Leukodystrophy

Leukodystrophy, also called “the leukodystrophies,” refers to a group of genetic disorders that are characterized by the imperfect development or maintenance of the white matter (myelin sheath covering nerve fibers in the brain). The myelin sheath is an extremely complex fatty substance made of many components. Each of the leukodystrophies affects one of these components. Specific leukodystrophies include metachromatic leukodystrophy, Krabbe leukodystrophy, adrenoleukodystrophy, Pelizaeus-Merzbacher disease, Canavan disease, Alexander disease, Zellweger syndrome, Refsum disease, and cerebrotendinous xanthomatosis. A number of novel leukodystrophies have recently been described. Among those, CACH/VWM syndrome is the most common. The most common signs seen in most leukodystrophies include gradual changes in an infant or child who previously appeared well. Changes may appear in body tone, movements, gait, speech, ability to eat, vision, hearing, behavior, memory, or thought processes. The symptoms (problems), which vary according to the specific type of leukodystrophy, may be difficult to recognize in the early stages of the disease.

TREATMENT

Treatment for most of the leukodystrophies is symptomatic and supportive, and may include medications, physical, occupational and speech therapies, and nutritional, educational, and recreational programs. Bone marrow transplantation is showing promise for a few of the leukodystrophies.

WHAT WILL HAPPEN

The prognosis (likely outcome) for the leukodystrophies varies according to the specific type of leukodystrophy.

RESEARCH BEING DONE

NINDS supports research on genetic disorders including the leukodystrophies. The goals of this research are to increase scientific understanding of these disorders, and to find ways to prevent, treat, and, ultimately, cure them.

Research is continually being done to learn more about this problem.

Provided by: The National Institute of Neurological Disorders and Stroke

Document Released: 12/08/2003

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