



Fabry disease (mRNA-3630)

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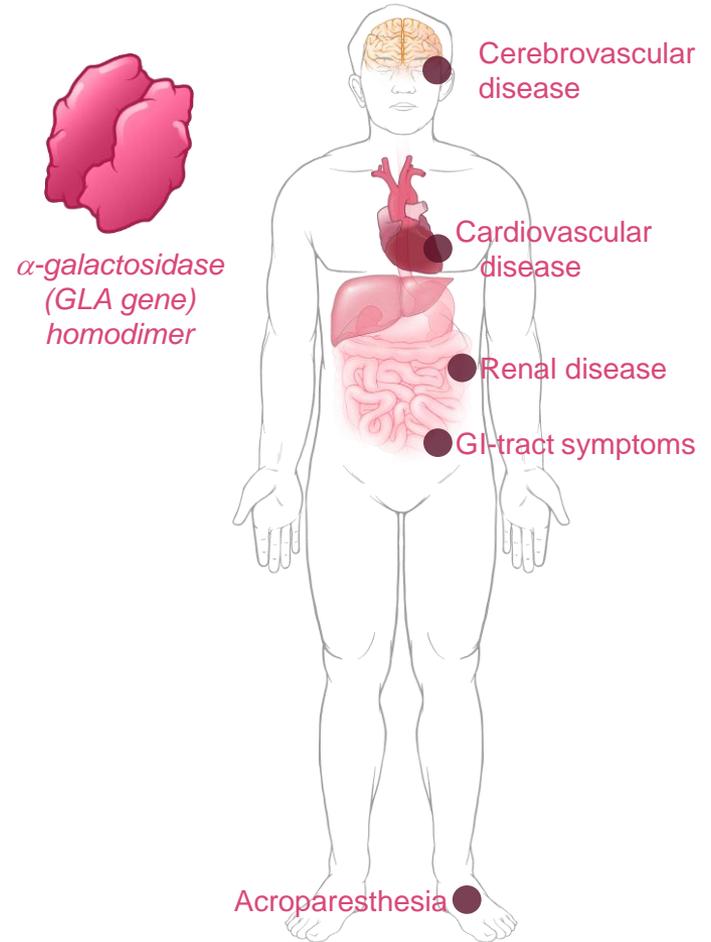
Modality	Program #	Program Indication		Preclinical development	Phase 1	Phase 2	Phase 3 and commercial	Moderna rights
 Systemic secreted therapeutics	mRNA-1944	Antibody against Chikungunya virus						Worldwide <i>DARPA funded</i>
	AZD7970	Relaxin <i>Heart failure</i>						50-50 U.S. profit sharing; AZ to pay royalties on ex-U.S. sales
	mRNA-3630	α -GAL <i>Fabry disease</i>						Worldwide

mRNA-3630 is in IND-enabling GLP toxicology studies

Fabry Disease (Fabry) overview

Progressive, multi-organ disease with unmet need

- Fabry disease is an X-linked hereditary defect in glycosphingolipid metabolism caused by mutations in the GLA gene, which encodes for the lysosomal protein alpha galactosidase
- **Disease burden:** Progressive, multiorgan, lysosomal storage disorder (LSD) resulting in cellular and tissue dysfunction
 - Impact to: vasculature, kidney, heart, GI, and neurological system
 - Childhood diagnosis; adult diagnosis post stroke or renal complications
- **Target population:** Annual incidence of ~1:80,000
- **Unmet need** despite approved treatments:
 - Recombinant ERT: Agalsidase beta (Sanofi/Genzyme); Agalsidase alpha (Shire/PLC)
 - Chaperone therapy: Migalastat (Amicus Therapeutics)



Moderna concept: IV-administered mRNA encoding α -GAL enzyme with native post-translational modifications to restore lysosomal enzyme activity for an extended duration

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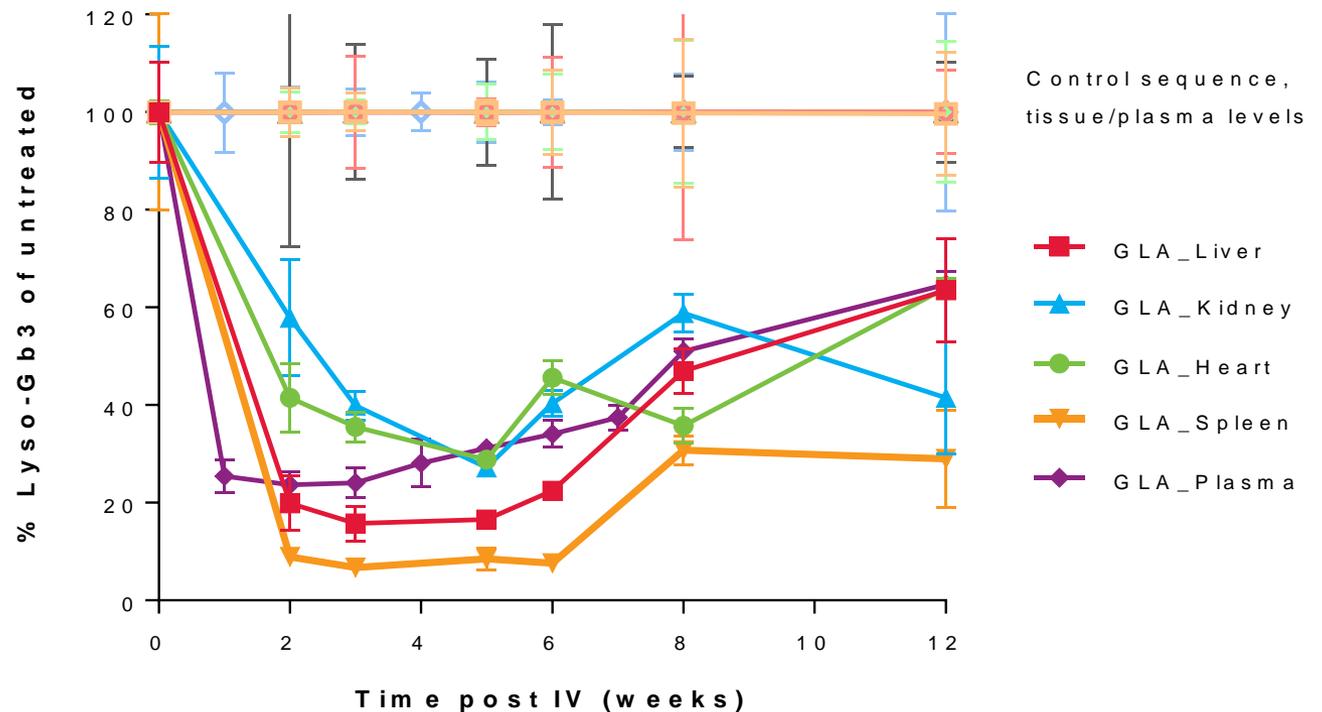
Pre-clinical data – sustained effect in multiple tissue types

Study Design:

Species:
Mouse

- Animals: GLA^{-/-}
- Dose: 0.5 mpk
- Dosing Schedule: Single
- Injection Route: IV
- Sample Size: 3

Reduction in Lyso-Gb3 in tissue with single administration of mRNA encoding α -GAL



With a single dose of our mRNA encoding for α -GAL we observed a sustained reduction in Lyso-Gb3 in pre-clinical studies

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