US Natural History Study of MLD

A Natural History Study of the rare lysosomal disease metachromatic leukodystrophy (MLD) will answer the question; how the rare disease develops as it does and provides the basis for correlation of endpoints to the planned US clinical trial.

Patients are being recruited for this Natural History Study of the disease. The study, which includes 10 patients with late-infantile MLD, has been approved by the Internal Review Board at University of North Carolina, Chapel Hill, North Carolina, USA and is being performed by Dr. Maria Escolar at the Center for the Study of Development and Learning and FPG Child Development Institute, University of North Carolina, Chapel Hill, North Carolina, USA, with support from the Danish biotech company Zymenex.

Supplemental information
Metachromatic Leukodystrophy (MLD), is one of 45 diseases within the family of Lysosomal Storage Diseases.
MLD is caused by an increased concentration of sulphatide in cells and an ensuing breakdown of "myelin", a substance that protects the nerves in the brain and the rest of the body. The disease occurs due to a lack of the enzyme Arylsulfatase A (ASA), which causes irreparable neurological damage. The disease is lethal and no therapy exists today. Children with late-infantile MLD are often diagnosed at the age of two years and are quickly bound to a wheelchair and become bedridden until they die within three to four years. The disease is rare and therefore unknown to the general public. The disease can in some ways be compared to Multiple Sclerosis, which also exists in several forms and can have a very quick and lethal progression.

Zymenex A/S has developed Metazym (recombinant human ASA enzyme). The company is a Scandinavian biopharmaceutical company, founded in 1998, with headquarters in Hillerød north of Copenhagen, Denmark and research laboratories in Stockholm, Sweden. The company is focused on research and development of pharmaceutical products for the treatment of rare, genetic diseases, for which there is no treatment today and which, due to the small patient populations, fall within "Orphan Diseases" and the Orphan Drug Acts. Zymenex is supported financially by the Danish venture capital investors BankInvest and Sunstone Capital and has received gifts from The British Trust for The Myelin Project, the MLD Foundation (USA) and the Athena Hope Foundation.

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