Understanding
ADA-SCID

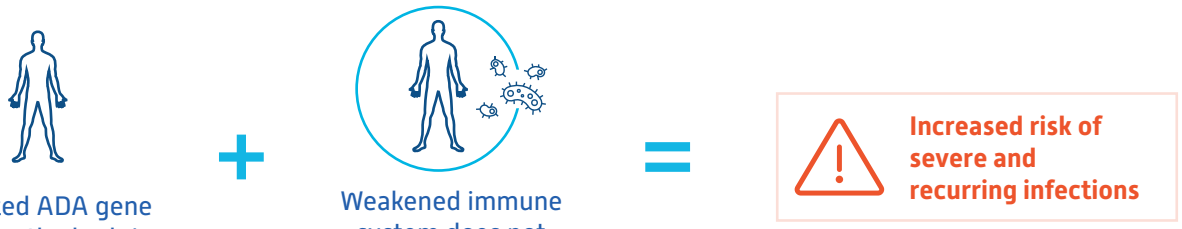
Leadiant Biosciences

What is ADA-SCID?[1]

Adenosine deaminase severe combined immune deficiency (ADA-SCID) is an ultra-rare, inherited genetic disorder, caused by a deficiency in the adenosine deaminase (ADA) enzyme, that is fatal when left untreated.

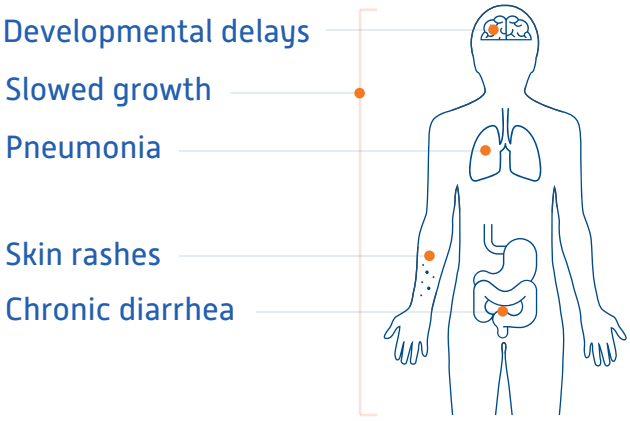
ADA is an enzyme produced in all cells and is most active in lymphocytes, a type of white blood cell that is a key part of the body's immune system.

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[1]

The most common symptoms of ADA-SCID:



Incidence and Prevalence[2]

- Affects approximately **1** in **200,000** to **1** in **1,000,000** newborns around the world
- Responsible for approximately **15%** of SCID cases
- Typically diagnosed within the **first few months** of life
- 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[1,3]

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[4]

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 Leadiant Biosciences, Inc.

Leadiant Biosciences, Inc., a wholly-owned subsidiary of **Leadiant 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 **Leadiant.com**.

References:

1. Adenosine deaminase deficiency. *Genetics Home Reference*; 2016. Available at: https://ghr.nlm.nih.gov/condition/adenosine-deaminase-deficiency. Accessed December 7, 2017.
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4. Booth C, Hershfield M, Notarangelo L, et al. Management options for adenosine deaminase deficiency; proceedings of the EBMT satellite workshop (Hamburg, March 2006). *Clin Immunol*. 2007;123:139-147.