Weill Cornell Medicine

Enhanced Expansion of Hematopoietic Stem Cells for Autologous Stem Cell Therapy

Lead Inventors:

Joseph Scandura, M.D., Ph.D.
Associate Professor of Medicine, Weill Cornell Medicine

Silvana Di Giandomenico, Ph.D.
Research Associate, Weill Cornell Medicine

Business Development Contact:
Brian Kelly
Director, Technology Licensing

(646) 962-7041
bjk44@cornell.edu

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Background & Unmet Need

- Hematopoietic stem cells (HSCs) play an essential role in promoting the lifelong production of mature blood cell lineages
- Stem cell transplantation (SCT) using donor-derived HSCs is an effective therapy to treat multiple hematological malignancies and cancers, including leukemia and myeloma
- However, current SCT methods suffer from limitations in effectively processing an adequate number of HSCs
- Additionally, present technologies are incapable of maintaining or expanding human HSCs *ex vivo*
- **Unmet Need:** There is a need for methods that effectively expand HSCs for use in SCT therapies

Technology Overview

- **The Technology:** Cornell inventors have identified several methods that allow for robust *ex vivo* expansion of HSCs for research and clinical use
- **PoC Data:** Human-derived HSCs cultured under hypoxic conditions (1% O_2) demonstrated increased expression of CD34+ cells, a phosphoglycoprotein which is used clinically to quantify HSCs levels (see Figure 1A)
- Co-culturing HSCs with endothelial feeder cells (ECs) also increased CD34+ expression in addition to similar markers of HSC levels (CD45, iHSC), see Figure 1B
- Treating cell cultures with N-acetyl cysteine (NAC), an antioxidant that reduces cellular damage from reactive oxygen species, likewise enhances expression of markers of HSC availability (see Figure 1C)

Inventors:
Joseph Scandura
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Patents:
US Application Filed
EP Application Filed

Publications:
Giandomenico et al. *Blood*. 2020.

Biz Dev Contact:
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Technology Applications

- Described methods effectively enhance HSC expansion and may increase efficacy of SCT therapies
- Methods may also be used to expand HSCs for research purposes
- Ability to maintain viable HSCs *ex vivo* affords ability to modify the cells (e.g., genetic manipulations)

Technology Advantages

- Multiple methods to expand HSCs may be used in isolation or in synchrony to enhance HSC expansion effects
- Described culturing conditions are easy to implement and produce robust effects on HSC expansion

Supporting Data / Figures

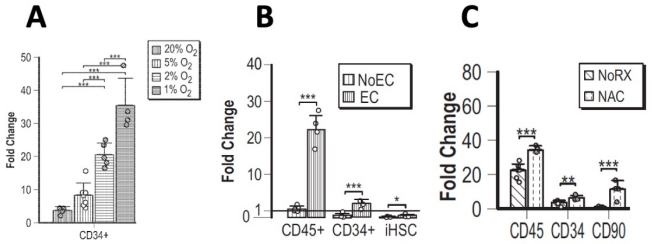

Figure 1: *Ex vivo* methods to expand HSCs. **A.** In HSCs cultured under conditions with varying oxygen levels, lower oxygen levels were associated with increased CD34+. **B.** Co-culturing HSCs with endothelial feeder cells (ECs) enhanced expression of HSC markers (CD45, CD34, iHSC). **C.** Treatment of cells with the antioxidant N-acetyl cysteine (NAC) also enhanced expression of HSC availability markers (CD45, CD34, CD90).

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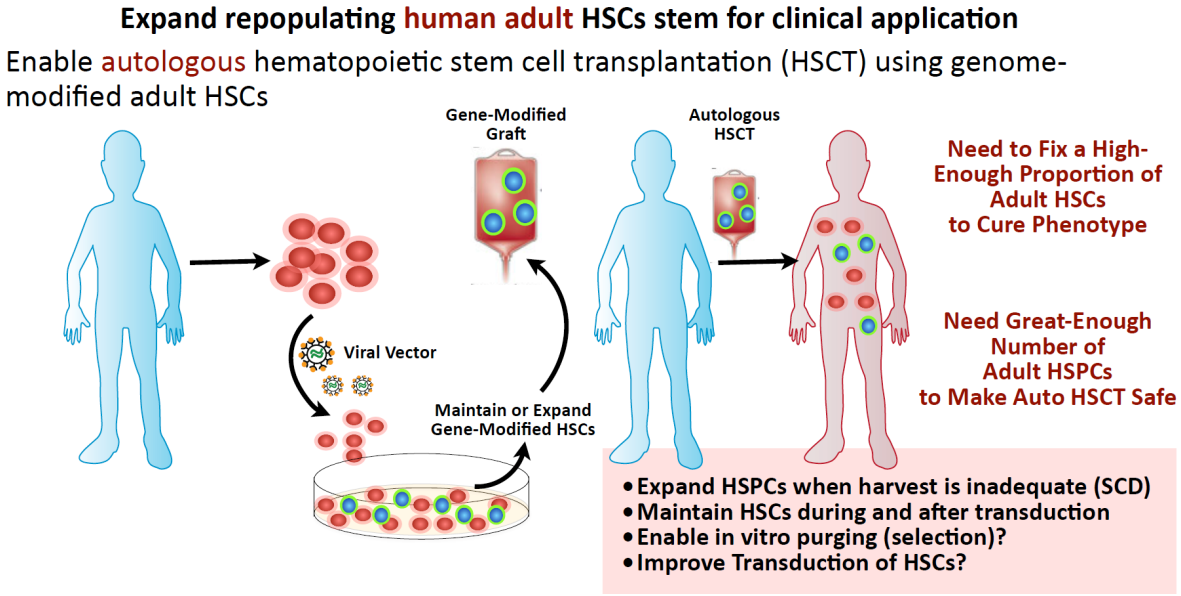

Figure 2: Schematic overview of the technology and associated clinical applications.

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