

## Aucatzyl<sup>®</sup> (obecabtagene autoleucel) – New orphan drug approval

- On November 8, 2024, the [FDA announced](#) the approval of [Autolus' Aucatzyl \(obecabtagene autoleucel\)](#), for the treatment of adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL).
- Aucatzyl is a B-lymphocyte antigen CD19 (CD19) chimeric antigen receptor (CAR) T cell therapy.
- The efficacy of Aucatzyl was established in an open-label, single-arm study in patients with relapsed or refractory B-cell ALL. Patients received Aucatzyl as a split dose infusion with a total recommended dose of  $410 \times 10^6$  CD19 CAR-positive viable T cells. The major efficacy measures were rate and duration of complete remission within 3 months after infusion. The efficacy evaluable population included 65 patients.
  - The complete remission (within 3 months) rate was 42% (95% CI: 29, 54). The median duration of complete remission was 14.1 months (95% CI: 6.1, not reached).
- Aucatzyl carries a boxed warning for cytokine release syndrome (CRS), neurologic toxicities, and secondary hematological malignancies.
- Additional warnings and precautions for Aucatzyl include prolonged cytopenias, infections, hypogammaglobulinemia, hemophagocytic lymphohistiocytosis/macrophage activation syndrome, and hypersensitivity reactions.
- The most common (non-laboratory) adverse reactions ( $\geq 20\%$ ) with Aucatzyl use were CRS, infections - pathogen unspecified, musculoskeletal pain, viral infections, fever, nausea, bacterial infectious disorders, diarrhea, febrile neutropenia, immune effector cell-associated neurotoxicity syndrome, hypotension, pain, fatigue, headache, encephalopathy, and hemorrhage.
- The total recommended dose of Aucatzyl is  $410 \times 10^6$  CD19 CAR-positive viable T cells to be administered as split dose intravenous infusion on day 1 and day 10 ( $\pm 2$  days) based on bone marrow blast assessment and preceded by fludarabine and cyclophosphamide lymphodepleting chemotherapy.
  - Refer to the Aucatzyl drug label for complete dosing and administration recommendations.
- Autolus' launch plans for Aucatzyl are pending.