



A Novel Platform for Expanding Long-Term Repopulating HSCs to Enable Curative Cell Therapies

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Stem Cell Research

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Chemically defined cytokine-free expansion of human haematopoietic stem cells

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**Advanced Proprietary
Core Technology**



Clinical



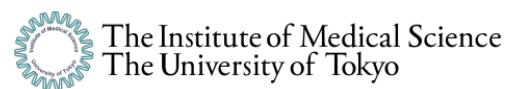
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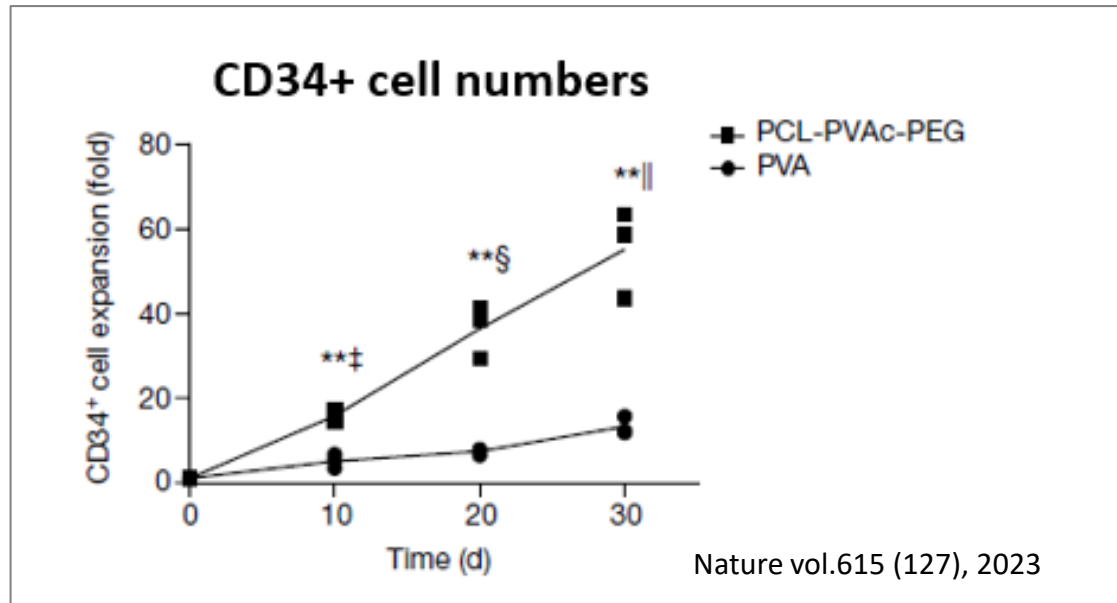
Motoo Watanabe, PhD
Executive Advisor



Our mission is to improve patients' lives through stem cell therapies

Advancement of our proprietary technology

Our original technology enabled expansion of HSPCs while preserving stem cell properties



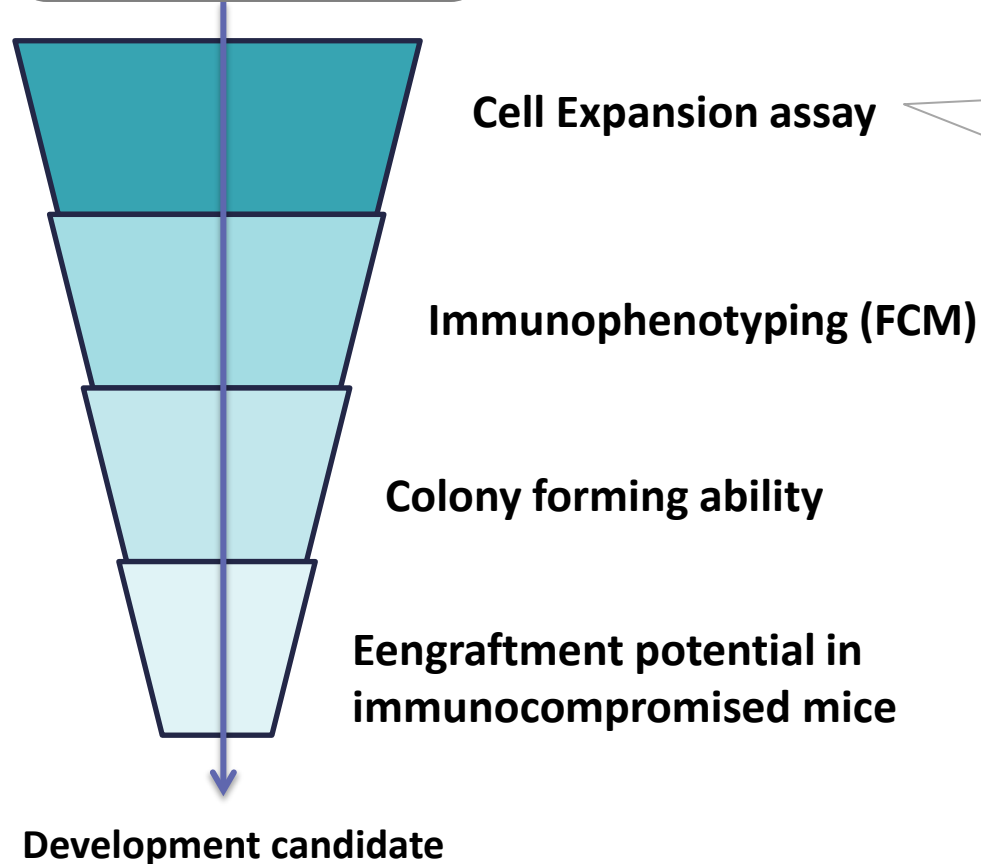
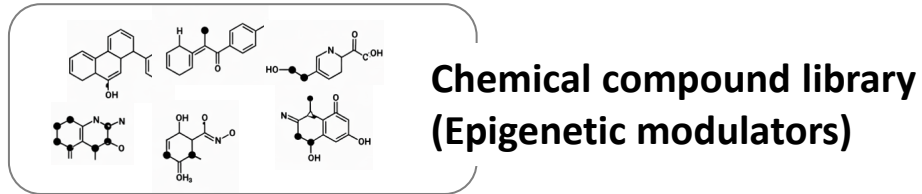
However, faster and more robust expansion is required for clinical translation



We screened chemical compounds to further enhance HSPC expansion

Hypothesis-driven screening targeting epigenetic regulators

Screening funnel

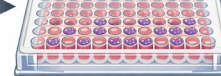
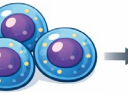


Screened for compounds that expand CD201+ cells (LT-HSC) and identified Lead compound

Umbilical Cord Blood



CD34+ cells



**Cultured
for 14 days**



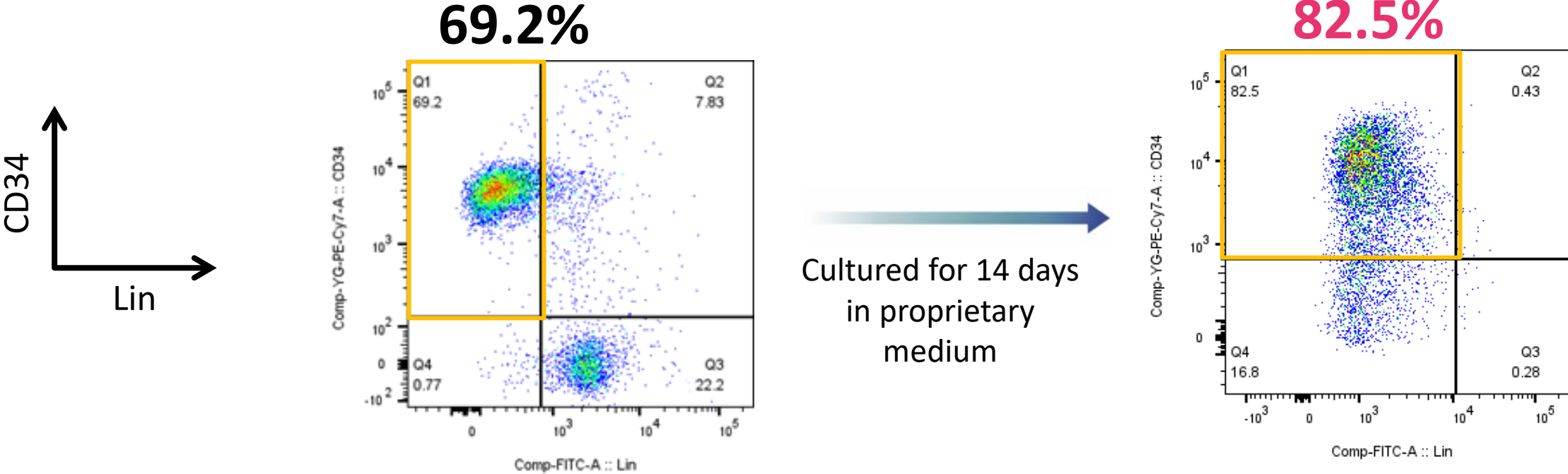
**Flow Cytometry
Analysis**

Readout: CD34+CD201+ cell number

Identified a lead compound and optimized culture conditions for CD201+ LT-HSC expansion

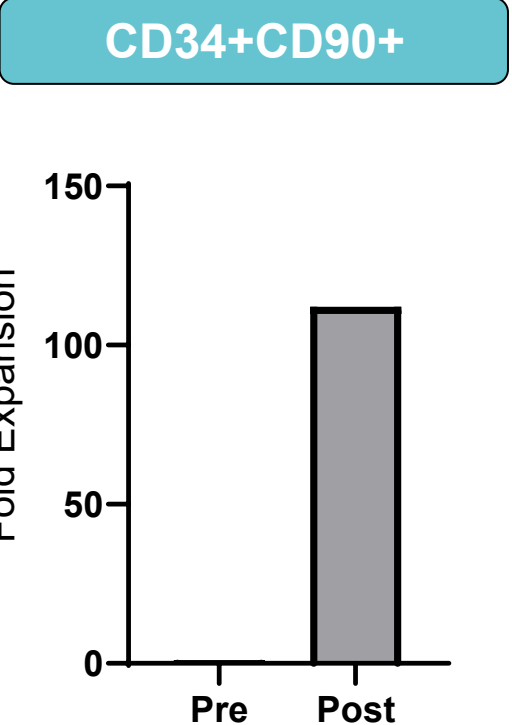
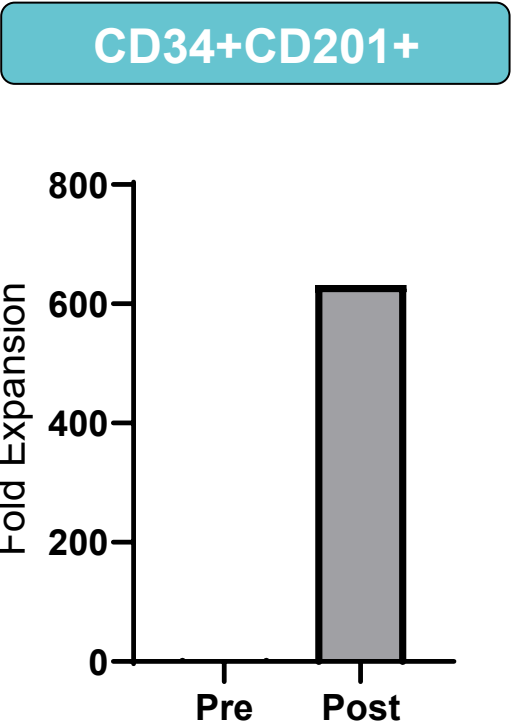
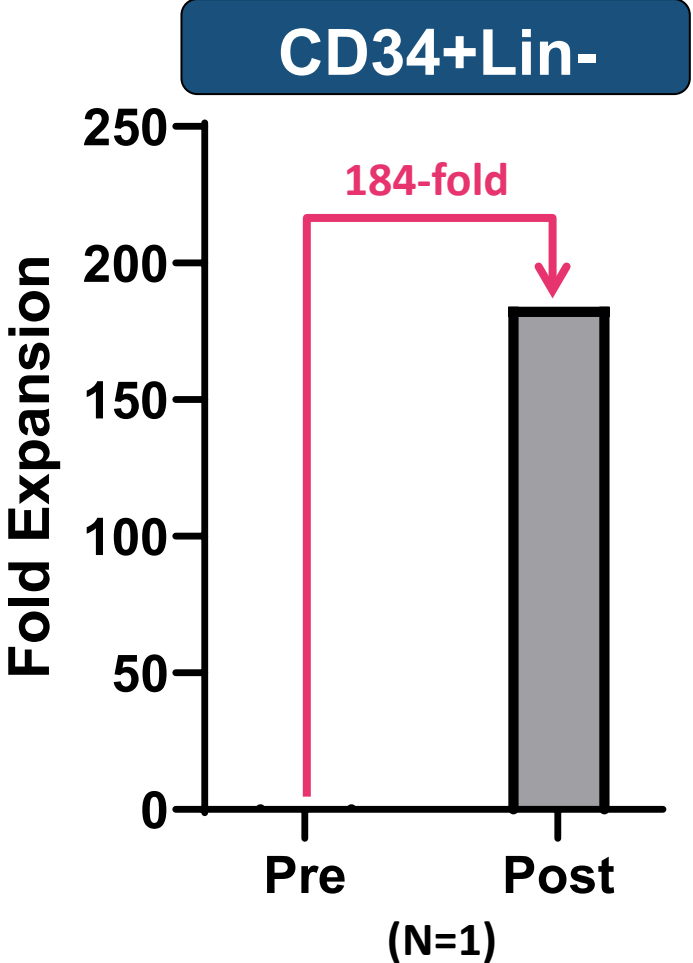
Expansion without loss of primitive HSPC phenotype

Our proprietary culture medium containing **development candidate** maintains a high proportion of CD34+Lin- cells even after 14 days of culture.



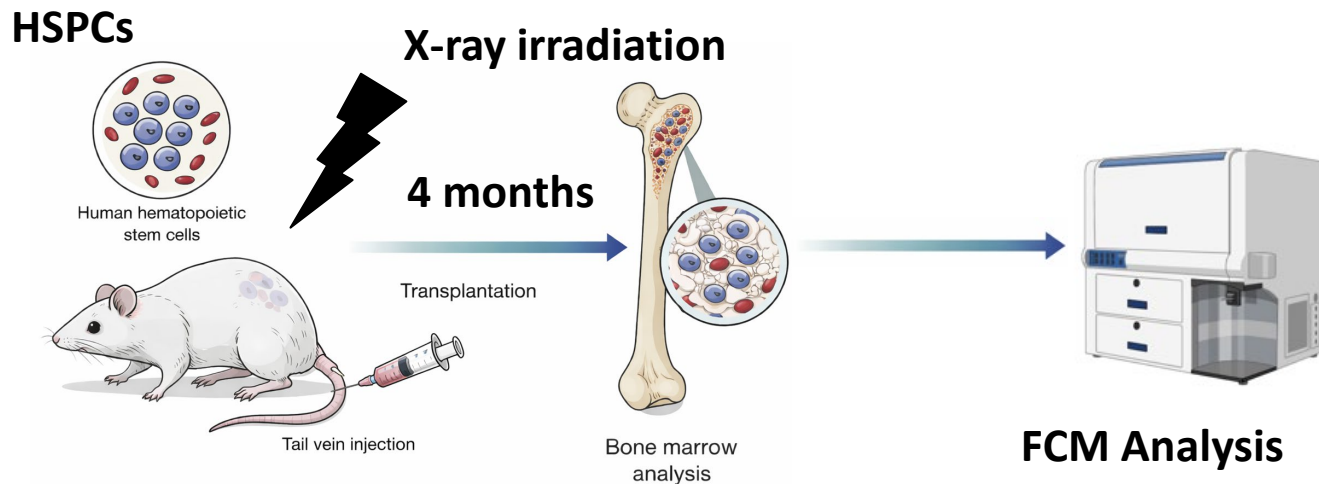
Expansion without loss of primitive HSPC phenotype

CD34+Lin- cells expanded 184-fold,
with a concurrent increase in CD201+/CD90+ primitive HSPC populations



Engraftment potential in immunocompromised mice

HSC frequency was significantly increased compared with the uncultured cells



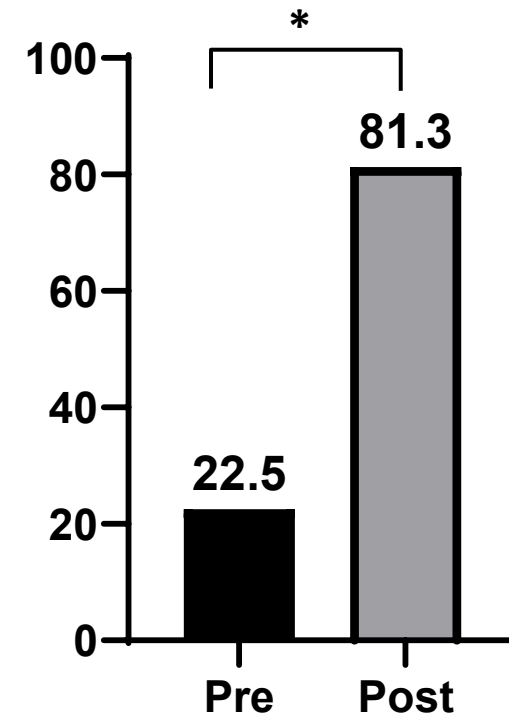
NOD/Shi-scid IL-2R γ KO

Mice were transplanted with four escalating doses of

- CB CD34⁺ cells without culture
- (equivalent) CB CD34⁺ cells with culture

The frequency of engraftable HSCs was determined by a limiting dilution assay.

Engrafted HSCs derived from 10,000
CB CD34⁺ cells



pairwise comparisons were performed using likelihood ratio tests within the ELDA framework *: $p < 0.05$

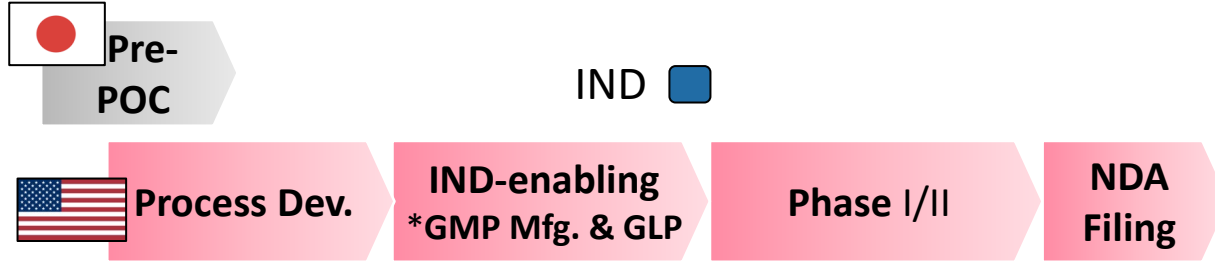
Pipeline and Platform



Pipeline

CLD-001

Non-malignant diseases

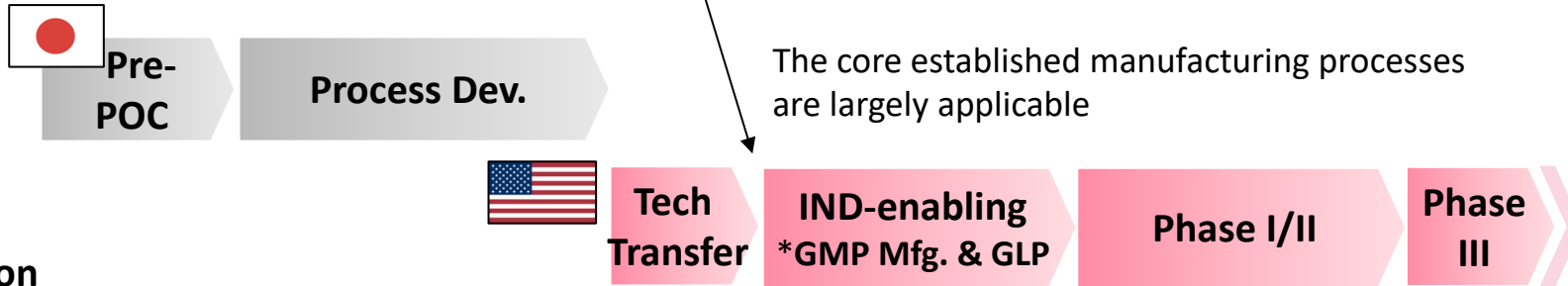


Pipeline

CLD-001

Blood cancer (Acute Leukemia)

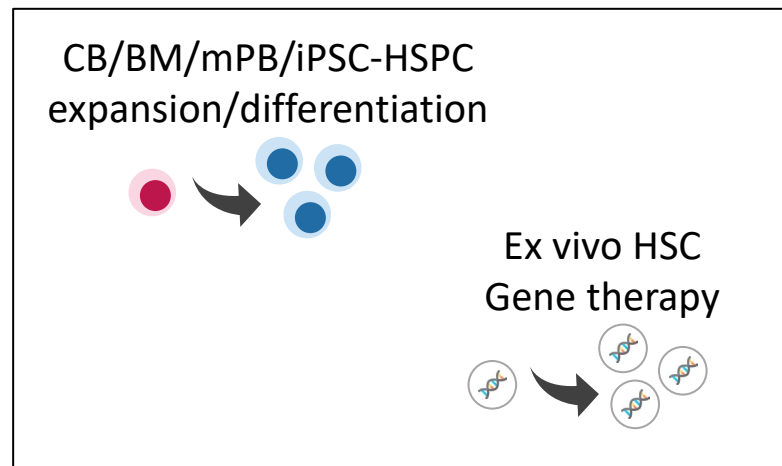
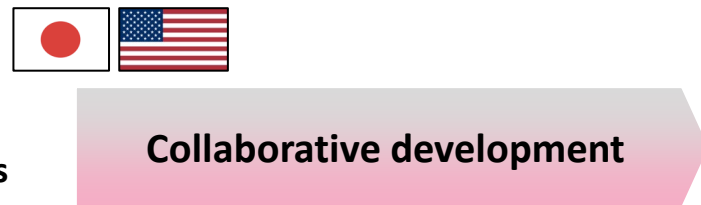
*Indication Expansion



Platform

Platform

Ex vivo Gene therapy
iPS-derived immune cells
CB-derived NK cells



Pipeline: CLD-001

- Allogenic CB derived Stem Cell Therapy
- IND (2027), P1/2 (2028) in the US

Platform

- Ex vivo expansion technologies for HSPCs derived from multiple tissue sources



Thank you

For further information:

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