

Disease

Mucopolysaccharidosis type IIIB¹

Gene

NAGLU

Inheritance	AR
Current Treatments	Tralesinidase alfa (BMN 250) enzyme replacement
Evidence for Treatment	expert opinion
Non-Molecular Testing Availability	yes
Non-Molecular Test	serum or plasma N-acetyl-alpha-D-glucosaminidase enzyme activity ²
Disease Frequency (per 100,000)	1 ^{3, 4}
Genes Included in Disease Frequency	NAGLU
Experimental / Investigational Therapies	https://clinicaltrials.gov/ct2/results?cond=Mucopolysaccharidosis+III&term=&cntry=&state=&city=&dist=

References

- ¹ <https://www.omim.org/entry/252920>
- ² <https://www.ncbi.nlm.nih.gov/books/NBK546574/>
- ³ <https://rarediseases.org/rare-diseases/mucopolysaccharidosis-type-iii/>
- ⁴ <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5891921/>