

# THE BEAM-301 CLINICAL STUDY

## STRIVING FOR A NEW POTENTIAL THERAPY FOR GSD1a

A phase 1/2, multicenter, open-label, single-ascending dose study to evaluate the safety and efficacy profiles of BEAM-301 in adult patients who have GSD1a and have the G6PC c.247C>T (R83C) variant(s)



### PURPOSE

Evaluate the safety and tolerability of BEAM-301 in patients who have GSD1a and at least one copy of the R83C variant



### DESIGN

Adult patients in up to 4 single-ascending-dose cohorts, each with approximately 3 patients per dose level



### DURATION

~18 visits over 2 years with short hospital stays for controlled fasting challenges and dosing; with flexibility for some at-home visits.



### Treatment Schedule

Consent

#### Screening

Screening period that may last up to 4 weeks.

Enrolled

#### Run-In Period

A 4-week run-in period

#### Hospitalization for Study Drug infusion Day 1

A dosing period in which participants will receive a single intravenous infusion of BEAM-301.

#### Follow-Up

Approximately 2 years of follow-up

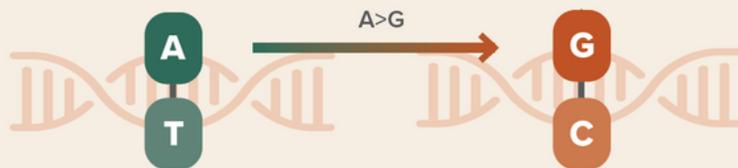


### BEAM-301 Mechanism of Action

BEAM-301 base editing to correct the R83C variant in the G6PC gene

NONFUNCTIONAL G6Pase

FUNCTIONAL G6Pase



G6PC gene with R83C mutation

Corrected G6PC gene

Nonviral lipid nanoparticle delivery of the base-editing components guide RNA and mRNA leads to the correction of the R83C variant via an A:T-to-G:C base pair substitution primarily in the liver, where glycogen is converted to glucose.



### Inclusion Criteria



Age  $\geq$  18 years



Diagnosis of GSD1a and at least one copy of the G6PC c.247C>T (R83C) variant that is confirmed by genetic testing



History of at least 1 episode of hypoglycemia (blood glucose  $<$ 60 mg/dL) within the 2 years before signing the informed consent

\*Additional protocol-defined eligibility requirements apply, including exclusionary criteria.



Have any questions?  
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