Jaguar Gene Therapy
Two Conway Park
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www.jaguargenetherapy.com

Dear Galactosemia Foundation Community,

For individuals living with Type 1 Galactosemia and those caring for them, we understand the journey to a treatment option has been a long one. We established Jaguar Gene Therapy in 2019 with the mission to accelerate breakthroughs in gene therapy for those living with severe genetic diseases. Our team at Jaguar Gene Therapy is proud to partner with you and the Galactosemia Foundation to achieve shared goals.

Jaguar Gene Therapy has announced the development of JAG101, an investigational gene therapy for Type 1 Galactosemia. Gene therapy, including JAG101, involves introducing healthy versions of a faulty gene, such as the GALT gene, back into the body to restore the natural function of the cells. The healthy gene, or the replacement gene, is transferred into the body using an adeno-associated virus (AAV) vector.

Right now, we are in the early stages of the development process, and our JAG101 investigational gene therapy for Type 1 Galactosemia is in preclinical (animal model) testing. We are working hard to conduct the necessary studies, in compliance with all guidelines and regulations, so we can advance the JAG101 program as safely as possible to human clinical trials in the coming years.

We have a lot of hard work ahead of us, and we sincerely hope that by working together, we can help to accelerate a gene therapy breakthrough for the galactosemia community. We hope the below list of frequently asked questions helps you understand more about us and our technology. We look forward to working with you as we continue this journey.

Warm regards,
Joe Nolan, CEO

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Frequently Asked Questions (FAQs)

Who is Jaguar Gene Therapy?

We are a proven team of experts in gene therapy who are fiercely passionate and fearlessly dedicated to the patients and families we serve. We are headquartered in Lake Forest, IL and have a Process Sciences Lab in Cary, NC and a commercial manufacturing facility in Durham, NC. Jaguar Gene Therapy is a growing organization with experience in developing AAV gene therapies.

- Our Team: We consistently integrate patient and family feedback early and often into the development process to guide our goals, decisions, and actions. We fight on with urgency, agility, and integrity because it is our responsibility to do more for patient communities.
- Our Pipeline: Our initial preclinical pipeline targets diseases, including Type 1 galactosemia, genetic causes of autism spectrum disorder, Phelan-McDermid Syndrome and/or neurodevelopmental disorders with SHANK3 mutation or deletion, and Type 1 diabetes.
- Our Platform: Our experience allows us to harness our knowledge of the AAV gene therapy platform and thereby aim to minimize development risk and deliver therapies as safely and as quickly as possible.
- Our Partnerships: Our partnerships provide access to key academic institutions, renowned academic experts and world-class laboratories, which allows for a diversified pipeline of gene therapy technologies and approaches.

What does “Preclinical” and “Pipeline” mean?

Pre-clinical means that we are currently doing research with animal models and not yet conducting trials in humans, and our pipeline refers to the three programs that we are working to advance.

What is Gene Therapy?

Gene therapy involves introducing working versions of a faulty gene into the body to restore the natural function of the cells. Our initial preclinical pipeline utilizes the AAV vector, the vehicle in which the treatment is delivered into the body, to target severe genetic diseases with significant unmet need.

What is AAV, and why would you use it?

A gene therapy treatment requires a vehicle to deliver a working gene into target cells. In Jaguar Gene Therapy’s case, this vehicle is called an adeno-associated virus (AAV) vector.

We chose AAV because it is nonpathogenic (this means it does not cause disease), and it has been shown to efficiently gain access to target cells. Using the AAV vector as a vehicle, a working version of the faulty gene can be delivered to its target. This is intended to help restore production of an important protein or enzyme and, in turn, the function of the cell in the person’s body. After the vector delivers the gene, the AAV vector is no longer required. It breaks down and is processed by the person’s body.

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Would this gene therapy be a cure?
Our goal is to change the course of the disease by treating its root cause. In the case of Type 1 Galactosemia, we believe it will be important to treat as early as possible to reduce the harmful effects of galactose and its other metabolites (GAL-1p and galactitol). The preclinical research currently underway will help us understand what this could mean as a treatment option for people affected by Type 1 Galactosemia.

When would clinical trials start for JAG101?
Jaguar Gene Therapy is still very early in the development process. We are currently in the preclinical phase, which means we are doing animal studies that must be complete before we can safely begin human clinical trials. We are working hard to meet all regulatory requirements so we can advance the Type 1 Galactosemia program as safely as possible to human clinical trials in the coming years. We are committed to keeping the galactosemia community informed as we approach new milestones in the development process. Up-to-date information can be found on our website at www.jaguargenetherapy.com.

Would there be an age requirement to be able to enroll in an eventual clinical trial?
We don't know yet; however, it is possible. Exact inclusion and exclusion criteria for human clinical trials will be established based on what we learn in our ongoing preclinical studies.

Would being in a clinical trial for a gene therapy exclude you from being able to participate in another clinical trial later?
Unfortunately, we do not know the answer to this. It would depend on the goals of investigational therapies and applicable regulatory guidance.

Where can I find more information about Jaguar and JAG101?
You can always visit our website for up-to-date information. We are proud to partner with the Galactosemia Foundation. We will send more letters like these when we have more information to share. We also plan to attend the Galactosemia Foundation conference in 2022 and look forward to meeting some of you there!