

Disease

WAS associated disorder^{1, 2}

Gene

WAS

Inheritance	XL
Current Treatments	Waskyra (etuvetidigene autotemcel (OTL-103) - autologous CD34+ HSPC transduced with a lentiviral vector encoding WAS, granulocyte colony-stimulating factor (G-CSF), Bone marrow transplant (hematopoietic stem cell transplantation (HSCT)), Antibiotic prophylaxis, immunoglobulin replacement
Evidence for Treatment	expert opinion
Non-Molecular Testing Availability	yes
Non-Molecular Test	complete blood count ¹
ClinGen	https://actionability.clinicalgenome.org/ac/Pediatric/ui/stg2SummaryRpt?doc=AC011
Disease Frequency (per 100,000)	0.175 ^{1, 3}
Genes Included in Disease Frequency	WAS
Experimental / Investigational Therapies	https://clinicaltrials.gov/ct2/results?cond=Wiskott-Aldrich+Syndrome&term=&cntry=&state=&city=&dist=

References

- ¹ <https://www.ncbi.nlm.nih.gov/books/NBK1178/>
- ² <https://www.ncbi.nlm.nih.gov/pubmed/31750279>
- ³ <https://pubmed.ncbi.nlm.nih.gov/35075289/>