

Disease

Familial thrombotic thrombocytopenic purpura^{1, 2}

Gene

ADAMTS13

Inheritance	AR
Current Treatments	Plasma infusion or exchange. Adzynma (recombinant ADAMTS13)
Evidence for Treatment	guideline ³
Non-Molecular Testing Availability	yes
Non-Molecular Test	ADAMTS13 activity ⁴
Experimental / Investigational Therapies	https://clinicaltrials.gov/ct2/results?cond=ADAMTS13&term=&cntry=&state=&city=&dist=

References

¹ <https://pubmed.ncbi.nlm.nih.gov/31759790/>

² <https://ashpublications.org/blood/article/138/Supplement%201/4252/481609/Recombinant-ADAMTS13-for-Patients-with-Severe>

³ <http://content.guidelinecentral.com/guideline/get/pdf/3712>

⁴ <https://pubmed.ncbi.nlm.nih.gov/33072671/>