ALEXION®
AstraZeneca Rare Disease

Lysosomal Acid Lipase Deficiency (LAL-D) and Your Health

This guide will help you

- Track the signs and symptoms of LAL-D
- Talk about your lab tests with your doctor

This information is intended as educational information for patients. It does not replace a doctor's judgment or clinical diagnosis. Speak with your doctor about your medical condition and any specific symptoms that you may be experiencing.

WHAT IS LAL-D?

LAL-D is a rare, progressive, genetic condition in which infants, children, and adults have an uncontrolled buildup of fatty material (cholesteryl esters and triglycerides) in their liver, intestines, blood vessel walls, and other tissues. This buildup can cause continuous damage that may affect the function of many organs throughout your body.

WHAT ARE THE RISKS OF HAVING LAL-D?

You're probably already aware that LAL-D is a serious, chronic, potentially life-threatening disease that can damage vital organs such as your liver, spleen, stomach and intestines, and cardiovascular system.

When seen in infants, LAL-D is a medical emergency and rapidly progressive, resulting in severe complications within the first year of life. When seen in children and adults, LAL-D can lead to serious health problems that can occur at any time without warning.

Most people with LAL-D experience complications in more than one organ system. However, no single sign or symptom can give a complete picture of your LAL-D.

TALK TO YOUR DOCTOR ABOUT

- Your symptoms of LAL-D (old, new, or different from your last visit)
- The frequency and importance of regular doctor's visits
- Different ways to help manage your LAL-D

Keeping track of how LAL-D is affecting your body and writing down what happens over time may help you and your doctor manage your LAL-D.

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TRACKING THE SIGNS AND SYMPTOMS OF LAL-D FOR YOUR NEXT DOCTOR'S VISIT

Here you will find assessment forms for recording your signs and symptoms of LAL-D. In addition to tracking your signs and symptoms, you may record your lab test results on the following pages to get a better picture of your LAL-D. By sharing this information, you and your doctor can better understand how well your LAL-D is being managed.

Contact your doctor or get medical help right away if you are experiencing a medical emergency.

Signs or Symptoms	Do You Have the Sign or Symptom Now?		NOTES When Did the Sign or Symptom Start?/ How Long?/How Often?
Distended abdomen (large belly size)	Yes	No	
Stomach pain and/or cramps	Yes	No	
Nausea	Yes	No	
Frequent vomiting	Yes	No	
Frequent diarrhea	Yes	No	
Easy bruising	Yes	No	
Poor growth (failure to thrive) (for infants only)	Yes	No	
Yellow discoloration of skin or white of the eyes	Yes	No	
Other	Yes	No	

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TRACKING YOUR LABS WITH YOUR DOCTOR

Please note that not everyone diagnosed or awaiting diagnosis of LAL-D will undergo all of these tests. Check with your doctor about what lab tests, scans, or procedures are right for you and any other important test results that may not be listed here.

Potential Labs and What They Evaluate	Lab Tests, Scans, or Procedures	Results (Low/Normal/High)	Date
Lipid Panel			
Fat in blood (including "bad cholesterol" and "good cholesterol," which assess risk of heart disease)	Low-density lipoprotein cholesterol (LDL-c)		
	High-density lipoprotein cholesterol (HDL-c)		
	Triglycerides (TGs)		
	Total cholesterol (TC)		
Complete Blood Count			
Infections, inflammation	White blood cells (WBCs)		
Bleeding, bruising, anemia, tiredness	Red blood cells (RBCs)		
	Hematocrit		
	Hemoglobin		
Blood clotting	Platelet count		
Comprehensive Metabolic Panel			
Proteins	Total protein		
	Albumin		
Liver function	Alanine aminotransferase (ALT)		
	Aspartate aminotransferase (AST)		
	Bilirubin		

Discuss any important test results with your healthcare team and ask to understand what these test results mean.

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Other Potential Labs and Evaluations	Lab Tests, Scans, or Procedures	Results (Low/Normal/High)	Date
Growth (for infants and children)	Height-for-age percentile		
	Weight-for-age percentile		
Bleeding problems	Prothrombin time/international normalized ratio (PT/INR), partial thromboplastin time (PTT)		
Biomarker (a marker found in your blood that indicates activation of certain immune cells)	Chitotriosidase (ChT, a macrophage inflammatory marker)		
Fat in liver (hepatic steatosis)	Ultrasound, magnetic resonance imaging (MRI), imaging technology for fibrosis, or biopsy		
Type of fat in liver (microvesicular or mixed hepatic steatosis)	Liver biopsy		
Scarring/damage in liver (fibrosis & cirrhosis)	Computerized tomography (CT), MRI, or liver biopsy		
Enlarged liver (Hepatomegaly)	Blood tests, ultrasound, or CT		
Enlarged spleen (Splenomegaly)			
Hardening of the adrenal glands above the kidneys (Adrenal calcifications)	X-ray		
Cardiac function	Echocardiogram		
	Electrocardiogram (EKG or ECG)		
	Stress test		

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TAKE NOTES OF SIGNS AND SYMPTOMS BETWEEN VISITS

Be prepared for your next visit by writing down any changes you have seen or felt, and any questions you may have for your healthcare team in the lines below.

Notes

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Notes

It's very important to note all of your signs and symptoms, as well as your lab results in one place and show them to your doctor at your next visit.

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To learn more about LAL-D, visit **LALDSOURCE.COM**

Join the LAL-D registry to help physicians learn more about people with LAL-D.

To learn more, visit **LALDEFICIENCYREGISTRY.COM**.

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LAL-D registry

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