ENZYVANT Resubmits Biologics Licensing Application (BLA) to FDA for RVT-802 for Pediatric Congenital Athymia

CAMBRIDGE, Mass., April 27, 2021 – Enzyvant today announced the resubmission of the Biologics Licensing Application (BLA) to the U.S. Food and Drug Administration (FDA) for RVT-802, cultured human thymus tissue. RVT-802 is a one-time tissue-based regenerative therapy for the treatment of pediatric patients with congenital athymia.

The FDA made several regulatory requests related to Chemistry, Manufacturing and Controls (CMC) in a December 2019 Complete Response Letter (CRL) that followed the initial RVT-802 BLA submission in April 2019. Enzyvant has been working since that time to address each of the FDA requests. The expected action date provided by the FDA under the Prescription Drug User Fee Act (PDUFA) is October 8, 2021.

“Resubmission of the RVT-802 BLA is important progress in the mission to bring a desperately needed treatment option to families battling the dire consequences of congenital athymia,” said Rachelle Jacques, CEO of Enzyvant. “Thank you to everyone involved in the RVT-802 development program, including families who participated in clinical trials, for your efforts to achieve this milestone and make a difference in the lives of families who will face this condition in the future.”

An ultra-rare condition in which children are born without a thymus, congenital athymia can lead to profound immunodeficiency, immune dysregulation and high susceptibility to infections. With only supportive care, patients with congenital athymia typically die from infections or autoimmune manifestations by age two or three. Currently, there are no FDA-approved treatments available for congenital athymia.

“The bleak prospects of children with congenital athymia have driven our work over the past two decades to develop a therapy that will provide a functioning thymic environment and enable patients to fight off fatal infections,” said Louise Markert, M.D., Ph.D., principal investigator for RVT-802 clinical trials and Professor of Pediatrics and Immunology at the Duke University School of Medicine. “Resubmission of the RVT-802 BLA is a reason for families to be optimistic that an approved treatment option for congenital athymia may soon be available.”

About Congenital Athymia

Children with congenital athymia are born without a thymus, making them severely immunodeficient and unable to fight infections. Athymia is initially detected by T-cell deficiency observed in newborn screening for SCID (severe combined immune deficiency), which is now required in all 50 U.S. states. SCID and congenital athymia are both primary immunodeficiency disorders but they are distinct conditions. Congenital athymia is associated with multiple conditions such as complete DiGeorge Anomaly (cDGA), CHARGE syndrome, FOXN1 deficiency, TBX1 gene mutation and diabetic embryopathy. Pediatric congenital athymia is ultra-rare with an estimated incidence in the U.S. of ~17 to 24 live births each year.

About Investigational RVT-802

Investigational RVT-802 is a novel one-time tissue-based regenerative therapy that has been studied by researchers at Duke University across 10 clinical studies for more than 25 years. Investigational RVT-802 is cultured human thymus tissue for the treatment of pediatric congenital athymia and is implanted in a single surgery. It is designed to establish a functioning thymic environment where T cells can develop the ability to correctly identify and interact with antigens and fight infections. Investigational RVT-802 has been granted multiple FDA designations: Regenerative Medicine Advanced Therapy (RMAT), Breakthrough Therapy, Rare Pediatric Disease and Orphan Drug. The European Medicines Agency (EMA) has granted Orphan Drug designations and the Advanced Therapy Medicinal Product (ATMP) designation for RVT-802.

About Enzyvant

Enzyvant, a wholly owned subsidiary of Sumitovant Biopharma Ltd. (wholly owned by Sumitomo Dainippon Pharma Co., Ltd.), is a biotechnology company dedicated to developing novel, transformative regenerative therapies for people with devastating rare diseases. Enzyvant's lead asset is the investigational tissue-based regenerative therapy, RVT-802, for congenital athymia, an ultra-rare and life-threatening pediatric immunodeficiency. RVT-802 has been granted multiple regulatory designations, including the U.S. Food and Drug Administration designation as a Regenerative Medicine Advanced Therapy (RMAT).

For more information about Enzyvant, visit Enzyvant.com. Follow @Enzyvant on Twitter, Facebook and LinkedIn.

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