position around this concept

Orchard therapeutics

Orchard Proprietary Position Covering the Concept and Specifics of the HSC-antigen Specific Treg Therapy

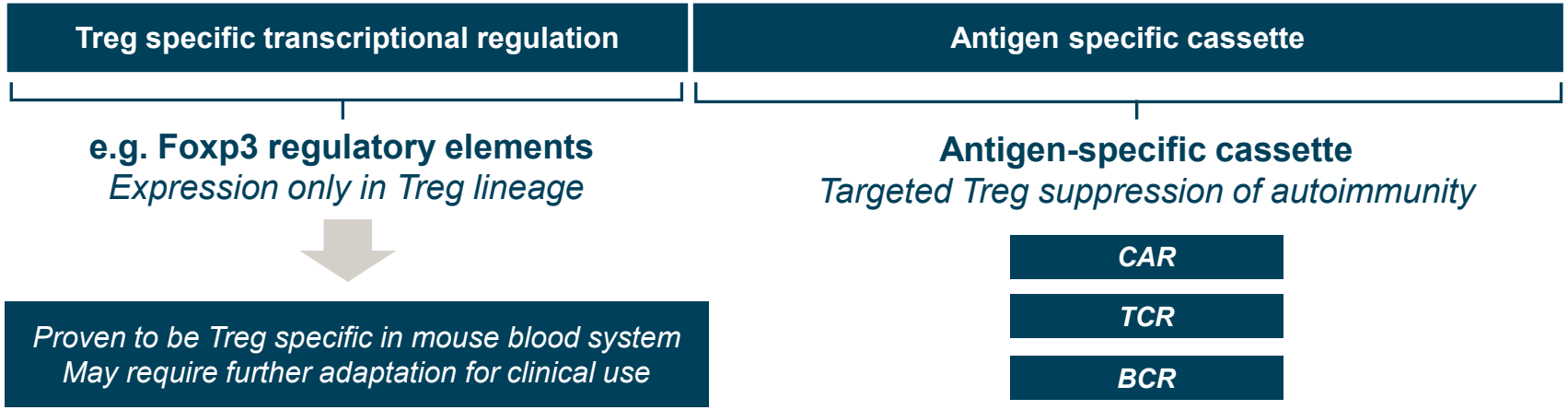
Therapeutic concept:

Methods for treating autoimmune diseases by way of regulatory T cells derived from genetically modified pluripotent hematopoietic cells

Disease applications:
Multiple auto-immune diseases

Broad classes and specific indications of autoimmune diseases, inflammatory disorders and related diseases are claimed within the patent

Vector design:
Treg-specific expression of an antigen-specific cassette



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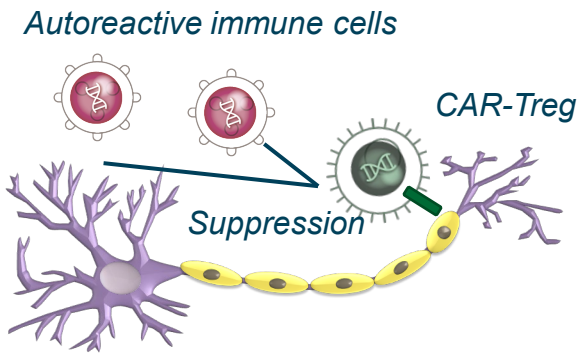
Potential Applications of the HSC CAR-Treg Technology

Multiple sclerosis

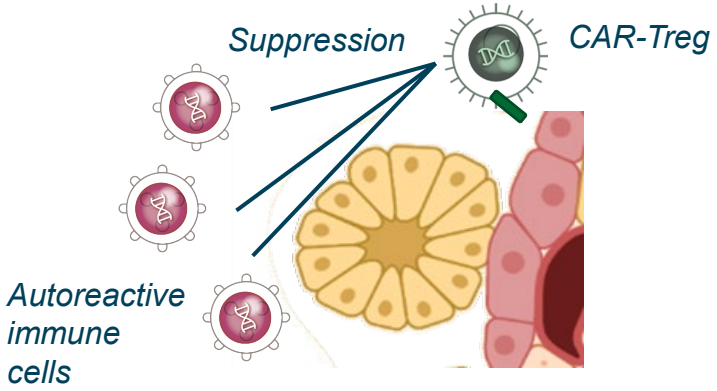
- **Identified antigen:** MOG and MBP
- **Use of HSC transplant:** Yes, 100s per year with limited efficacy
- **Unmet medical need:** High, particularly in progressive disease



Myelin sheath

Type 1 Diabetes

- **Identified antigen:** GAD65, chromogranin A, others
- **Use of HSC transplant:** Yes, but limited efficacy
- **Unmet medical need:** Many patients not reaching HbA1c goals



Pancreatic beta-cells

Rheumatoid arthritis

- **Identified antigen:** Limited, ova is one example
- **Use of HSC transplant:** Yes, but limited efficacy
- **Unmet medical need:** Significant proportion of patients non controlled on existing therapies

Synovial tissue

 Image credit: Biorender

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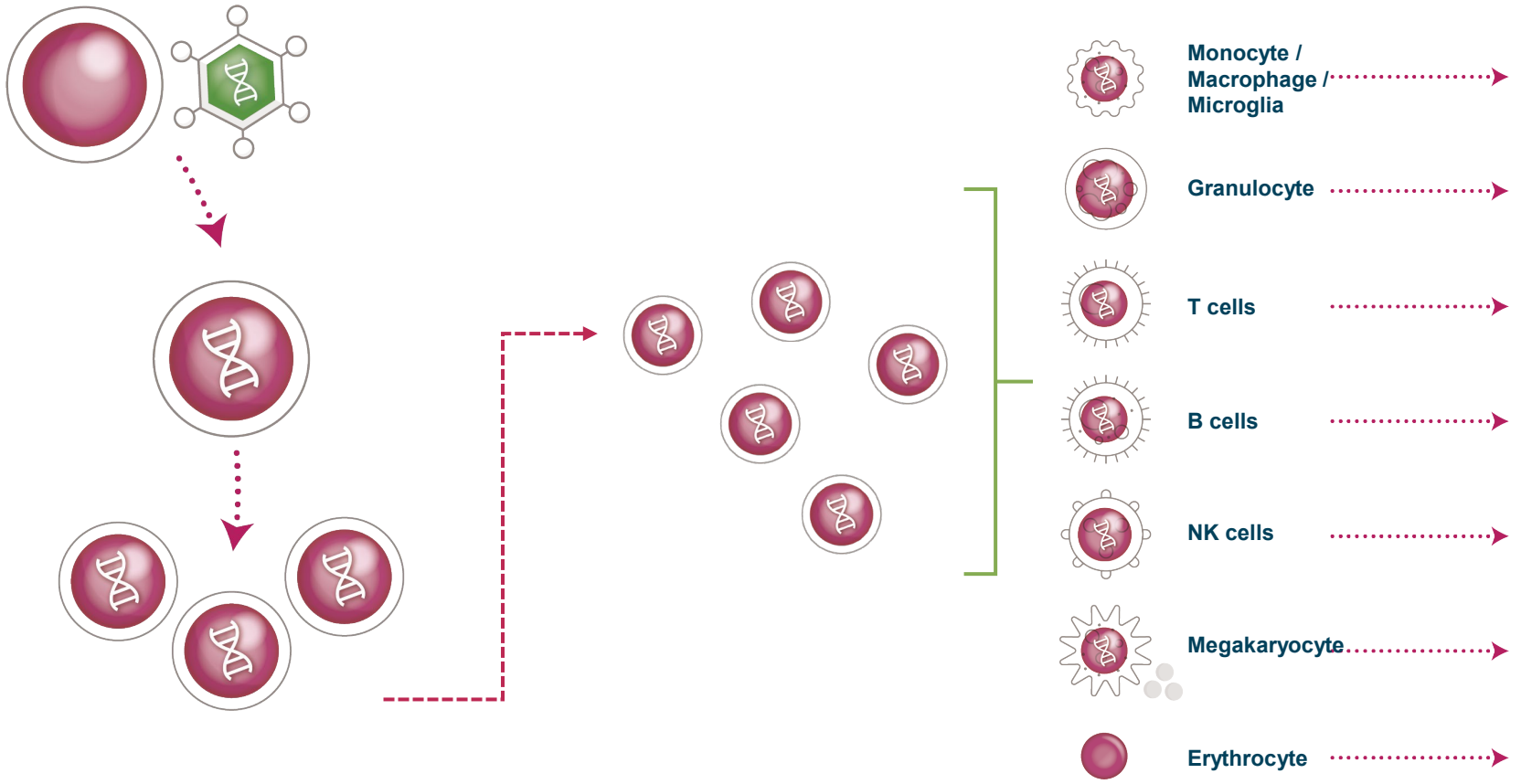
The Power of HSC Gene Therapy: *Secretion*



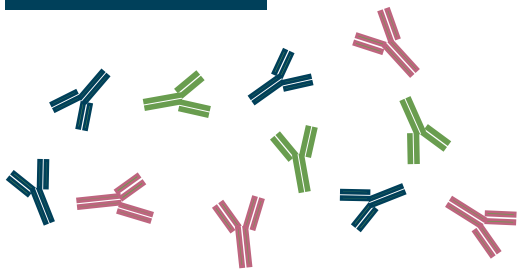
Enzymes

ARSA – MLD
IDUA - MPS-IH
SGSH – MPS-IIIA

Proteins and other factors

C1-INH - HAE

Monoclonal Antibodies



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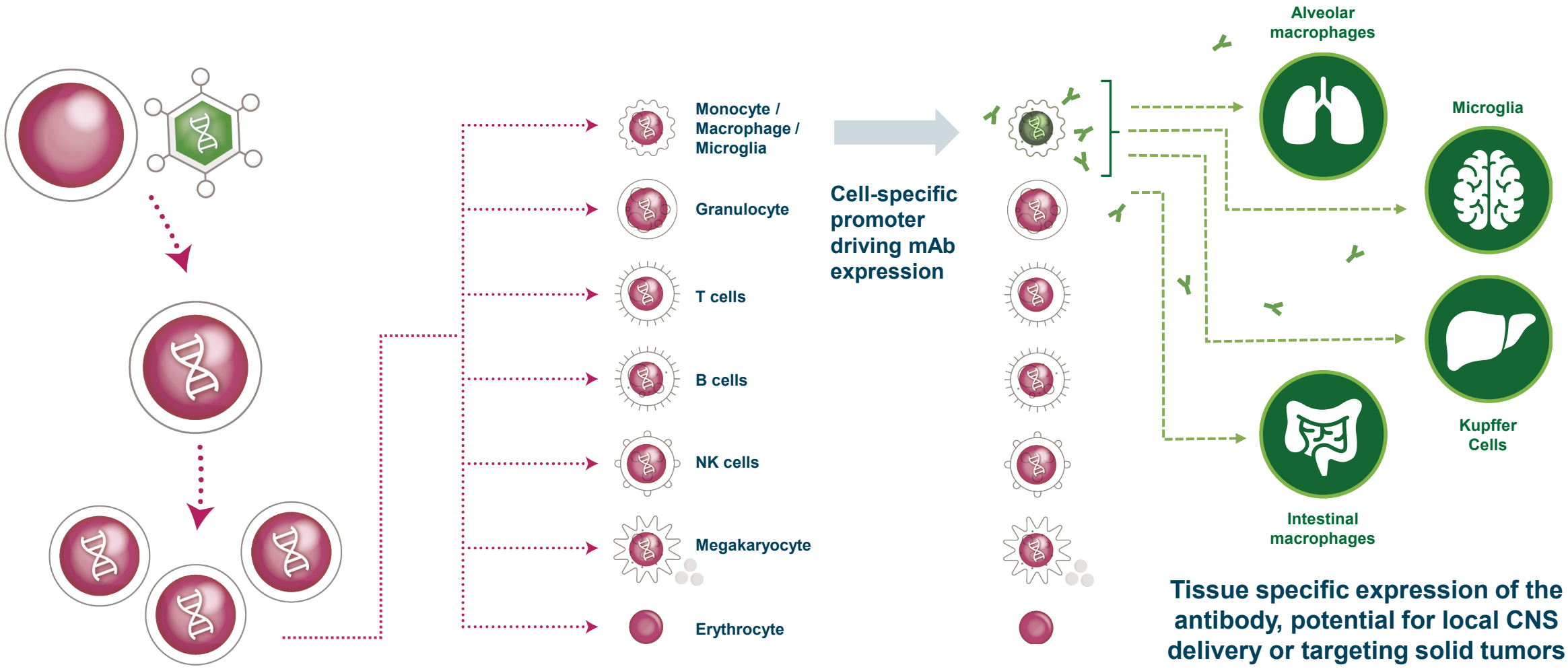
Potential for Tissue Specific Production and Secretion of mAbs



Tissue specific expression of the antibody, potential for local CNS delivery or targeting solid tumors

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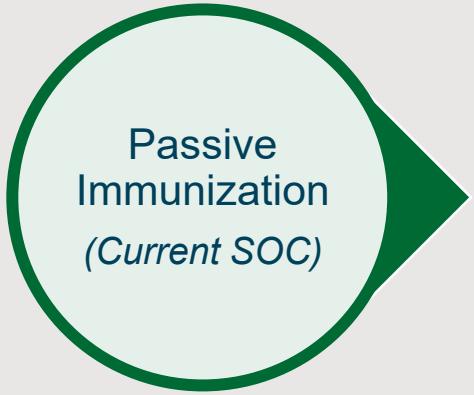
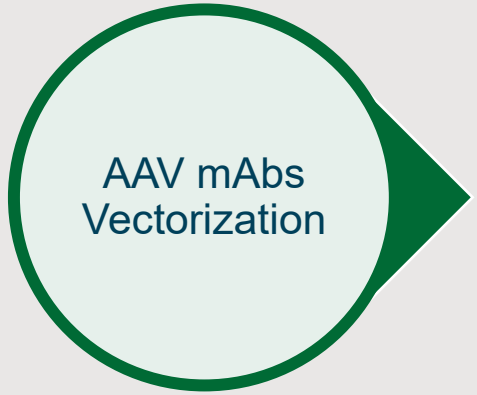
Orchard's mAbs Vectorization Offers Potential Advantages Over AAV Approaches and Current Therapies



- ***Limitations of passive immunization***
  - Repeated IV injections / short term effects
  - Limited tissue penetration and distribution across BBB
  - Limited effect for intracellular targets
  - Immune barriers to long term efficacy due to arising anti-drug antibodies



- ***Potential limitations of Vectorizing mAbs with AAV approach***
  - Limited packaging capability (4.5kb)
  - Limited tissue distribution
  - Both predicted immunogenicity (to AAV capsid) and unpredicted immunogenicity
  - Tapering levels of transgenes over time

- ***Orchard's HSC Approach to Vectorizing mAbs***
  - Larger genetic payload (10kb)
  - Broad tissue distribution
  - Low immunogenicity due to immune system reset during the autologous HSC gene therapy
  - Durability of LV delivered transgenes

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Key Takeaways for Antigen-specific Treg Cells and Vectorized Antibodies

HSC gene therapy is well-suited for severe autoimmune disorders due to ability of HSCs to differentiate into Tregs (subset of T cells that suppress inflammation)

Orchard's approach - combine demonstrated durability of HSC gene therapy in genetic diseases with suppressive potential of antigen-specific Tregs (proprietary position established)

HSC vectorization of mAbs has potential advantages over standard antibody administration in terms of efficacy and improved targeting within tissues due to migration of gene modified HSCs

New areas of research could represent significant commercial opportunities in large indications for Orchard alone or with potential partners interested in utilizing HSC approach

Orchard therapeutics

HSC Gene Therapy: Differentiators and Applications Beyond Rare

TIME	AGENDA TOPIC	SPEAKER
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7:50 – 8:00am	Q&A	

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Partnership Model for Disease Expertise in Larger Indications

THE OPPORTUNITY:
Pharma / biotech partnership

- Evaluate partners based on disease expertise and commercial footprint
- Provides support for larger market opportunities driving development resourcing and accelerating time to market

Orchard therapeutics + Pharming

Orchard and Pharming combines expertise and experience to develop a best-in-class Hereditary Angioedema gene therapy

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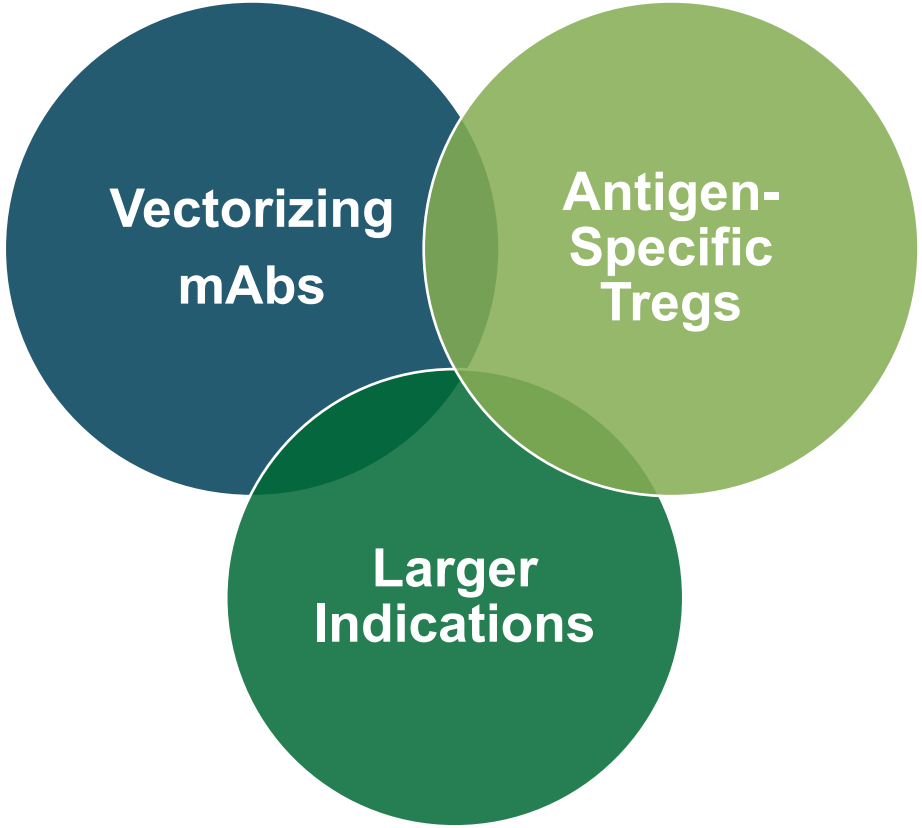
Opportunities for New Capital from Business Development or Financial Sponsors



HSC Gene Therapy Platform Approach

Partnerships built on Treg technology

- Targeted individual asset deals
- Broader platform partnerships

Partnerships using other technologies

- Leveraging work in CNS and colitis
- Vectorizing antibodies for tissue-specific delivery

Structured financings and/or joint ventures

Orchard therapeutics

HSC Gene Therapy: Differentiated Profile and Potential Beyond Rare

- HSC gene therapy has a differentiated, validated profile; not all lentiviral vectors are the same
- Promising data supports the continued advancement of OTL-104 toward IND-enabling toxicology and biodistribution studies
- New areas of research could represent significant opportunity in large indications for Orchard alone or with potential partners interested in utilizing HSC approach
- Opportunities for new capital from business development or financial sponsors

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Q&A