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Glycogen storage disease type 1a (GSD1a) (mRNA-3745)

Last program update: May 7, 2020

Modality	ID #	Program *Indication*	Preclinical development	Phase 1	Phase 2	Phase 3 and commercial	Moderna rights
Systemic intracellular therapeutics	mRNA-3704	MUT *Methylmalonic acidemia, MMA*					Worldwide
	mRNA-3927	PCCA/PCCB *Propionic acidemia, PA*					Worldwide
	mRNA-3283	PAH *Phenylketonuria, PKU*					Worldwide
	mRNA-3745	G6Pase *Glycogen storage disease type 1a, GSD1a*					Worldwide

mRNA-3745 nominated as fourth systemic intracellular therapeutics DC

Glycogen storage disease type 1a (GSD1a) overview

New intracellular therapeutics DC – mRNA-3745

- GSD1a is a rare inherited metabolic disease resulting from a deficiency in the metabolism of glucose, due to mutations within the enzyme glucose 6-phosphatase, G6Pase
- **Disease burden:** Life-threatening hypoglycemia, long-term liver & kidney damage
- **Target population:** Incidence of ~1:100k live births
  - —2,500 patients in US[1], and >4,000 patients in the EU[1]
- Standard of care:
  - —Strict diet control
  - —Frequent consumption of uncooked cornstarch to improve hypoglycemia

Glucose 6-phosphatase (G6Pase)

Moderna concept: IV-administered mRNA encoding G6Pase enzyme to restore deficient or defective intracellular enzyme activity

[1]Based on estimated incidence of 1:100,000

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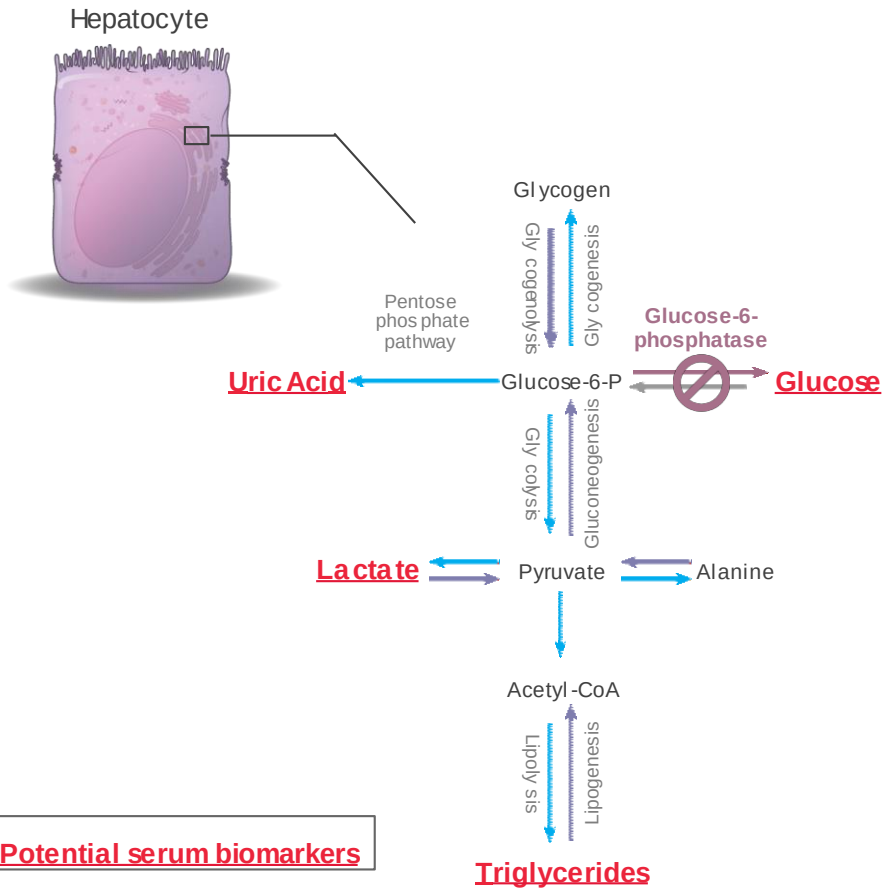
Glycogen storage disease type 1a (GSD1a) overview

Defect in G6PC gene disrupts glucose metabolism pathways



- Missing or defective G6Pase leads to:
  - —Hypoglycemia
  - —Lactic acidemia
  - —Hyperlipidemia
- Metabolite buildup associated with hepatomegaly, hepatocellular adenomas, and higher risk of liver cancer
- Restoring G6Pase activity should increase blood glucose levels, decrease uric acid, lactic acid, triglyceride levels

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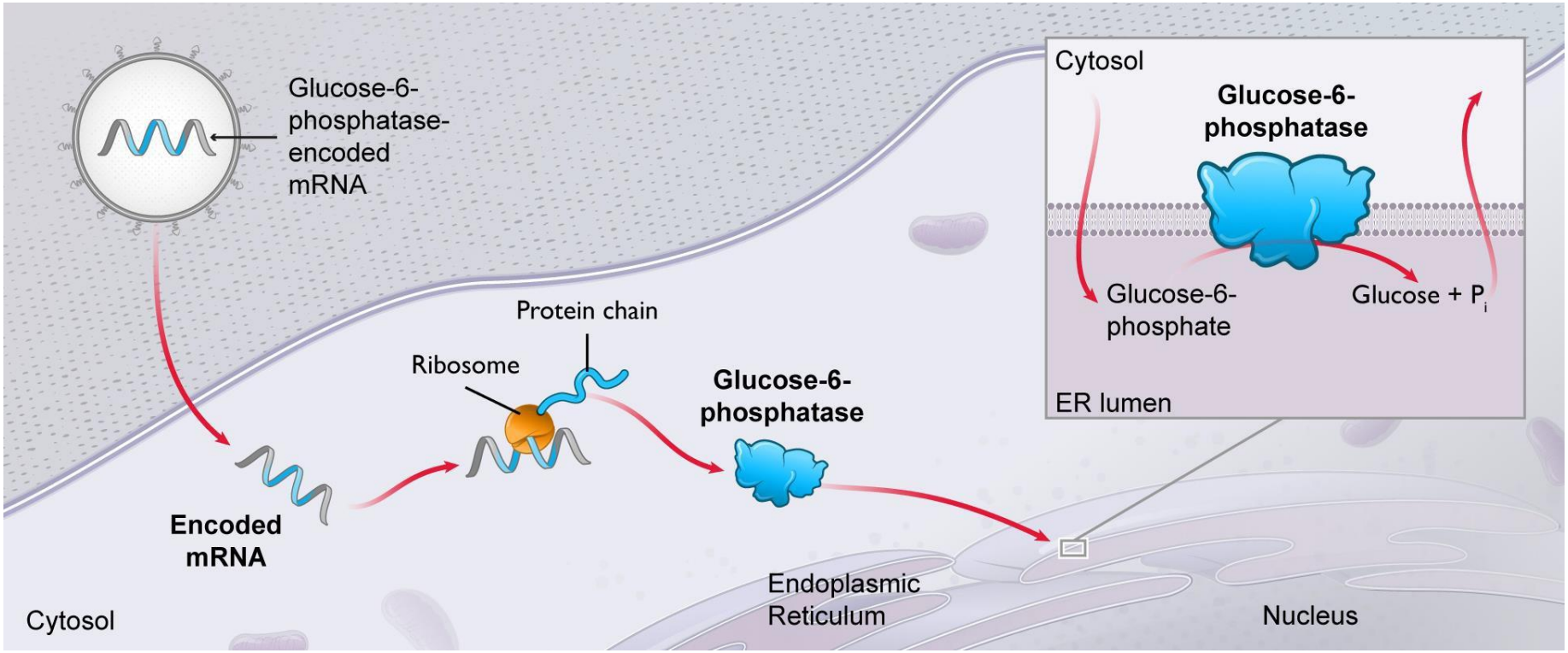
mRNA-encoded enzyme localized to endoplasmic reticulum



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Glycogen storage disease type 1a (GSD1a) (mRNA-3745)

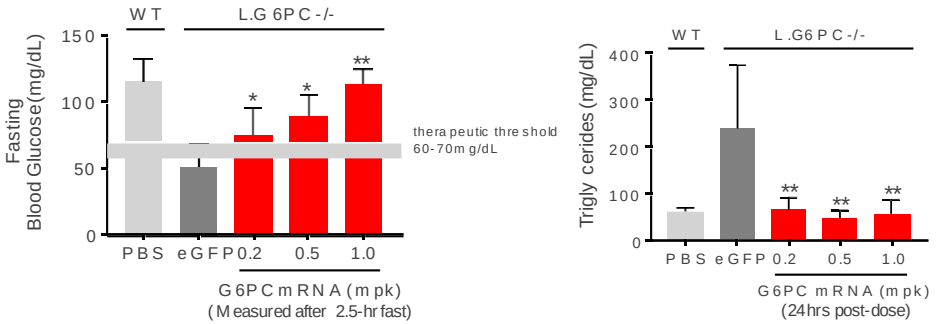
Preclinical data – restoration of enzymatic activity

Study Design:

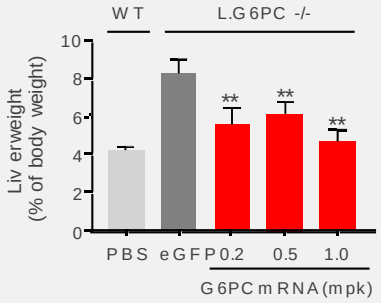
Species: **Mouse**

- Animals: Liver-Specific G6PC Knockout (L.G6PC -/-)
- Dose: 0.2, 0.5, 1.0 mpk
- Dosing Schedule: single dose
- Injection Route: IV
- Sample Size: 5-8

Serum biomarkers after single dose of G6Pase mRNA



Reduction in liver weight 24 hours after IV administration of G6Pase mRNA



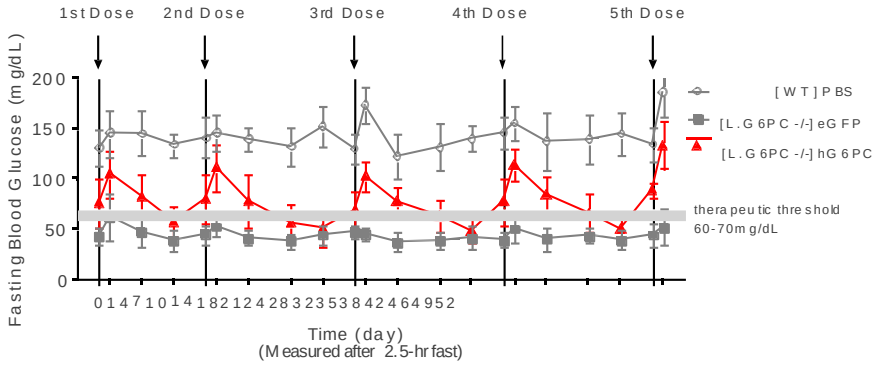
Notes: eGFP is a negative control. Asterisks based on one-way ANOVA of post-treatment vs. eGFP levels:
*p<0.05
**p<0.0001

Study Design:

Species: **Mouse**

- Animals: Liver-Specific G6PC Knockout (L.G6PC -/-)
- Dose: 0.25 mpk
- Dosing Schedule: approx. every other week
- Injection Route: IV
- Sample Size: 7-9

Restoration of blood glucose above therapeutic threshold with repeat dosing of G6Pase mRNA



We have demonstrated preclinical proof-of-concept for G6Pase mRNA therapy in in vivo studies

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