



Gene Therapy for Severe Leukocyte Adhesion Deficiency-I (LAD-I)

Results from the First Treated Patient

What is LAD-I?

LAD-I is a rare genetic disorder that affects the body's immune system. White blood cells (specifically neutrophils) are unable to produce a protein called **CD18**. Without functioning CD18, the neutrophils are unable to successfully go to the infection to fight it.

DIAGNOSIS

FLOW CYTOMETRY
<2% CD18 expression on neutrophils

GENETIC TESTING
ITGB2 gene mutation

SYMPTOMS & PROGNOSIS



Delayed umbilical cord separation, recurrent severe infections (e.g., skin lesions)



Antibiotics generally ineffective



Frequent hospitalizations



Generally poor Quality of Life



A clinical trial studying gene therapy as a curative option is underway

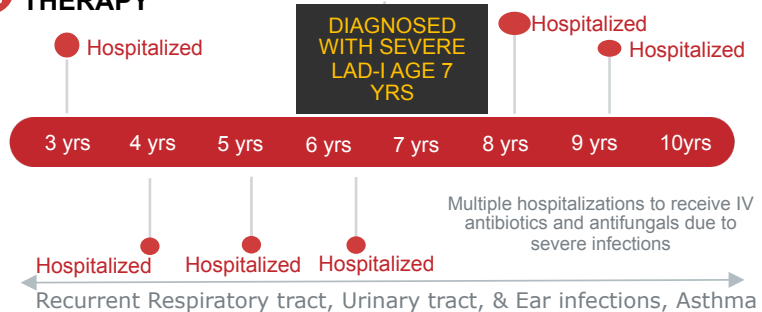
Clinical trials.gov: NCT03812263



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1ST PATIENT SUCCESSFULLY TREATED WITH GENE THERAPY³

MEDICAL HISTORY OF 1ST PATIENT TREATED WITH GENE THERAPY



KEY STEPS IN GENE THERAPY FOR LAD-I:

- 1 Blood stem cells are taken from the patient
- 2 Therapeutic gene is inserted into the patient's own cells at a lab
- 3 Patient will receive a drug called busulfan to help get body ready to receive the gene therapy
- 4 Finally, patient receives gene therapy product

RESULTS SHOW PATIENT TOLERATED TREATMENT WELL WITH CD18>47% & VISIBLE SIGNS OF IMPROVEMENT IN SKIN LESIONS

BEFORE TREATMENT

- CD18 <1%
- Recurrent skin infections
- Multiple hospitalizations



6-MONTH AFTER TREATMENT

- CD18 >47%
- Visible signs of healing skin lesions
- No safety issues
- Infusion well-tolerated



References:

1. Qasim W et al. Pediatrics 2009; 123: 836-40
2. Almaraz Novoa E et al. J Allergy Clin Immunol Pract. 2018 July-August (6) 1418-1420.
3. <https://www.rocketpharma.com/wp-content/uploads/2020/05/ASGCT-I-ADI-Kohn-05-2020-fixed.pdf>
4. <https://rocketpharmaceuticals.qcs-web.com/news-releases/news-release-details/rocket-pharmaceuticals-presents-positive-updates-fa-and-lad-i>



This clinical trial is currently enrolling:

✉ LADclinicaltrial@rocketpharma.com

🌐 <https://www.rocketpharma.com/lad-i-clinical-trial-for-patients/>

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