

Leukodystrophies are a group of rare, progressive, metabolic, genetic diseases that affect the brain, spinal cord and often the peripheral nerves. Each type of leukodystrophy is caused by a specific gene abnormality that leads to abnormal development or destruction of the white matter (myelin sheath) of the brain. The myelin sheath is the protective covering of the nerve and nerves can't function normally without it. Each type of leukodystrophy affects a different part of the myelin sheath, leading to a range of neurological problems. The leukodystrophies can affect either adults or children, but are more common in children. Some types of leukodystrophy affect males and females equally but other types predominantly affect males. Treatment of most leukodystrophies is symptomatic and supportive. Medications and physical therapy may be helpful for spasticity and motor difficulties. Anti-epileptic medications should be provided for seizures and burning paresthesia from peripheral neuropathy may respond to medications for neuropathic pain. Please review the NORD report on the specific type of leukodystrophy for information about successful therapies. Genetic counseling is beneficial for affected individuals and their families.