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US FDA approves first gene therapy for severe Leukocyte Adhesion Deficiency Type I

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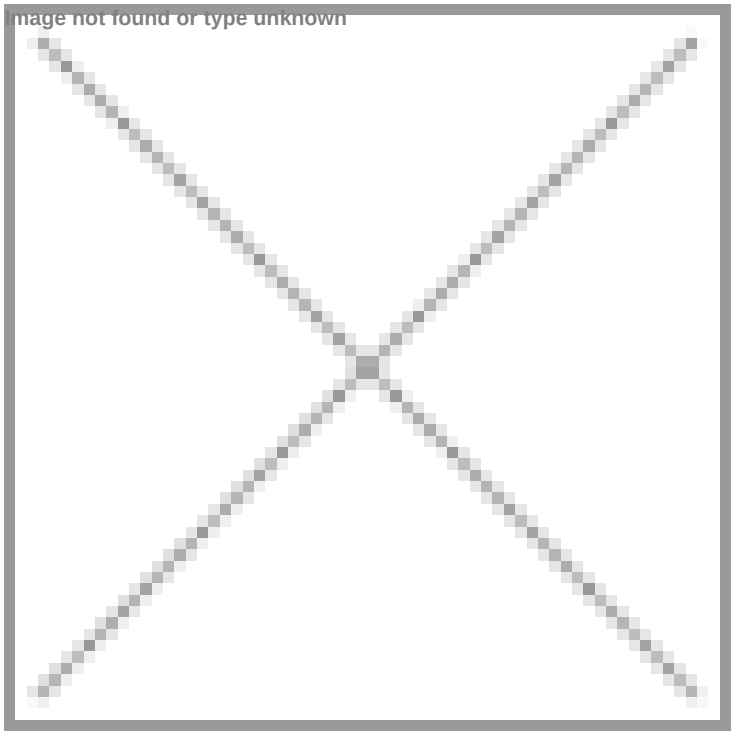
A single dose of Kresladi is infused intravenously to address the underlying cause of severe LAD-I



The US Food and Drug Administration (FDA) has approved Rocket Pharmaceuticals' Kresladi (marnetegragene autotemcel), the first gene therapy for the treatment of severe Leukocyte Adhesion Deficiency Type I (LAD-I).

Kresladi is indicated for the treatment of pediatric patients with severe leukocyte adhesion deficiency I (LAD-I) due to biallelic variants in *ITGB2* without an available human leukocyte antigen (HLA)-matched sibling donor for allogeneic hematopoietic stem cell transplant.

Severe LAD-I is a rare, inherited immune deficiency caused by mutations in the *ITGB2* gene, which prevent white blood cells from effectively fighting infections.

Kresladi consists of the patient's own hematopoietic (blood) stem cells (HSCs), which are genetically modified to introduce functional copies of the *ITGB2* gene. Following conditioning, a single dose of Kresladi is infused intravenously to address the underlying cause of severe LAD-I by restoring CD18 and CD11a cell surface expression in white blood cells, including neutrophils.