

Disease

Lysosomal acid lipase deficiency¹

Gene

LIPA

Inheritance	AR
Current Treatments	Sebelipase alfa enzyme replacement
Evidence for Treatment	guideline ²
Non-Molecular Testing Availability	yes
Non-Molecular Test	leukocytes lysosomal acid lipase enzyme activity ³
Disease Frequency (per 100,000)	0.49 ^{4, 5}
Genes Included in Disease Frequency	LIPA
Experimental / Investigational Therapies	https://clinicaltrials.gov/ct2/results?cond=Wolman+Disease&term=&cntry=&state=&city=&dist=

References

- ¹ <https://www.ncbi.nlm.nih.gov/books/NBK305870/#lal-def.Summary>
- ² <https://www.ncbi.nlm.nih.gov/pubmed/31767214>
- ³ <https://www.ncbi.nlm.nih.gov/books/NBK305870/>
- ⁴ <https://www.ncbi.nlm.nih.gov/pubmed/30315827>
- ⁵ <https://www.ncbi.nlm.nih.gov/pubmed/31180157>