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(54) Title: MATERIALS AND METHODS FOR THE TREATMENT OF LYSOSOMAL ACID LIPASE DEFICIENCY (LAL-D)

(57) Abstract: The disclosure provides gene therapy vectors, such as adeno-associated virus (AAV), designed for treatment of Lysosomal Acid Lipase Deficiency (LAL-D) disorders such as Wolman Disease and cholesterol ester storage disease (CESD). The disclosed rAAV provide a wild type lipase A (LIRA) cDNA to a subject in need which results in expression of the wild type protein.



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