
Supplementary information

**Interim analyses of a first-in-human phase
1/2 mRNA trial for propionic acidaemia**

In the format provided by the
authors and unedited

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mRNA-3927 Therapy for Propionic Acidemia: Interim Analysis From the First-in-human, Open-label, Nonrandomized, Phase 1/2 PARAMOUNT Study

Koeberl et al

Supplementary Table 1. Protocol-required laboratory assessments.

Laboratory assessments	Parameters	Value exclusionary for study participation
Laboratory safety assessments		
Hematology	Platelet count	<75,000 mm ³
	Red blood cell count	Not exclusionary
	Hemoglobin	<9 g/dL
	Hematocrit	Not exclusionary
	RBC indices: mean corpuscular volume, mean corpuscular hemoglobin	Not exclusionary
	Percent reticulocytes	Not exclusionary
	White blood cell count	<2000 mm ³
	Neutrophil count	<1000 mm ³
	Neutrophil percent	Not exclusionary
	Lymphocyte count/percent	<800 mm ³
	Monocyte, eosinophil, basophil count/percent	Not exclusionary
Comprehensive metabolic panel ⁺ (CMP ⁺ , with amylase and lipase)	Blood urea nitrogen	Not exclusionary
	Potassium	>5.5-6.0 mmol/L or symptomatic and <3.0 mmol/L
	Carbon dioxide (bicarbonate)	Not exclusionary
	Sodium	<130 mmol/L and symptomatic or >150 mmol/L and intervention initiated
	Chloride	Not exclusionary
	Creatinine	Not exclusionary (estimated glomerular filtration rate is evaluated instead)
	Glucose (nonfasting)	Not exclusionary
	Anion gap	Not exclusionary
	Calcium	Corrected serum calcium <8.0 mg/dL and symptomatic, or >11.5 mg/dL and symptomatic
	Aspartate aminotransferase	>2.0 × ULN
	Alanine aminotransferase	>2.0 × ULN
	Total protein	Not exclusionary
	Albumin	<3.0 g/dL
	Globulin, albumin/globulin ratio	Not exclusionary
	Gamma-glutamyl transferase	Not exclusionary
	Total bilirubin	>1.5 × ULN
	Direct bilirubin	>1.5 × ULN
	Alkaline phosphatase	>2.0 × ULN

Laboratory assessments	Parameters	Value exclusionary for study participation
	Lactate dehydrogenase	>1.5 × ULN
	Amylase	>1.5 × ULN and symptomatic or >2.5 × ULN and asymptomatic
	Lipase	>1.5 × ULN and symptomatic or >2.0 × ULN and asymptomatic
	Uric acid	Not exclusionary
	C-reactive protein	Not exclusionary
	Phosphorus	Not exclusionary
	Bicarbonate	Not exclusionary
	Creatinine phosphokinase	Not exclusionary
	Total cholesterol	Not exclusionary
	Triglycerides	Not exclusionary
Coagulation	Prothrombin time	>1.2 × ULN
	Activated partial thromboplastin time	>1.5 × ULN
Other safety assessments		
Pregnancy test (performed at local laboratory)	β-hCG pregnancy test (as needed for women of childbearing potential)	Positive result

ULN, upper limit of normal.