Jaguar Announces FDA Approval to Begin Phase 1 Clinical Trials of its JAG201 Gene Therapy for Phelan-McDermid Syndrome

We are honored and excited to share that the **first EVER gene therapy for Phelan-McDermid syndrome** (PMS) has been approved to begin clinical trials in 2024!

Jaguar Gene Therapy issued a **press release** this morning announcing that the U.S. Food and Drug Administration (FDA) has cleared the company's Investigational New Drug (IND) Application for JAG201, a gene therapy that delivers a functional *SHANK3* gene to address the root cause of PMS: a deletion or harmful mutation of one of an individual's two *SHANK3* genes.

Eager to inform the PMS community about JAG201 and its upcoming Phase 1 clinical trials, Jaguar has asked us to distribute this **PMS community letter and FAQ**.

On **February 22**, Jaguar will also make a live presentation and answer questions about its JAG201 news at a **community webinar**, hosted by CureSHANK.

Register here for the webinar, and submit questions for Jaguar to **connect@cureshank.org.

The team at **Jaguar is thankful for the help of the PMS community** in helping to pave the way for this FDA approval with our record-breaking Externally-Led Patient-Focused Drug Development **(EL-PFDD) meeting** just over a year ago. In its press release, Jaguar cites the meeting to underscore the need for treatments:

> *Key insights from the [EL-PFDD] meeting include: PMS has severe quality of life impacts on those living with the disease, as well as on parents and siblings; PMS has an overwhelming unmet medical need; and existing therapies and medical treatments are not very effective.*

We at CureSHANK have been working closely with Jaguar for the past two years to help support the realization of their remarkable achievement. We are delighted to share such promising news to our PMS community, which has recently suffered much loss and discouragement. And we are most thankful to our generous donors, without whose generous support we could not keep pressing forward in our mission to accelerate treatments for Phelan-McDermid syndrome.

With hope and gratitude,
The CureSHANK Team