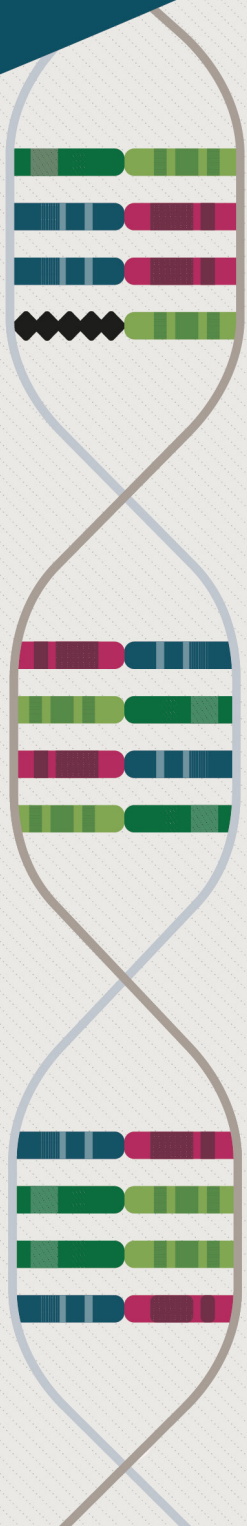
Orchard therapeutics™

What is ADA-SCID?

Adenosine deaminase severe combined immunodeficiency

A rare and life-threatening inherited immune disorder

What causes ADA-SCID?



ADA-SCID is caused by a change, or mutation in the ADA gene. This leads to absent or very low levels of the ADA enzyme such that the immune system is severely compromised or completely lacking.

When there is not enough of the ADA enzyme, the white blood cells responsible for fighting infections are missing, or do not work properly.

How is ADA-SCID inherited?

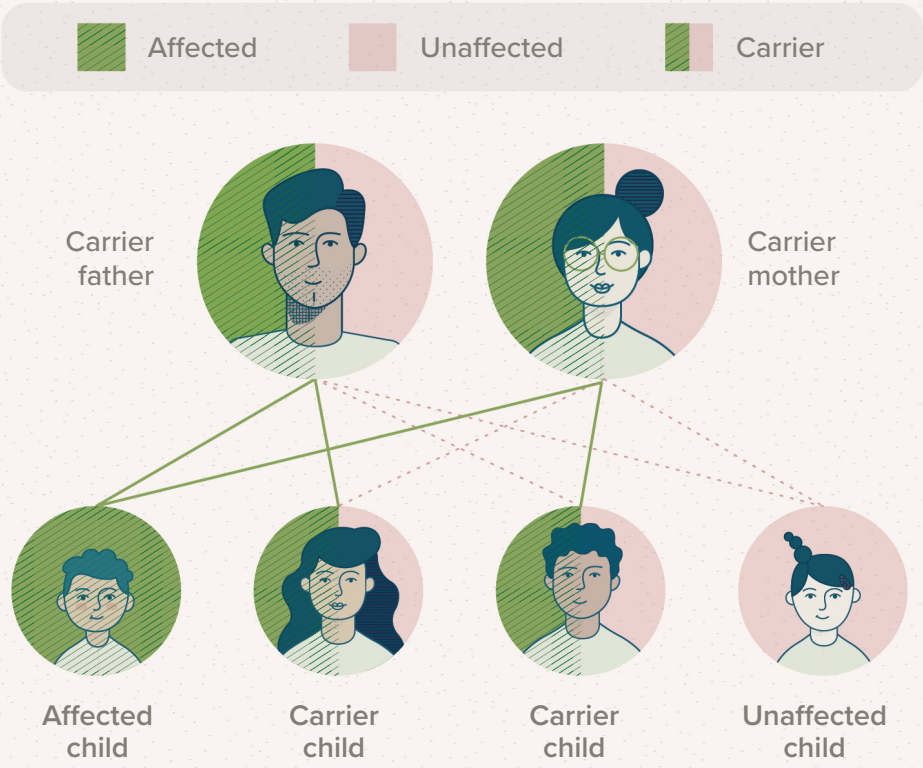
Approximately

1 in 500.000

babies born will have ADA-SCID

Autosomal recessive inheritance

ADA-SCID is an autosomal recessive condition. This means that a child must inherit the mutated gene from both parents to have the condition.
ADA-SCID affects both boys and girls.

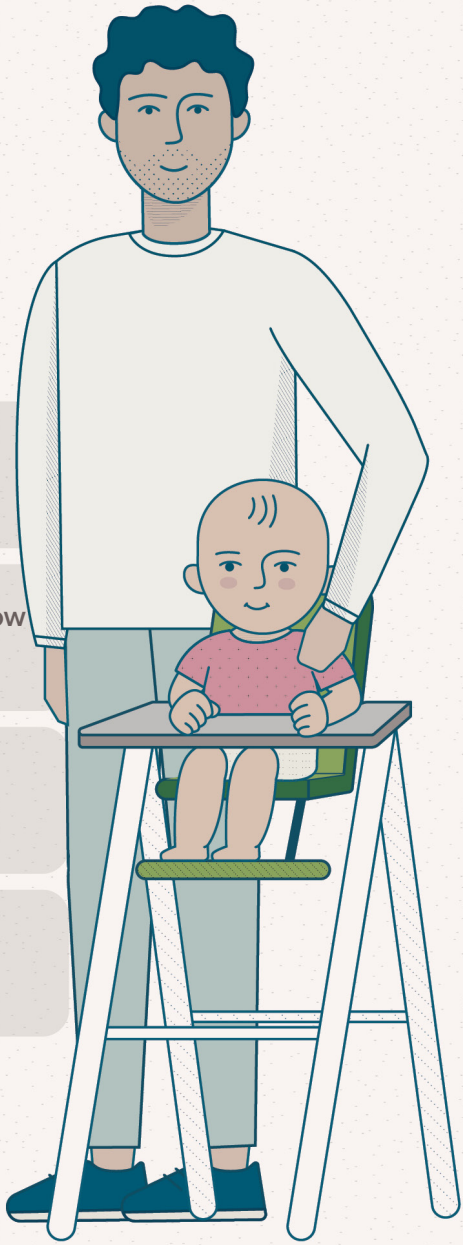


How does ADA-SCID affect the body?

Children with ADA-SCID are at risk of serious, repeated infections. The ADA enzyme is found throughout the body and a shortage may cause other problems.

Symptoms may include:

- Developmental delay
- Serious infections
- Skeletal problems
- Slower to grow and thrive
- Skin inflammation
- Frequent diarrhea
- Recurrent lung infections
- Risk of deafness



For more information about ADA-SCID and its management, please consult your healthcare provider.

Useful terms

Adenosine deaminase (ADA)
An enzyme essential for the development and functioning of the immune system.

Mutation
A change in the structure of a gene or group of genes. Such changes can be passed on from parent to child. Many mutations cause no harm, but others can cause genetic disorders, such as ADA-SCID.

Genes
Small sections of DNA that contain the instructions for individual characteristics, like eye and hair color, and how to make proteins, the functional building blocks of the cell. Proteins are responsible for making sure that the cells in the body function properly.

Enzyme
A type of protein produced by the body's cells that increases the rate of chemical reactions, enabling the body to build up, or break down substances that are necessary for life and normal functioning.

Immune disorder
A dysfunction of the immune system.

Immune system
Defends the body against foreign invaders, such as bacteria, viruses and fungi.

Infection
The invasion and multiplication of micro-organisms, such as bacteria, viruses and fungi, which may cause symptoms or harmful effects within the body.

White blood cells (lymphocytes/leukocytes)
A type of blood cell that plays an important role in the immune system's response to infection. White blood cells are formed in the bone marrow.

References

• Orphanet. SCID due to ADA deficiency.; Whitmore KV, Gaspar HB. Front Immunol. 2016;7:314.

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