JAGUAR GENE THERAPY

An Evening With

Jaguar Gene Therapy

February 22, 2024

hosted by cureSHANK

Our Mission

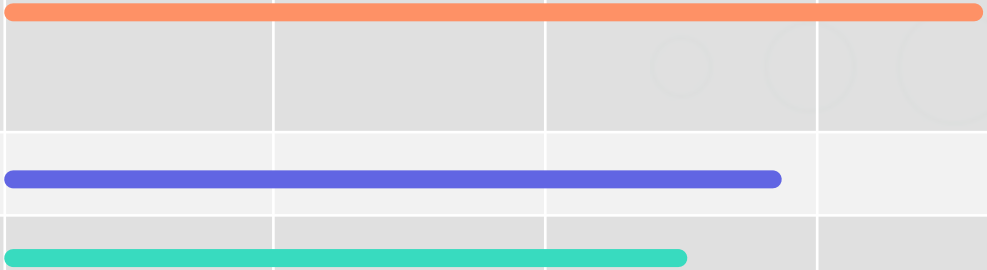
TO ACCELERATE BREAKTHROUGHS GENE THERAPY FOR PATIENTS LIVING WITH SEVERE GENETIC DISEASES

Because people's lives depend on us doing our jobs with passion and purpose, we're held to a high set of standards. There's a patient and a family at the center of everything we do, and they are counting on us.

Time is of the essence, and we are focused on moving gene therapy solutions from bench to bedside as safely and rapidly as possible.

Gene Therapy Programs in Development

	Indication	Target Identification	Discovery	Preclinical	IND Clearance	Clinical
JAG201	A genetic form of autism where a *SHANK3* mutation or deletion is present, and Phelan-McDermid syndrome					
JAG101	Type 1 galactosemia					
JAG301	Type 1 diabetes					



JAGUAR GENE THERAPY

Gene Replacement Therapy Is a Potential Treatment for Patients with Genetic Diseases

PATIENTS WITH MANY GENETIC DISEASES HAVE A DEFECTIVE OR MISSING GENE

Gene replacement therapy is a type of treatment that involves introducing a functional copy of the defective or missing gene back into the body to restore the production of a protein and the natural function of the cells with the goal of changing the course of the disease.

- It is a therapeutic approach being investigated for the treatment of multiple diseases[1]
- Many gene therapies are in early research or clinical trials, and some have already been approved by the FDA[2]

1. National Institutes of Health. Genetics Home Reference. Help me understand genetics. Accessed November 18, 2020. https://ghr.nlm.nih.gov/primer
2. Food and Drug Administration FDA continues strong support of innovation in development of gene therapy products. Press release. Accessed November 18, 2020

About JAG201

JAG201 is an investigational gene therapy to treat *SHANK3* haploinsufficiency including Phelan-McDermid syndrome and a genetic form of autism where a *SHANK3* mutation or deletion is present

- JAG201 aims to deliver appropriate *SHANK3* genetic function via the AAV9 vector to address *SHANK3* haploinsufficiency by enabling expression of SHANK3 protein to restore synaptic function
- Designed to be a one-time treatment
- The JAG201 program is exclusively licensed from the Broad Institute of MIT and Harvard

BROAD INSTITUTE

What's Next for JAG201?

Publish data from out preclinical (animal model) studies

Finalize protocol for first-in-human trial

Initiate a Phase I trial of JAG201 in the U.S. before the end of 2024

Stay Up to Date

JAGUAR IS VERY PROUD TO PARTNER WITH CURESHANK. IF YOU FOLLOW THEIR SOCIAL CHANNELS OR YOU ARE ON THEIR EMAIL LIST, YOU WILL RECEIVE TIMELY UPDATES ABOUT JAGUAR'S JAG201 PROGRAM.

Check out our website:
www.jaguargenetherapy.com

Follow us on LinkedIn:
https://www.linkedin.com/company/jaguargenetherapy/

Have questions? Contact us!
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