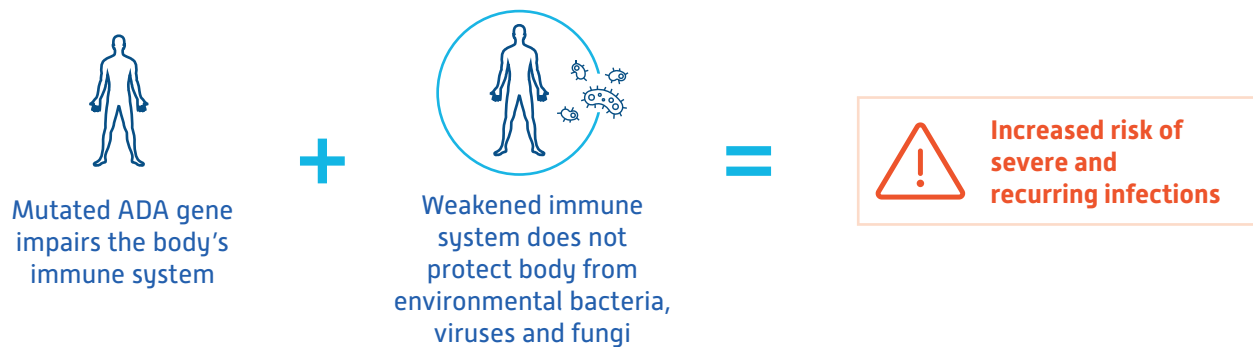


## What is ADA-SCID?<sup>1</sup>

Adenosine deaminase severe combined immunodeficiency (ADA-SCID) is a rare immune disorder caused by a deficiency in adenosine deaminase (ADA).

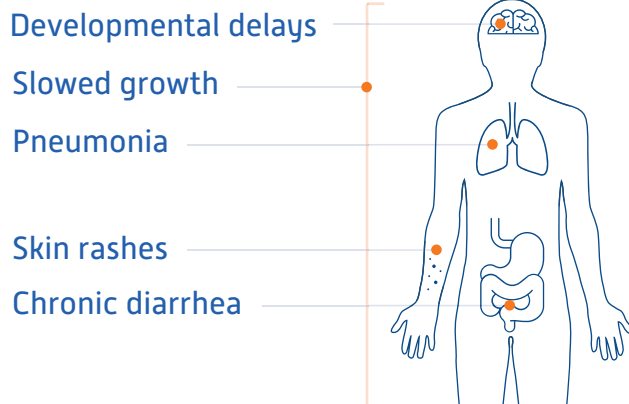
ADA is an enzyme produced in all cells and is most active in Lymphocytes, a type of white blood cell that is a key part of the body's immune system.

ADA-SCID results from mutations (changes) in the ADA gene, which reduce or eliminate the enzyme's protective activity and increase the risk of severe and recurring infections.



## Signs and Symptoms<sup>1</sup>

The most common symptoms of ADA-SCID:



## Incidence and Prevalence<sup>2</sup>

- Affects approximately **one** in **200,000** to **one** in **1 million** newborns worldwide
- Responsible for approximately **15%** of SCID cases
- Typically diagnosed within **6 months** of birth
- Most babies with ADA-SCID die by the age of **2** unless they are diagnosed early and effective treatment is started

## Genetic Testing for SCID<sup>1,3</sup>

SCID can be caused by mutations in at least **15 different** genes.



Genetic testing involves examining your DNA, the chemical database that carries instructions for your body's functions. Genetic testing can reveal changes (mutations) in your genes that may cause illness or disease.

Today, all 50 states, the District of Columbia, and Puerto Rico are currently screening or committed to newborn screening for SCID within the next few years.

## Treatment Options<sup>4</sup>

Patients should always consult a physician to see which therapy is best for them.



**Hematopoietic stem cell transplant (HSCT)**  
replaces defective immune cells with healthy immune cells from a donor



**Enzyme replacement therapy (ERT)**  
replaces the missing ADA enzyme and allows the immune system to function properly



**Gene therapy**  
replaces mutated genes with properly functioning ones, potentially restoring levels of ADA. There are currently no gene therapies approved for the treatment of ADA-SCID in the U.S.

## About Lediant Biosciences, Inc.

Lediant Biosciences, Inc., a wholly-owned subsidiary of **Lediant Biosciences S.p.A.**, is a research-based pharmaceutical company that dedicates considerable scientific and financial resources to the research, development, and distribution of novel and effective therapies to address the needs of people living with rare diseases and improve their quality of life. For additional information, please visit **Lediant.com**.

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