Automated Closed Cell Processing System De-Risks Gene-Edited CD34+ Hematopoietic Stem Cell Manufacturing

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OVERVIEW

- Traditional formulation, fill, finish (F/F/F) conditions include manual, high-risk steps that are prone to operator error and possible contamination.
- The Sepax™ C-Pro is an alternative for F/F/F of hematopoietic stem cells (HSCs) with similar performance to the manual condition.
- Advantages include automation, consistency, and a user-friendly interface. As a closed system, there is a lower risk of contamination.
- The Sepax™ C-Pro successfully maintained cell viability, recovery, purity and potency, which de-risks cell therapy manufacturing.

METHODS

- Purified HSCs from two donors were gene-edited and cultured. After culture, the cells were split and formulated according to a manual or Sepax™ protocol.
- For both conditions, cells were removed of their spent media and formulated for cryopreservation. The samples were then analyzed post-thaw.

Figure 1. Experimental Design

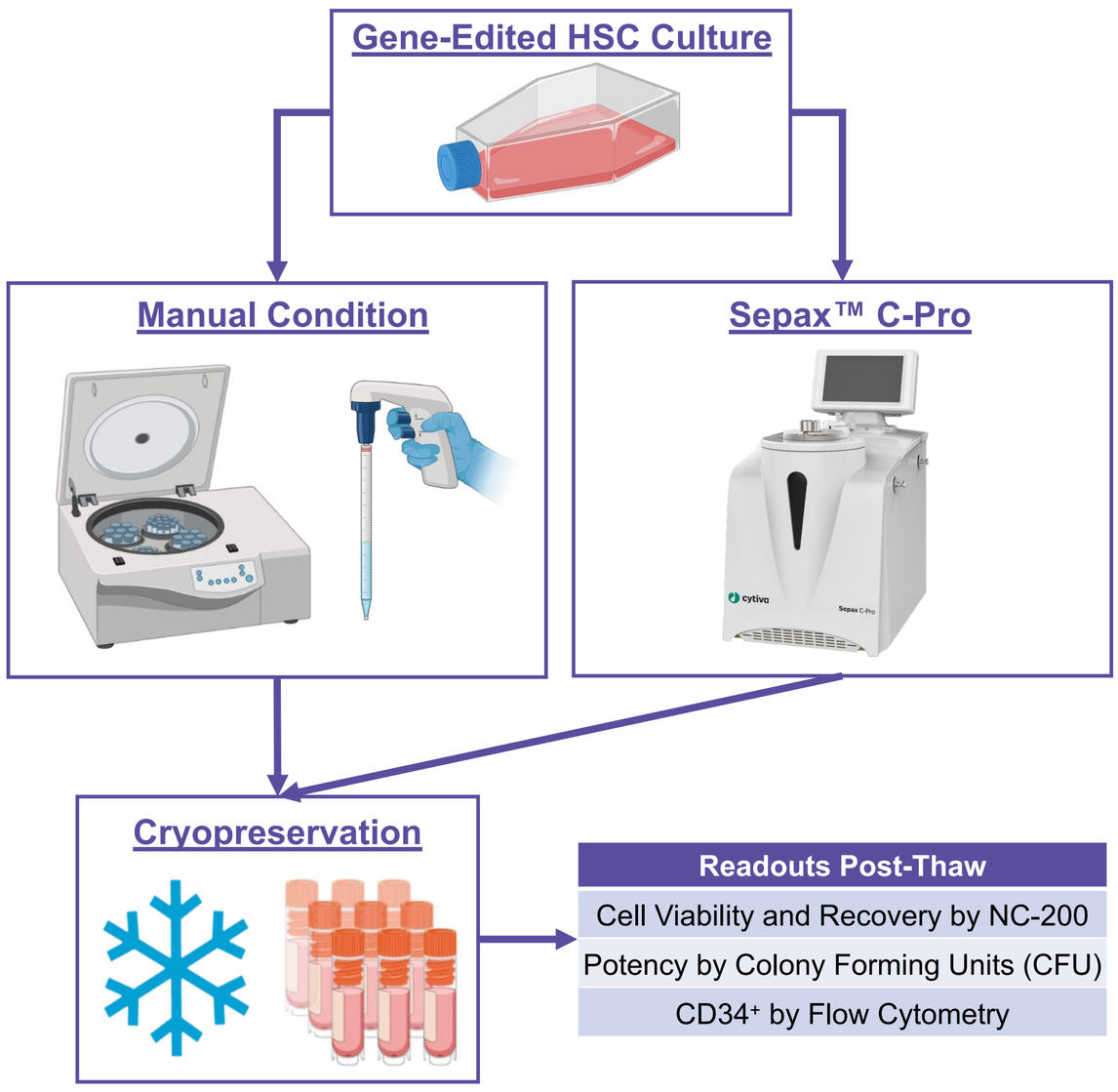
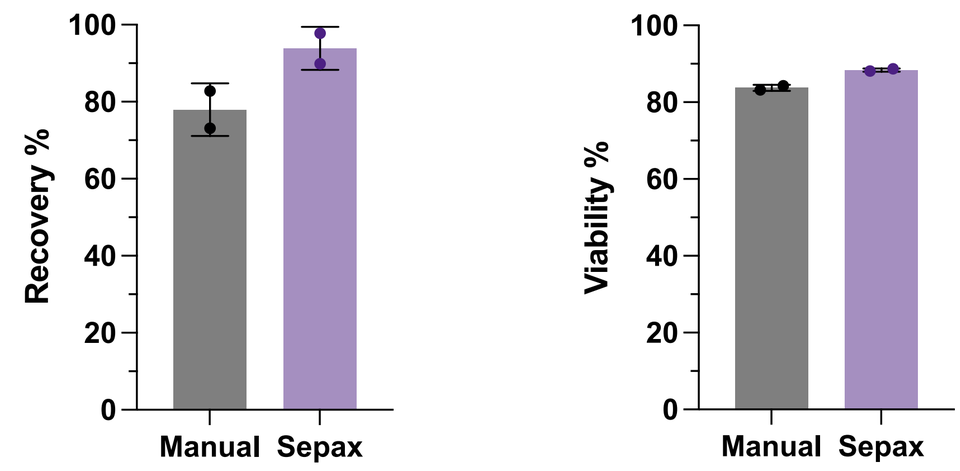


Table 1. Sepax™ C-Pro allows for closed and automated cell therapy workflows

Characteristic	Manual F/F/F	Sepax™ C-Pro
Core Technology	Centrifugation	Pneumatic syringe/centrifuge
Protocols	Multi-step	Integrated protocols to specific applications
Fill/ Finish	Manual and open	Automated and closed
Aseptic Risk	High	Low
Usability	Skilled operator	Single kit set-up Less operator interaction
Data Management	Operator's task	eSOP integration
Regulatory	Written documentation	21 CFR Part 11 Compliant
Footprint	Large	Small

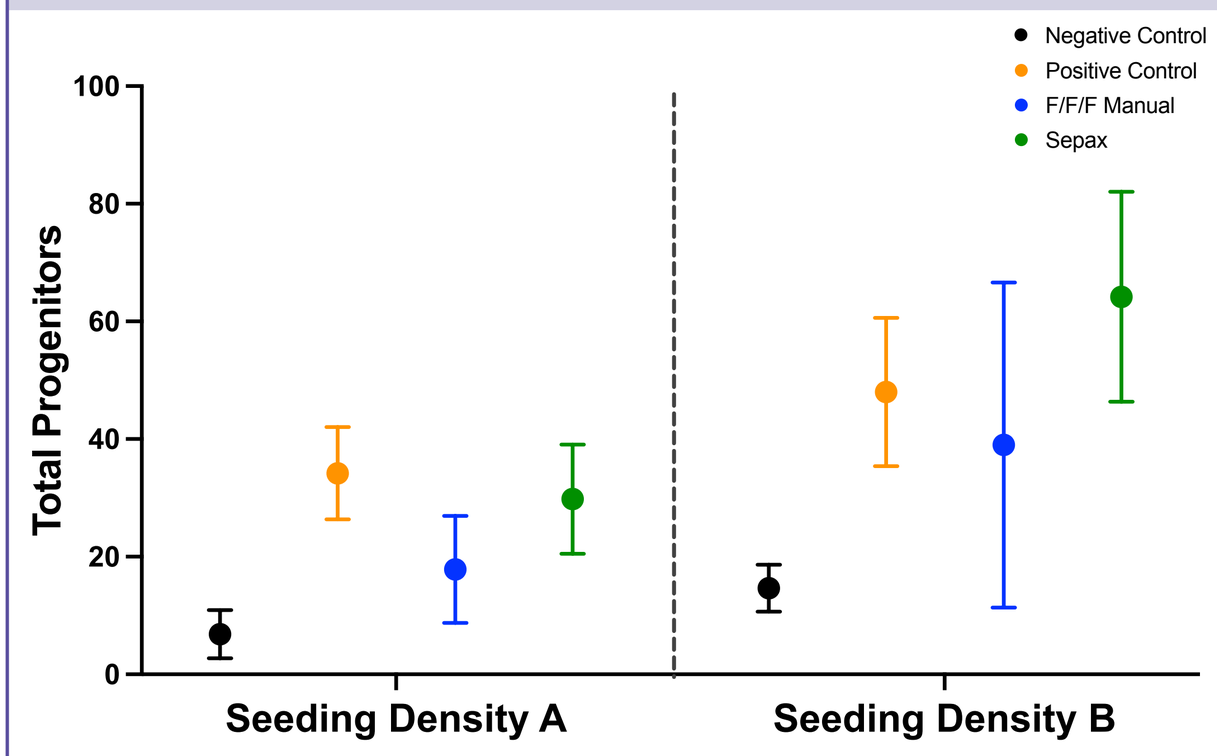
RESULTS

Figure 2. Post-thaw recovery and viability is maintained by the Sepax™ C-Pro



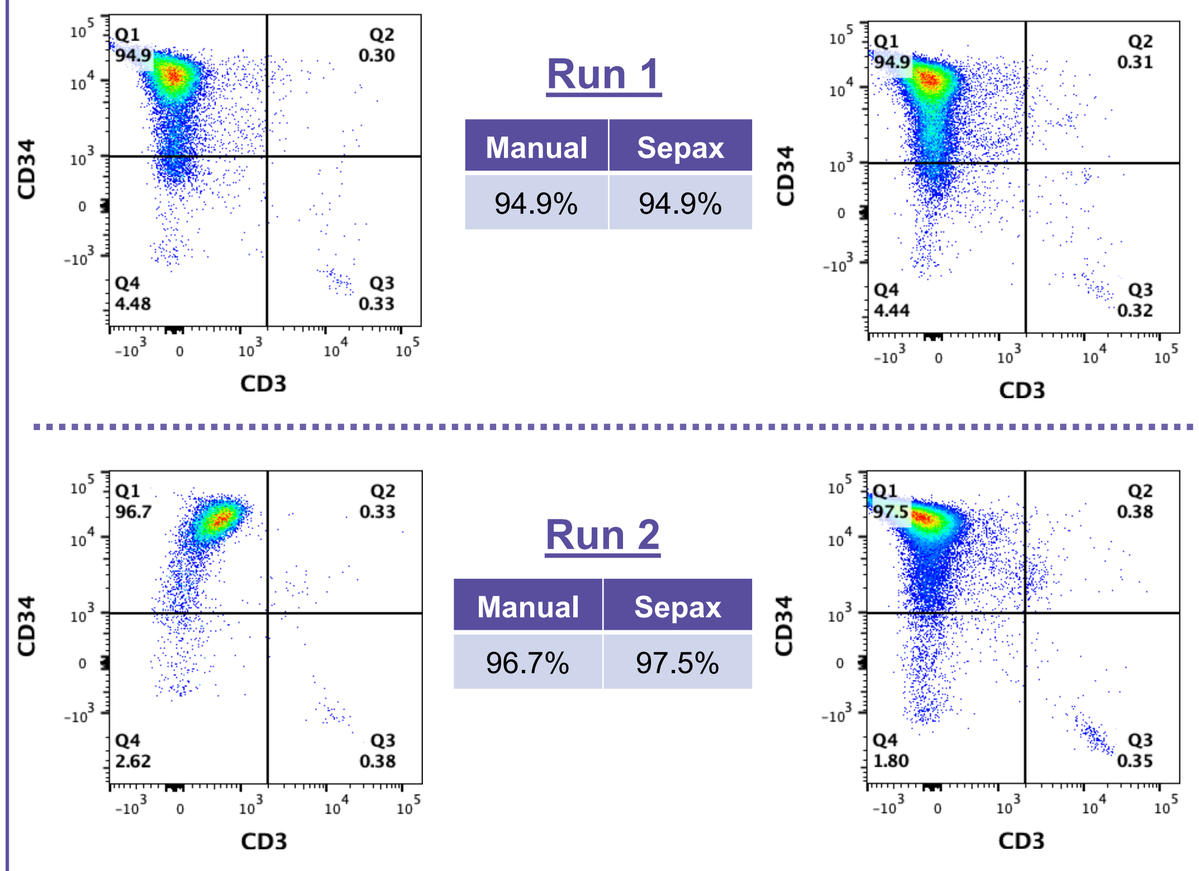
- CD34+ cell recovery post-thaw was higher in the Sepax™ C-Pro vs. the manual condition (93.9 ± 5.6% vs. 78.0 ± 6.8%, respectively).
- Cell viability remained consistent for both Sepax™ C-Pro and manual conditions (88.4 ± 0.5% vs. 83.8 ± 0.8%, respectively).

Figure 3. Sepax™ C-Pro shows no impact to CD34+ potency



- In a CFU assay, gene edited CD34+ cells were seeded at different densities in methylcellulose.
- Colonies were evaluated 14 days post-cell seeding.
- Sepax™ C-Pro did not negatively impact the number of progenitor colonies observed.

Figure 4. %CD34+ was preserved post-thaw for Sepax™ C-Pro vs. the manual condition



- The %CD34+ was comparable with 96.2 ± 1.9% in Sepax™ C-Pro and 95.8 ± 1.3% in the manual condition.

Acknowledgments

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