

Ryoncil[®] (remestemcel-L-rknd) – New orphan drug approval

- On December 18, 2024, the [FDA announced](#) the approval of [Mesoblast's Ryoncil \(remestemcel-L-rknd\)](#), for the treatment of **steroid refractory acute graft versus host disease (SR- aGvHD) in pediatric patients** 2 months of age and older.
- **SR-aGvHD is a serious and life-threatening condition** that can occur as a complication of allogeneic hematopoietic stem cell transplantation (allo-HSCT).
 - Approximately 10,000 patients undergo an allo-HSCT in the U.S. annually, including 1,500 children. **Approximately 50% develop aGvHD and almost half of those do not respond to steroids**, the first-line treatment.
- Ryoncil is the **first FDA approved allogeneic bone marrow-derived mesenchymal stromal cell (MSC) therapy**. These MSCs are isolated from the bone marrow of healthy adult human donors.
- The efficacy of Ryoncil was established in a prospective, single-arm study in 54 pediatric patients with SR-GvHD after receiving allo-HSCT. The main outcome measures were day-28 overall response rate (complete response rate and partial response rate) and the duration of response.
 - The overall response rate was 70% (95% CI: 56.4, 82.0).
 - The median duration of response was 54 days (95% CI: 7, 159+).
- Warnings and precautions for Ryoncil include hypersensitivity/acute infusion reactions, transmission of infectious agents, and ectopic tissue formation.
- The most common non-laboratory adverse reactions ($\geq 20\%$) with Ryoncil use were viral infectious disorders, bacterial infectious disorders, infection – pathogen unspecified, pyrexia, hemorrhage, edema, abdominal pain, and hypertension.
- The recommended dosage of Ryoncil is 2×10^6 MSC/kg body weight per intravenous infusion given **twice a week for 4 consecutive weeks for a total of 8 infusions**. Infusions should be administered at least 3 days apart.
- Mesoblast's launch plans for Ryoncil are pending. Ryoncil will be available as a cell suspension for intravenous infusion in a target concentration of 6.68×10^6 MSCs per mL in 3.8 mL contained in a 6 mL cryovial.