

Use of immunosuppression to enable engraftment of allogeneic hematopoietic stem cells

In 2021, almost 200,000 patients were diagnosed with leukemia and other blood and immune diseases in the US. Stem cell transplants can be used to restore healthy bone marrow in patients with these diseases by stimulating new bone marrow growth and restoring the blood and immune system. A challenge faced by stem cell transplants is the successful engraftment of hematopoietic stem cells, which is necessary to sustain long-term and effective hematopoiesis. The Czechowicz Lab at Stanford has demonstrated that antibody-based immunosuppression can enable engraftment of allogeneic hematopoietic stem cells. They have shown that this treatment alone is sufficient to enable engraftment of allogeneic hematopoietic stem cells even in mismatched settings. Additionally, they have shown that in bone marrow failure syndromes (e.g. Fanconi Anemia) this is sufficient for disease cure. Furthermore, the invention can be combined with CD117 mAb conditioning. Existing methods to enable stem cell engraftment require chemotherapy and/or irradiation, which are non-specific and cause significant toxicities in patients. The invention offers a safer alternative by using antibodies that offer immune and HSC-targeted conditioning to patients.

Applications

- Curative treatment option for multiple diseases – particular focus on bone marrow failure disorders (e.g. Fanconi Anemia)
- Hematopoietic stem-cell therapies

Advantages

- Does not require chemotherapy, irradiation and/or HSC-targeted conditioning

Patents

- Published Application: [WO2023133207](#)
- Published Application: [20250177452](#)

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