

# MORQUIO A QUESTIONS AND ANSWERS



## What is Morquio A?

Morquio A is a rare and progressive inherited disease that affects major organ systems in the body. It is a form of mucopolysaccharidosis (MPS), a lysosomal storage disorder.

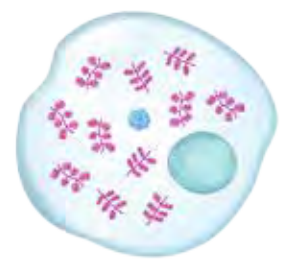
People with Morquio A do not make enough of a specific enzyme, called N-acetylgalactosamine-6 sulfatase, or GALNS (gal-en-es), which breaks down and recycles cellular waste called glycosaminoglycans, or GAGs (gags).

Healthy Cell



GALNS breaks down and recycles GAGs.<sup>2</sup>

Morquio A Cell



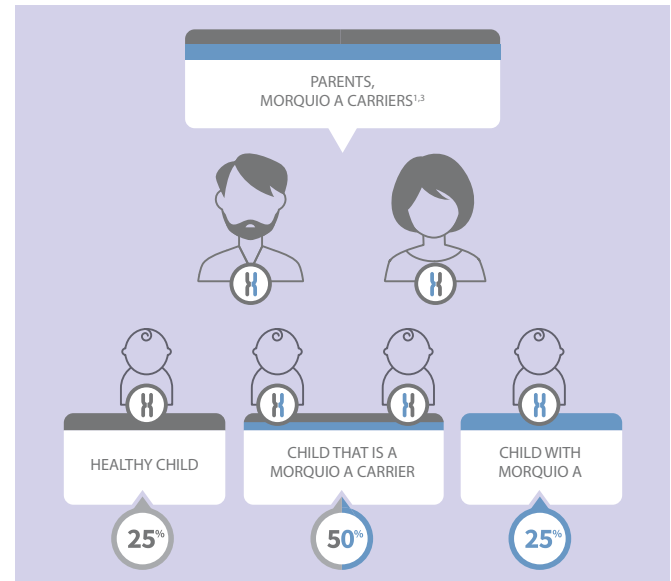
Without enough GALNS, GAGs build up in cells throughout the body.<sup>2</sup>



## What causes Morquio A?

Morquio A is a recessively inherited condition. This means that both parents must have a genetic mutation in the GALNS gene to pass Morquio A onto their children.

When both parents who are carriers have children, each pregnancy presents a 25% chance (1 in 4) of passing Morquio A on to that child.



People with Morquio A experience a variety of signs and symptoms as GAGs build up throughout the body.

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## What are the signs and symptoms of Morquio A?

Morquio A is heterogeneous (het-er-o-ge-ne-ous), which means that the signs, symptoms, and severity are different for each person. While Morquio A affects each person differently, it is common to have complications such as oddly shaped bones, curved spine, knock knees, and irregular chest growth.

## How does Morquio A affect people's lives?

It's common for people with Morquio A to see many different doctors and have surgeries and therapies that may cause them to miss school or work on occasion. Furthermore, people with Morquio A may experience hearing loss and may need hearing aids in one or both ears, and if they have blurred vision, glasses may be required. These symptoms can make it hard for people with Morquio A to participate in some activities. It's important to note that Morquio A does not affect a person's ability to think and they can go to school or work like anyone else.

## How many people have it?

Morquio A is considered a rare inherited disease. It is estimated that Morquio A occurs in 1 in 200,000 to 450,000 newborns.

People with Morquio A may be shorter, but they face big challenges every day. With the support of understanding friends, family, and healthcare professionals, people with Morquio A can lead fulfilling lives.



Learn more about Morquio A at [morquiosity.com](https://morquiosity.com)