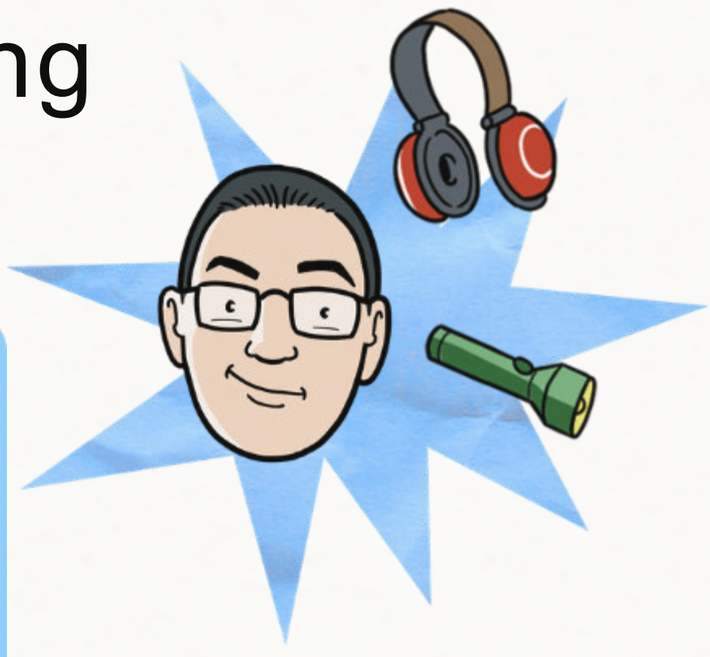
Mucolipidosis Type III(For Young People)



The character of Harry has Mucolipidosis Type III. It is commonly known as ML3. In medical terms, it is also referred to as Pseudo Hurler's Polydystrophy. ML3 is a degenerative condition which means the symptoms get worse as the child grows older. It's a rare condition which not many people have heard of.

What is it?: In everyday life, our bodies are constantly recycling. Old or damaged materials are broken down and new ones are built to replace them. This recycling happens inside our cells, in a part called the lysosome. To do this job, lysosomes need special chemical tools called enzymes. These enzymes have to be delivered to the lysosome, and to get there they need a kind of "address label" or signal.

In children with ML III,this signal doesn't get attached. Because of that, the enzymes never reach the lysosome and instead leave the cell. Without these enzymes, waste materials aren't broken down and start to build up inside cells.
Babies may not show many signs at first, but over time the buildup of waste damages more and more cells, and symptoms begin to appear.

Symptoms: ML III affects many different parts of the body. People with ML III may have different severity of symptoms or may not have all of the symptoms of the condition. Below is a really broad overview of what can be affected:

- Height & appearance
- Ears & eyes
- Brain
- Heart, lungs, abdomen and spleen
- Hands
- Bones and joints

If you'd like to find out more about how people are impacted, please visit the MPS Society website.

How is ML III treated?:
At the moment, there is no cure for the disease,but treatments are available to help manage the symptoms.

Did you know?
ML III is very rare it is estimated to occur in about 1 in 100,000 to 400,000 people worldwide. During a 10 year period (1989 to 1999) 5 babies were born with ML III in the UK.

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Useful to know: Living with a rare disease as a young person can be a mix of tough, confusing, and validating moments. It's not one single experience but made up of a lot of different ones. If you have a friend with a rare disease, you may have heard them deal with these before:

- Having to explain themselves - Because their condition is rare, they may have to explain it to a lot of people, a lot of times. There may be people who need to know e.g. teachers, doctors, family members. But there may also be people who ask to be nosy. It can be exhausting and difficult for them to know how much they need to share and to who.
- Growing up fast - Your friend may have had to be involved in some complicated conversations from a young age. They have to learn about their condition and often advocate for themselves.
- Living with uncertainty- Disabilities and health condition can be unpredictable. Treatments and symptoms aren't often straightforward. Your friend may sometimes feel anxious or frustrated.
- Feeling different - This can be difficult at any age. But it can be even harder for teenagers. People with rare conditions may not always be able to take part in activities others take for granted.

Supporting your friend: Your friend will appreciate space where they can be themselves.

- Listen first - Rare diseases can be different on a day to day basis. Let them tell you how things are instead of assuming.
- Be flexible - Your friend will really want to keep the plans they make with you. But sometimes that won't be possible whether that's because they don't feel well enough, or that they have a last minute appointment. Being flexible can be really helpful.
- Believe them - We can't always see when someone is in pain. Even if we can't see it, your friend might be struggling. If they let you know, believe them. Being told you look fine when you don't feel it can feel isolating.

Sources:
https://globalgenes.org/blog/navigating-emotions-a-guide-for-teenagers-with-rare-diseases/
https://mpssociety.org.uk/conditions/related-conditions/mucolipidosis-type-3#treatment-options-for-people-with-the-condition