

Innate Lymphoid Cell (ILC) Therapy for GvHD

Product

ILC3/4 Cell Therapy

Indication

Graft Versus Host Disease

Value Propositions

- ▶ Prophylaxis and treatment of GvHD
- ▶ Potential use in other diseases such as IBD

Market

- ▶ \$384 million—Global GvHD market (7.9% CAGR 2018-2028)

Intellectual Property

- ▶ PCT pending*
- ▶ Available for licensing

Background on CU5426H

Hematopoietic stem cell transplantation (HSCT) is curative for a variety of malignant and non-malignant disorders with no other therapeutic options. Graft versus host disease (GvHD) accounts for morbidity and mortality after HSCT. GvHD occurs in 30 to 60% of HSCT cases and carries approximately a 50% mortality rate. It is expected that HSCTs will be increasingly used in the next decade, and, accordingly, the incidence of GvHD will increase. Currently, GvHD prophylaxis and treatment varies significantly among physicians and institutions. Unfortunately, many patients with GvHD remain refractory to steroids and other immunosuppressant agents.

Technical Innovation

Drs. Michael Verneris and Dejene Tufa have pioneered methods to generate and isolate subclasses of innate lymphoid cells (ILCs) derived from hematopoietic stem cells after in vitro differentiation. ILCs are tissue resident lymphocytes that are involved in the homeostasis of various organ systems. ILC2 and ILC3s are important for intestinal health and are depleted in cases of GvHD. These ILC subclasses could be used as GvHD prophylaxis in anticipation of HSCT or as direct treatment of GvHD following HSCT. Inventor experiments infusing stem cell-derived ILC2 and ILC3s into GvHD mouse models have demonstrated improvement in GvHD score, intestinal inflammation, and overall survival (see Figure below). The inventors believe that similar benefit can be demonstrated in human cases of GvHD and other causes of intestinal inflammation such as Crohn's disease.

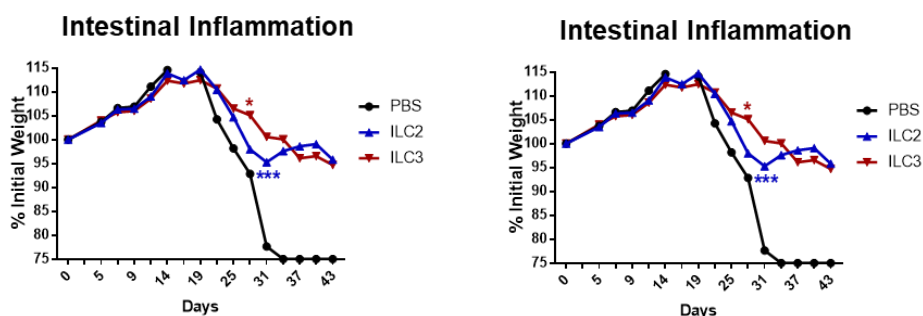


Figure: Three depictions of PBS control (GvHD model w/o intervention, black line) compared to GvHD model with ILC2 and ILC3 IV infusion (in blue and red, respectively). **Left:** Weight as a percentage of beginning as a reflection of intestinal inflammation.

Resources & Documents:

**PCT pending: PCT/US21/52449—"Methods to Generate Human ILC2 and LTi-ILC3 Cells from Hematopoietic Stem Cells using CD48 ligation"—Filed September 28th, 2021.*

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