

About Lysosomal Storage Disorders

LSDs are a group of more than 40 disorders caused by inborn errors of metabolism (which are problems in the genes that affect how cells break down certain molecules).

People with LSDs are either lacking or in short supply of particular enzymes that are found in the lysosome (a special compartment of the cell). Because of this, molecules that are meant to be broken down by the missing or deficient enzymes build up within the lysosome and can prevent the cell from working properly.

Separately, lysosomal storage diseases are each rare diseases. As a group, lysosomal storage diseases are estimated to affect 1 in 7,700 live births.

Living with a lysosomal storage disease means living with a rare disease that is sometimes not well understood

Symptoms vary widely across the different LSDs, some of which may cause relatively minor problems, while others can be or become very serious and life threatening. Once symptoms have appeared they are usually progressive, unless disease-specific treatment is available and instituted. Medical management options for LSDs often cross several medical specialties. Some LSDs have disease-specific treatment options to consider. Be sure to speak with your doctor about the best medical care options for you or your loved ones.

Learn more about lysosomal storage disorders at www.lysosomallearning.com

Patient organizations are also a great resource for learning about lysosomal storage diseases. You can find a list of them at www.expressionofhope.com

For further information please call Genzyme at:
U.S. 800-745-4447 or 617-768-9000, Europe 31-35-699-1499
Other areas, please contact your local Genzyme office.

