Jaguar Gene Therapy
Two Conway Park
150 N. Field Drive, Suite 300
Lake Forest, IL 60045

www.jaguargenetherapy.com

Dear Phelan-McDermid Syndrome Foundation and CureSHANK Community,

For individuals living with Phelan-McDermid Syndrome (PMS) 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 the Phelan-McDermid Syndrome Foundation and CureSHANK to achieve shared goals.

Jaguar Gene Therapy has announced the development of JAG201, an investigational gene therapy for certain genetic causes of autism spectrum disorder, Phelan-McDermid Syndrome and/or neurodevelopmental disorders with SHANK3 mutation (also known as variant) or deletion. Gene therapy works by delivering a healthy version of a gene that is otherwise dysfunctional or missing, such as SHANK3. 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.

Right now, we are in the early stages of the development process, and our JAG201 investigational gene therapy 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 JAG201 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 with partners and patient communities, we can help to accelerate a gene therapy for those with Phelan-McDermid syndrome, and those with certain genetic causes of autism spectrum disorder and neurodevelopmental disorders resulting from SHANK3 mutation or deletion. 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. 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. JAG201 is designed to deliver appropriate SHANK3 genetic function using the AAV vector to treat the root cause of the disease and rescue neurodevelopmental behavioral abnormalities. It is not currently known if the treatment will succeed. The preclinical research currently underway will help us understand what this could mean as a treatment option.

When would clinical trials start for JAG201?
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 JAG201 program as safely as possible to human clinical trials in the coming years. We are committed to keeping the 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 JAG201?
You can always visit our website for up-to-date information. We are proud to partner with the Phelan-McDermid Syndrome Foundation and CureSHANK. We will send more letters like these when we have more information to share.