

Rethymic® (allogeneic processed thymus tissue-agdc) – New orphan drug approval

- On October 8, 2021, the [FDA announced](#) the approval of [Enzyvant's Rethymic \(allogeneic processed thymus tissue-agdc\)](#), for immune reconstitution in pediatric patients with congenital athymia.
 - Rethymic is not indicated for the treatment of patients with severe combined immunodeficiency (SCID).
- Congenital athymia is an ultra-rare immune disorder in which a child is born without a thymus. Children impacted by this disease typically die within the first two years of life and may have repeated, often life-threatening infections because they lack adequate working T cells.
 - The estimated incidence of pediatric congenital athymia in the U.S. is 17 to 24 live births each year.
- Rethymic is the first FDA-approved treatment indicated for immune reconstitution in pediatric patients with congenital athymia.
 - Rethymic is composed of human allogeneic (donor-derived) thymus tissue that is processed and cultured, and then implanted into patients to help reconstitute immunity in patients who are athymic. Dosing is patient customized, determined by the surface area of the Rethymic slices and the body surface area (BSA) of the patient.
- The efficacy of Rethymic was established in ten prospective, single-center, open-label studies that enrolled a total of 105 patients, including 95 patients in the primary efficacy analysis. Patients in the efficacy population received Rethymic in a single surgical procedure at a dose of 4,900 to 24,000 mm² of Rethymic / recipient BSA in m².
 - The Kaplan-Meier estimated survival rates were 77% (95% CI: 0.670, 0.841) at 1 year and 76% (95% CI: 0.658, 0.832) at 2 years. For patients who were alive at 1 year after treatment with Rethymic, the survival rate was 94% at a median follow-up of 10.7 years.
 - Without treatment, congenital athymia is fatal in childhood. In a natural history population observed from 1991 through 2017, 49 patients diagnosed with congenital athymia received supportive care only. The 2-year survival rate was 6%, with all patients dying by 3 years of age. The most common cause of death was infection in 26 (53%) patients.
- Warnings and precautions for Rethymic include infection control and immunoprophylaxis, graft versus host disease, autoimmune disorders, renal impairment, cytomegalovirus infection, malignancy, transmission of serious infections and transmissible infectious diseases, vaccine administration, anti-HLA antibodies, and HLA typing.
- The most common adverse reactions (> 10%) with Rethymic use were hypertension, cytokine release syndrome, rash, hypomagnesemia, renal impairment/failure, thrombocytopenia, and graft versus host disease.
- Rethymic is administered by a surgical procedure as a one-time dose. For complete dosage and administration recommendations, refer to the Rethymic drug label.

- Enzyvant's launch plans for Rethymic are pending.



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