



## **General Resources about Leukodystrophies**

<https://theglia.org/>

<https://www.huntershope.org/>

<https://ulf.org/>

## **What is Tay Sachs Disease?**

"Tay-Sachs is a genetic disorder caused by the absence of beta-hexosaminidase (HexA). This missing enzyme causes cells to become damaged, resulting in progressive neurological disorders. A cure for Tay-Sachs does not yet exist but there are many strategies for managing life with Tay-Sachs." (From the [www.ntsad.org](http://www.ntsad.org) website)

<https://www.ntsad.org/>

## **What is Vanishing White Matter Disease?**

"Vanishing White Matter Disease (VWM) is a genetic disorder that affects the nervous system and causes neurologic symptoms. Although initially recognized as a disease of young children, it is now known that the disease has a highly variable course with a wide range in severity. A striking feature of VWM is that in addition to a generally slow progression of symptoms, patients may show episodes of rapid neurological deterioration in response to certain stressors, such as infection or minor head trauma. The patient may partially recover following these episodes, but in severe cases the episode may even lead to coma and death."

(from the [www.ulf.org](http://www.ulf.org) website)

<https://ulf.org/leukodystrophies/vanishing-white-matter-disease/>

## **What is Canavan Disease?**

"Canavan disease is a progressive, fatal neurological disorder that begins in infancy. It is caused by an inherited genetic abnormality: the lack of an essential enzyme causes deterioration of the white matter (myelin) in the brain, thereby preventing the proper transmission of nerve signals." (from the [www.canavanfoundation.org](http://www.canavanfoundation.org) website)

<https://www.canavanfoundation.org/>

## **What is Adrenoleukodystrophy (ALD)?**

"Adrenoleukodystrophy (ALD) is an X-linked disease that is caused by an underlying genetic mutation in the ABCD1 gene, which affects the body's ability to create the protein that helps the process of breaking down very long-chain fatty acids (VLCFAs). The saturated VLCFAs build up in the brain, nervous system, and adrenal gland and eventually destroy the myelin sheath that surrounds the nerves. ALD affects approximately 1 in 17,000 people worldwide."

(from the [www.aldconnect](http://www.aldconnect) website)

<https://aldconnect.org/>

<https://www.aldalliance.org/>

<https://www.arrivederciald.org/>