LogicBio THERAPEUTICS

Genome editing for pediatric diseases

April 28th, 2019

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LogicBio Therapeutics, Inc. has filed a Registration Statement on Form S-1 (File No. 333-227523), as amended (the "Registration Statement"), with the Securities and Exchange Commission ("SEC") in connection with the offering to which this presentation relates. Before you invest, you should read the Registration Statement, the preliminary prospectus included within the Registration Statement, and other documents we have filed with the SEC for more complete information about us and this offering. You can obtain these documents for free by visiting EDGAR on the SEC website at www.sec.gov. Alternately, copies of the preliminary prospectus may be obtained by contacting Jefferies LLC, Attention: Equity Syndicate Prospectus Department, 520 Madison Avenue, 2nd Floor, New York, NY 10022, or by telephone at (877) 821-7388, or by email at Prospectus_Department@Jefferies.com.

We are an "emerging growth company" within the meaning of the Jumpstart Our Business Startup Act of 2012. As a result, we will be subject to reduced public company reporting requirements. No securities will be offered or sold and no offers to buy will be accepted prior to the time the Registration Statement becomes effective.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data and estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

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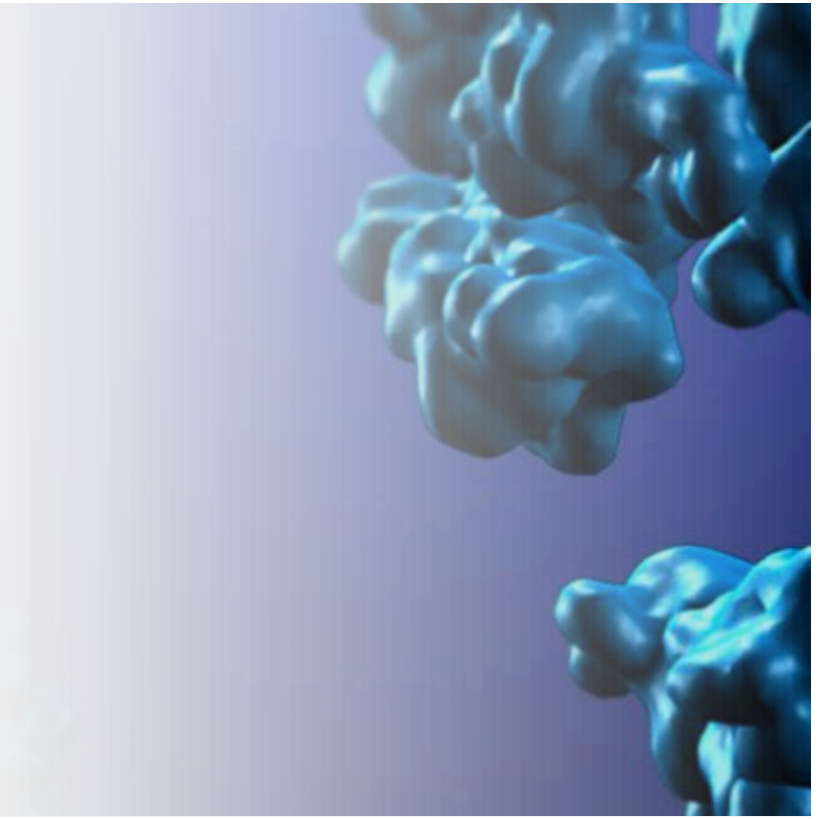
We anticipate that this presentation may contain forward-looking statements, including about our future expectations, plans and prospects, including statements about the preclinical and clinical development of our product candidates, timing of regulatory filings and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "hypothesize," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions. All forward-looking statements are subject to a number of risks, uncertainties and assumptions, and you should not rely upon forward-looking statements as predictions of future events. All forward-looking statements will be based upon current estimates and expectations about future events and financial and other trends. There is no guarantee that future results, performance or events reflected in the forward-looking statements will be achieved or occur. No person assumes responsibility for the accuracy and completeness of the forward-looking statements, and, except as required by law, no person undertakes any obligation to update any forward-looking statements for any reason after the date of this presentation.

LogicBio THERAPEUTICS

LogicBio Therapeutics

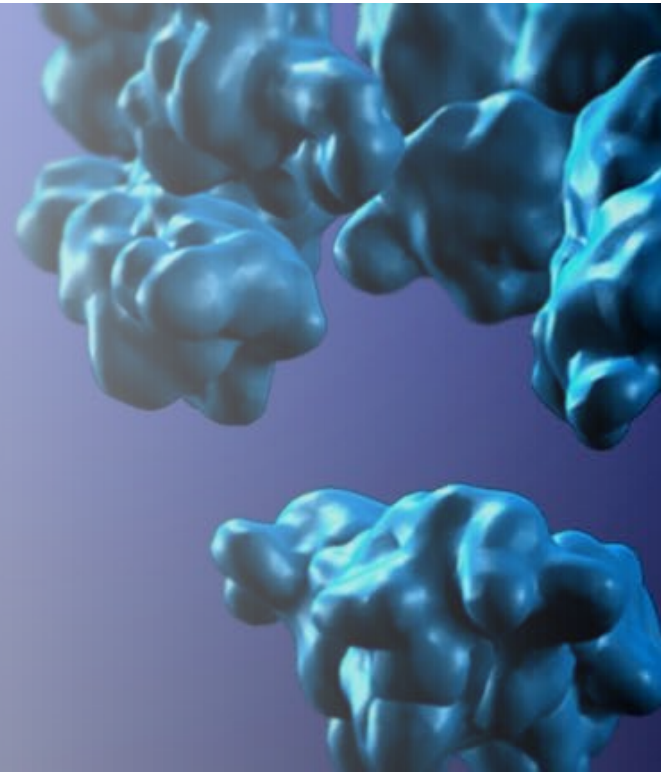
Striving to deliver on the promise of genetic medicine to transform the life of pediatric patients with rare disease

- **GeneRide™:** first-in-class genome editing proprietary platform for a durable treatment from a single injection
- Modular and broadly applicable platform with robust research pipeline
- Lead product LB-001 for methylmalonic acidemia, a rare inborn error of metabolism
- Experienced leadership team with track record of bringing novel drugs to market
- Successful IPO in October 2018 raising $80.5 million



GeneRide™: designed to deliver durable, precise and safer transformative therapies

- **Nuclease-free genome editing** by leveraging homologous recombination
- Therapeutic transgene expression driven by strong **endogenous promoters**
- **Site-specific integration**
- Durable treatment achieved with a **single vector therapy** in animal disease models



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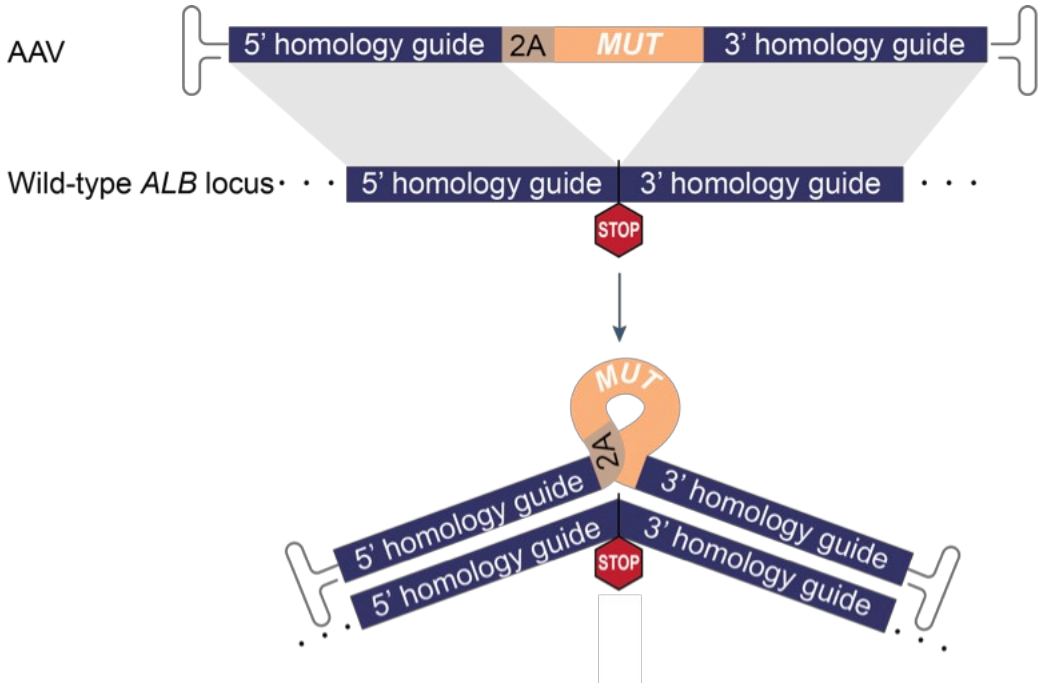
GeneRide: genome editing without nucleases

- Leverages the natural process of homologous recombination (HR)
- Achieves genome editing without engineered nucleases
- High fidelity, site-specific integration, driven by long homology guides
- Non-disruptive integration of therapeutic transgenes



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- Leverages the natural process of homologous recombination (HR)
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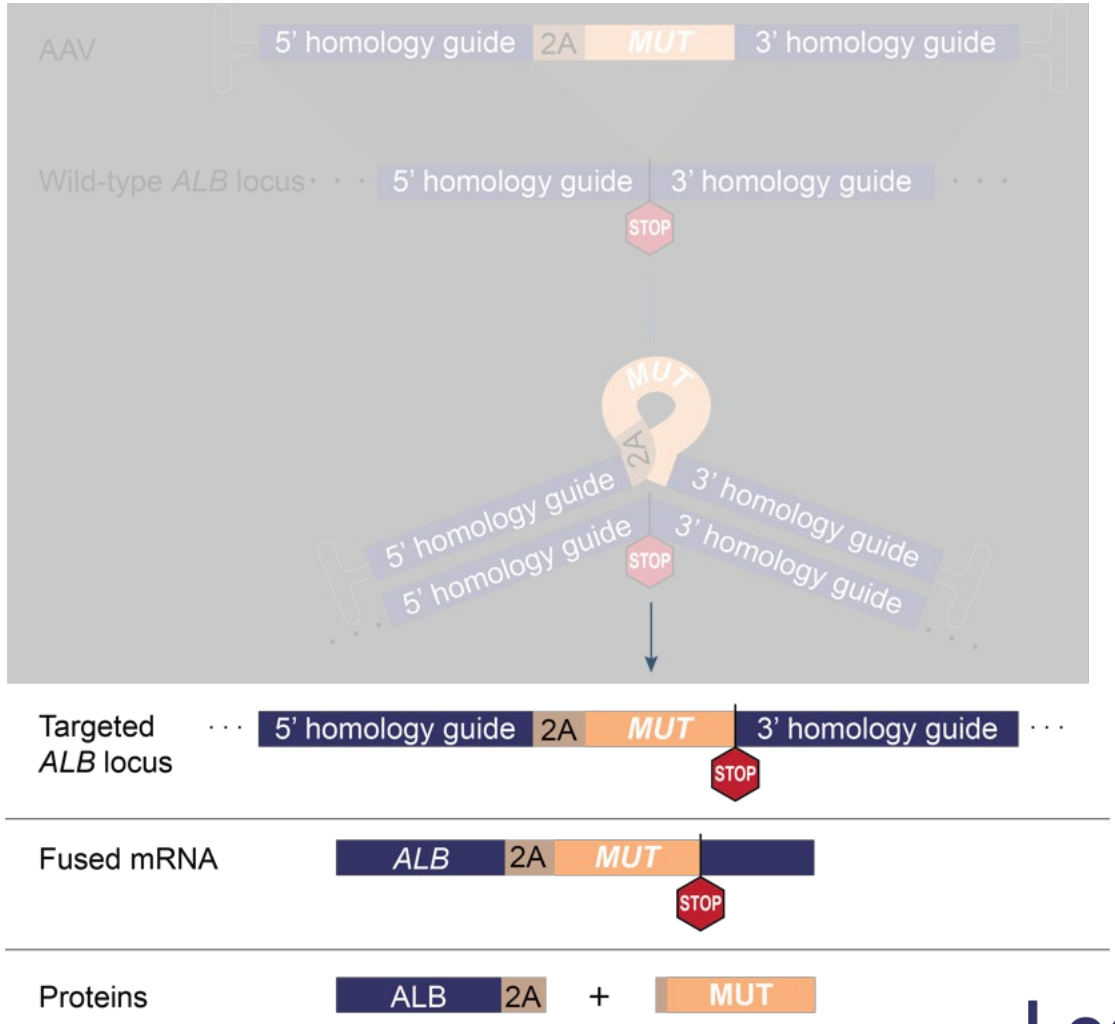
- High fidelity, site-specific integration, driven by long homology guides
- Non-disruptive integration of therapeutic transgenes
- Harnesses potent endogenous promoters to express transgenes



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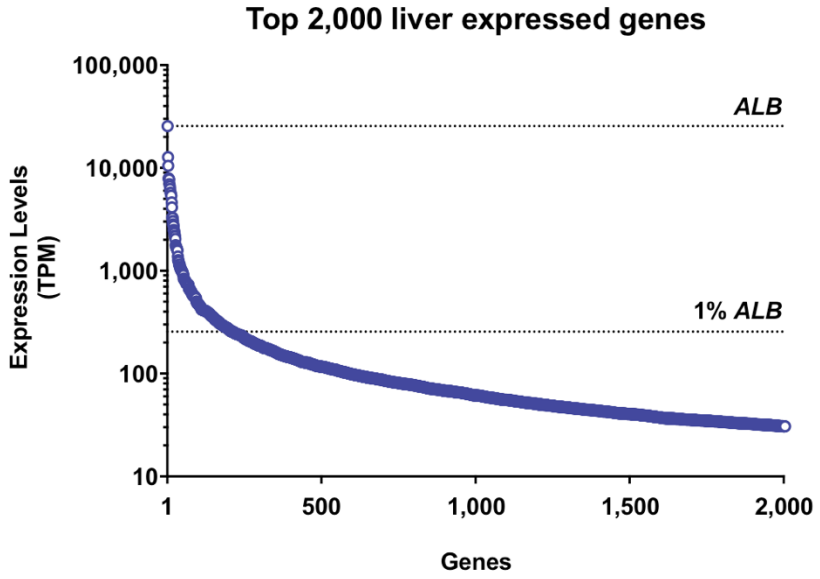
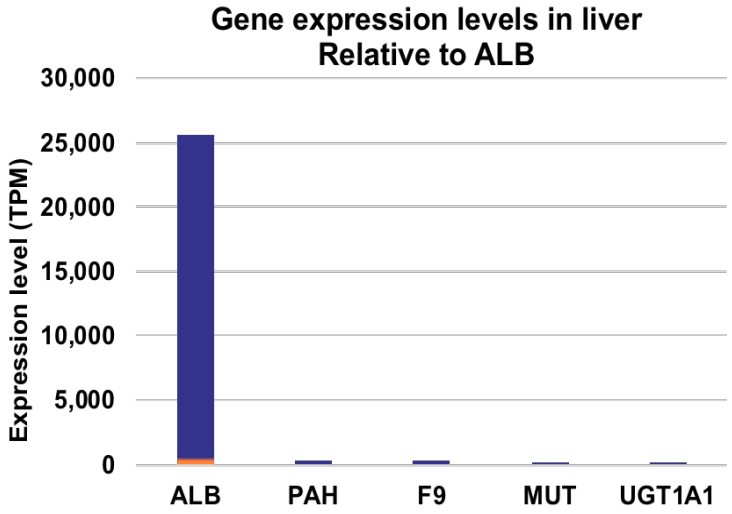
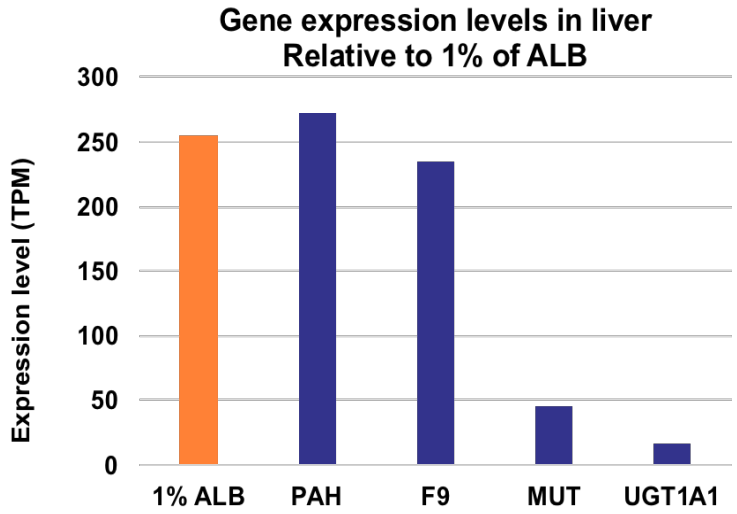
Liver-directed programs: riding the albumin promoter for therapeutic benefit

- Albumin is the most abundant protein in circulation and the most highly expressed gene in the liver
- Modest rates of integration at the *ALB* locus is sufficient to achieve near physiological expression levels of many disease-related liver proteins







Transcriptional profiling data from The GTEx Project

ALB – Albumin
PAH – phenylalanine hydroxylase (PKU)
F9 – Factor IX (Hemophilia B)

MUT – Methylmalonyl-CoA mutase (MMA)
UGT1A1 - UDP glucuronosyltransferase family 1 member A1 (Crigler-Najjar syndrome)

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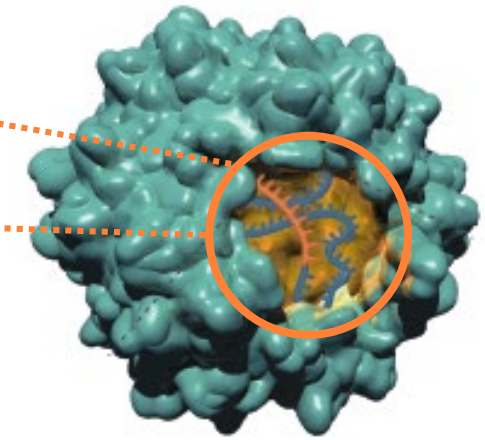
GeneRide: a modular and broadly applicable platform



Transgene – selected for specific indication

Efficacy demonstrated in multiple disease animal models

Stanford MEDICINE

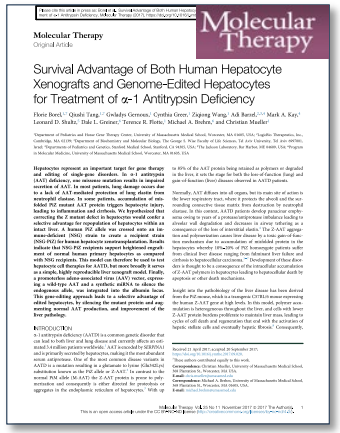
Hemophilia B

Barzel et al., *Nature*, 2015

NIH National Human Genome Research Institute

MMA

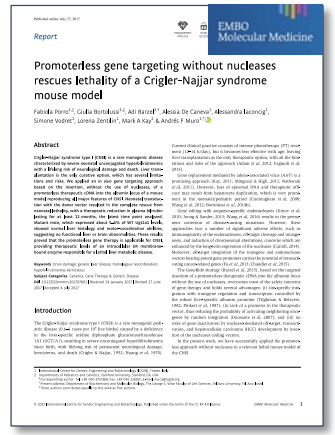
ASGCT, 2017 & 2018



UMASS University of Massachusetts Medical School

A1ATD

Borel et al., *Mol Ther*, 2017



ICGEB International Centre for Genetic Engineering and Biotechnology

Crigler-Najjar

Porro et al., *Mol Med*, 2017

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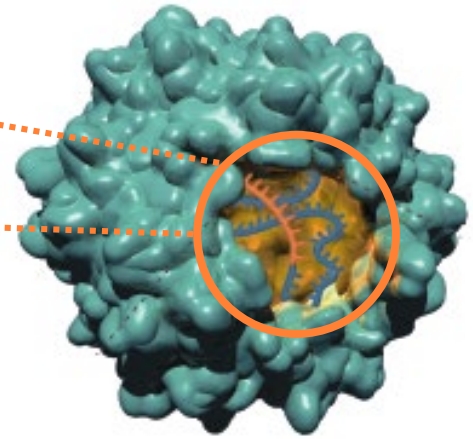
GeneRide: a modular and broadly applicable platform

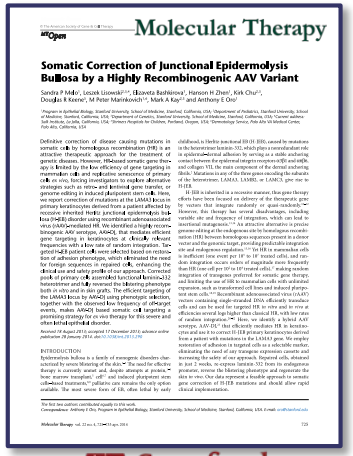
Transgene – selected for specific indication

Efficacy demonstrated in multiple disease animal models

Homology guides – therapeutic levels and tissue specific transgene expression driven by target locus

Genome editing with GeneRide demonstrated at multiple loci





Stanford MEDICINE

Melo et al., *Mol Ther*, 2014

Stanford MEDICINE

Sebastiano et al., *Sci Transl Med*, 2014

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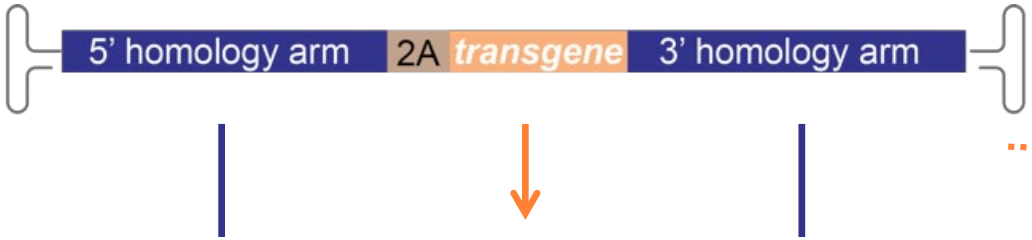
GeneRide: a modular and broadly applicable platform



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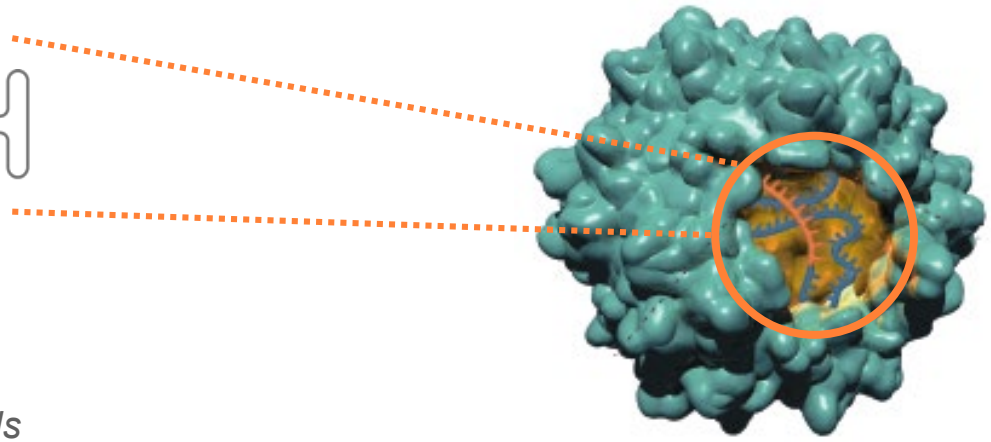

Capsids – Selected for tissue tropism

Strong human liver tropic capsids identified

Other tissue specific capsids in development

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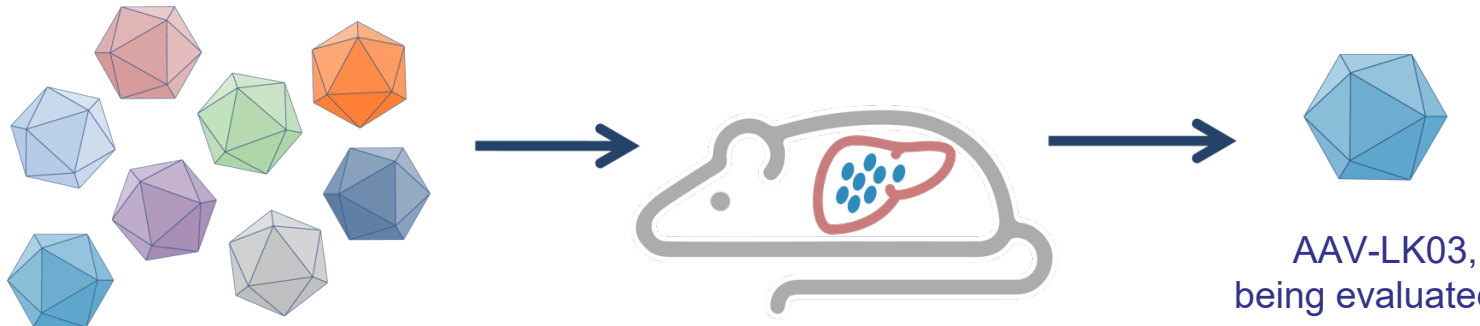
Identification of potent liver-targeting AAV to support our genome editing platform

- Best-in-class: novel approach identified human hepatocyte targeting capsid LK03
- Clinical translation: LK03 optimized to target human hepatocytes
- Broad IP: expanded to include additional novel capsids with enhanced tissue tropism

Stanford MEDICINE

Identification of LK03
Lisowski et al., Nature, 2014



AAV-LK03,
being evaluated in
clinical trials

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Growing pipeline across indications and target tissues

THERAPEUTIC AREA	CANDIDATE	DISCOVERY	IND ENABLING	PHASE 1/2	PHASE 3
Liver	LB-001	Methylmalonic acidemia (MMA)			

Discovery Engine Process

Indication selection

- High unmet need
- Neonatal onset
- Potential for meaningful clinical benefits
- Well understood biology
- Existing animal models / biomarkers

Initial target tissue

- Liver

Liver targeted candidates

- AAV-LK03
- ALB locus
- Disease related transgene

New target tissues

- Muscle
- CNS

New tissue candidates

- Tissue tropic capsid
- Highly expressed, tissue specific integration locus
- Disease related transgene

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Methylmalonic acidemia

Life-threatening inborn error of metabolism with no treatment options

- Incidence: 1 in 50,000 births (US)
- Organic acidemia caused by mutations in *MUT* pathway
- Results in metabolic deficiency and inability to metabolize AA
- Restricted diet: strict low-protein, high-calorie

- Poor quality of life with frequent hospitalizations
- No treatment, only aggressive management of symptoms
- Often fatal in newborns, life expectancy of 20 – 30 years
- Liver or liver-kidney transplants often needed

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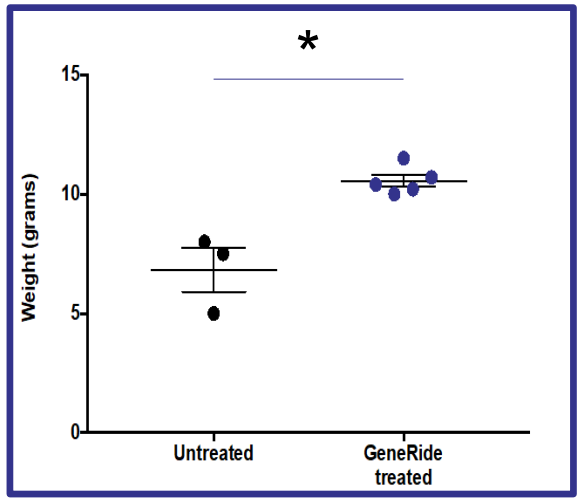
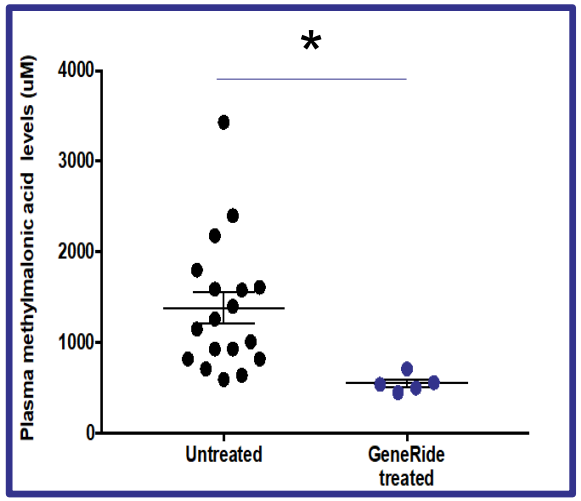
GeneRide integrated *MUT* to provide hepatocytes with a durable selective advantage in animal models

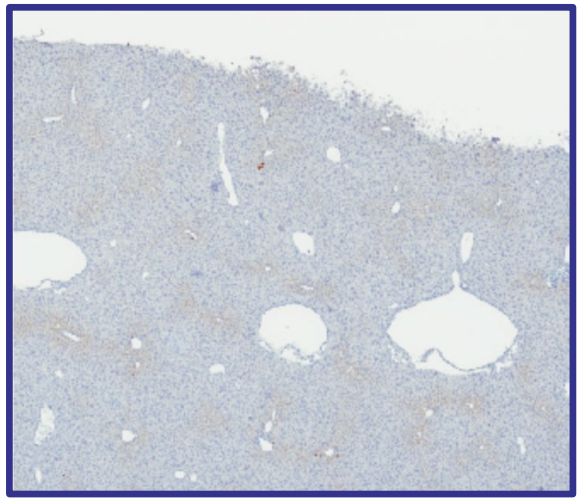
Weight of MCK-Mut mice at 1 month



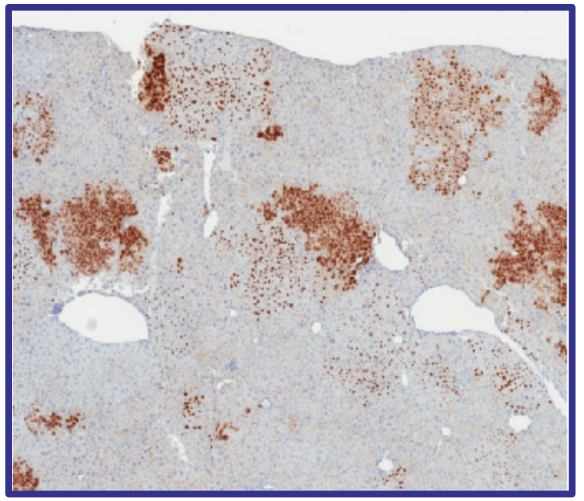
Plasma methylmalonic acid levels at 1 month



Treatment of control mouse, naturally expressing Mut



Treatment of MMA mouse, deficient in Mut in the liver



National Human Genome Research Institute

National Cancer Institute

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