How can I join the LAL Deficiency registry?

Joining the registry is simple. Just let your doctor know that you'd like to join, and he or she will ask you to sign a consent form. If you are signing up your child, you will sign the form on his or her behalf.

Your doctor will deliver the necessary records to the registry, always maintaining your privacy. No other procedures or tests are needed.

You may choose to leave the registry at any time without penalty and without changing your future medical care. If you wish to leave the registry, just let your doctor know.

Join the LAL Deficiency registry to help other people with LAL-D.

To learn more, visit www.laldeficiencyregistry.com.

ALEXION®

FOR PATIENTS

The LAL Deficiency registry:

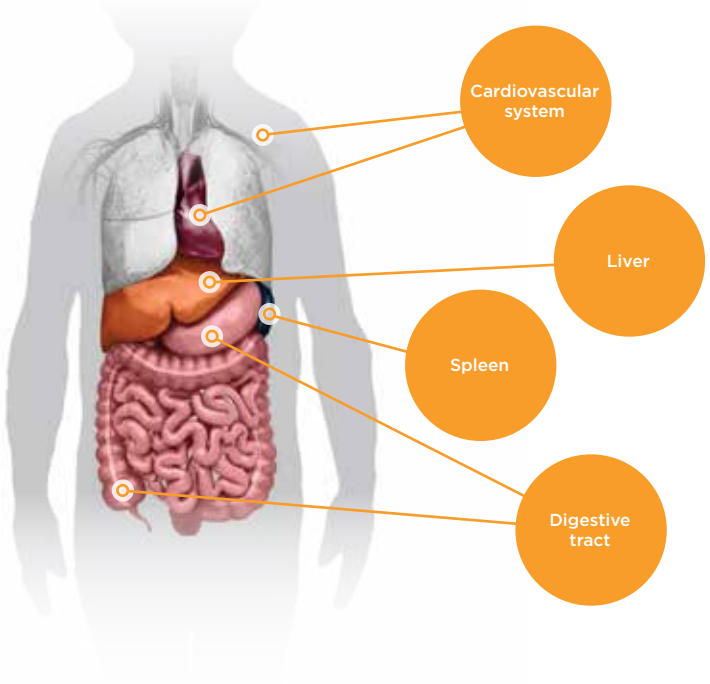
helping us learn more about Lysosomal Acid Lipase Deficiency (LAL-D)

Use this booklet to discover how you can help advance the knowledge of LAL-D

LAL-D registry

What is LAL-D?

LAL-D is a rare, genetic, progressive condition that can be diagnosed in infants, children, and adults. Without treatment, infants with rapidly progressive LAL-D may face serious complications within the first year of life. In children and adults, LAL-D can lead to serious health problems that can occur at any time.



People born with LAL-D are not able to break down fatty materials (cholesteryl esters and triglycerides) because their bodies do not make enough of an enzyme called lysosomal acid lipase (LAL).

People with LAL-D have very little or no LAL enzyme in their body. When the body does not have enough LAL, fatty material can build up in the liver, heart, and other organs, and cause problems. LAL-D is an inherited disease, so if you are diagnosed with LAL-D, there is a chance that other members of your family have it too.

LAL-D is an inherited disease that affects infants, children, and adults.

What is the LAL Deficiency registry, and why is it important to join?

Joining the LAL Deficiency registry helps doctors learn more about LAL-D

Because LAL-D is a rare disease, there is limited awareness and understanding of its signs and symptoms. The LAL Deficiency registry helps doctors to better understand the disease and discover ways to advance treatment. Anyone diagnosed with LAL-D may join anonymously, and a child with LAL-D can join with a parent or guardian's permission.

Anonymous information about your LAL-D helps doctors advance knowledge

Your privacy will always be first priority. The registry does not collect information that identifies you, such as your name. When you let your doctor know you would like to join the registry, your record will be given a unique ID number. Then, information about your LAL-D symptoms and care—along with everybody else's—will be made available in the registry.

Only your doctor will know which data belong to you. Your doctor may also use your data to make reports for you, helping to improve your personal LAL-D management.

The goal of the LAL Deficiency registry is to collect information from people with LAL-D around the world to better understand and treat this disease.

LAL-D registry