

Taysha's Quarterly **Advocacy Newsletter** 



"Spring work is going on with joyful enthusiasm." - John Muir

Between breaking down walls, making progress in clinical trial development, advocating for the community and welcoming our newest team members, spring has certainly sprung here at Taysha. Thanks for catching-up with us in this edition of Taysha Times.

## Wall Breaking: Hope Is an Action Word

We're breaking down walls at our cGMP manufacturing facility in Durham, North Carolina! This facility bolsters our manufacturing capacity and supports our crucial work to help fight rare diseases. Learn more about what to expect <u>here</u>.



Manufacturing, what this means for patients more broadly:

We asked Greg Gara, Senior Vice President of

"This building is about hope – and hope is a strong word for everyone impacted by rare disease, including our entire team here at Taysha. This building will make a true difference in saving lives. This is our way of putting hope in the ground."

Sharon King, mother to Taylor and President of Taylor's Tale, a CLN1 disease advocacy and research organization, also spoke at our wallbreaking ceremony and shared how the new facility represents an important step in building hope for the rare disease community. "In July 2006, I had no treatment, no hope and no

cure. I didn't believe it then, and I don't believe it now. I want to thank every one of you at Taysha Gene Therapies for your commitment to children and families like mine. I hope you remember hope is an action word."







Officer and Head of Quality Mish Gerhart, Chief Regulatory Officer and

Mish plays a critical role in our regulatory processes. We sat down with her to learn how her work with regulatory agencies informs the overall

efficiency to advance gene therapies into the clinic. Why is the patient and caregiver perspective so important in your work at Taysha? > At Taysha, learnings from patients and caregivers have become a core part

of our approach. You can't simply Google these ultra-rare diseases - and if you

do, there's very limited resources. It's only when talking to the caregiver and

the patient communities that we're able to truly learn about these diseases.

from these groups is so valuable as we develop our gene therapies.

This is where the education starts, and why every piece of feedback gathered

approval process, and how these collaborations enable speed and

Head of Quality

Taysha's pipeline includes numerous gene therapy candidates for ultrarare diseases. How do you integrate the patient insight into gene therapy development? > Science is science. It can't describe the human component. The patient is

the true expert for what it's like to live with their condition, which helps us shape our gene therapy development strategies. For example, as patients and caregivers talk about their daily struggles, we can potentially correlate these to meaningful clinical endpoints, and ensure clinical trial schedules and procedures can accommodate both patients and caregivers. How is Taysha supporting these programs from a regulatory perspective?

> One of the things we really focus on at Taysha is weaving patient feedback

into everything that we do - whether that's into a regulatory application or a

clinical trial protocol. In working closely with patients, we can start conversations that will not only help inform our programs at Taysha, but also provide education for regulatory agencies as well. Drug development is a journey, with a number of hurdles along the way. At Taysha, we have a commitment to these diseases and understanding them as best we can ensuring the patient voice is heard as we do our best to overcome these hurdles and advance our gene therapies forward.

#RareAlly

**Continuing the Journey** 



## #RareAlly is our way of recognizing the allies in rare disease who inspire us every day, who challenge us to give and be our best, and who we

lean on for support. Hear how Tracy Porter from our Human Resources team embodies what #RareAlly means and how she's found inspiration through her own

When it comes to rare disease, who is your rock, your source of strength or your ally – and why?



diagnosis journey.

with Systemic Sclerosis (Scleroderma). I quickly felt a circle of love, strength and inspiration fold around me. This circle is

comprised of my family, friends,

"Six years ago, I was diagnosed

co-workers and other sclero warriors living with this rare and presently incurable disease." Tracy Porter Chief People Officer We officially met the 100-employee mark – and we're continuing to grow! Join

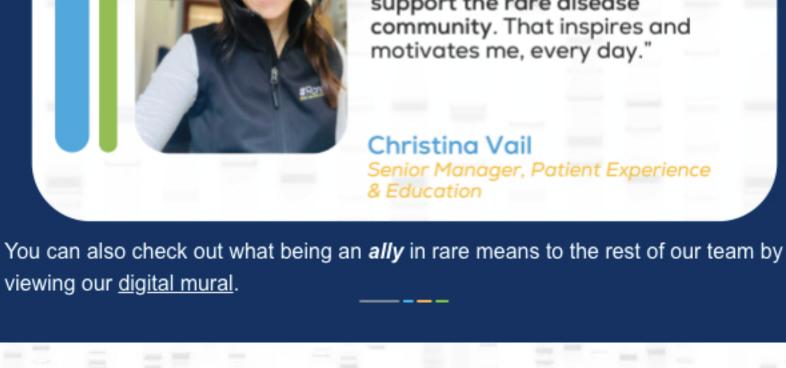
When it comes to rare disease, who is

to be grateful to do this work and

your rock, your source of strength or your ally - and why? "Each interaction I have with a patient or caregiver is a reminder

us in welcoming our new advocacy team member and #RareAlly, Christina

Vail, Senior Manager of Patient Experience and Education.



Acquiring TSHA-120 for the

treatment of GAN: We acquired

now known as TSHA-120, for the

clinical-stage gene therapy program,

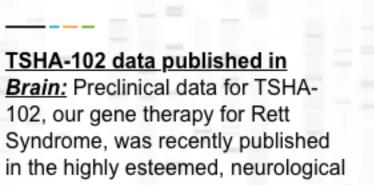
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more here.

support the rare disease community. That inspires and motivates me, every day.'

Christina Vail

Senior Manager, Patient Experience



treatment of giant axonal science peer-reviewed journal, neuropathy (GAN) from Hannah's Brain. Learn more about it in our Hope Fund and the National video summary of the findings here. Institutes of Health (NIH). Learn

We've been busy. Here's a quick recap of our latest news and updates.

The Latest at Taysha

Blazing trails for gene therapy: We recently announced positive new preclinical data in neurodegenerative diseases, neurodevelopment disorders and genetic epilepsies. Together, these data highlight our next wave of novel gene therapies with the potential to support numerous patient populations.

disease) clinical trial recruitment: A Phase 1/2 clinical trial of TSHA-101 is currently enrolling patients diagnosed with infantile GM2 gangliosidosis. The trial is being conducted at Queen's University in Kingston, Ontario, Canada.

Supporting GM2 gangliosidosis

(Tay-Sachs disease and Sandhoff

Founder and CEO, RA Session II, Dr. Prasad also gave an interactive spoke at this year's SXSW 2021 presentation on gene therapy to Lab Conference alongside Kasey Rascals, a virtual camp for kids Woleben of the Cure SURF1 Foundation and Rare Village

Foundation, as well as other notable

advancements being made in gene

leaders in the space, regarding

therapy today. Listen to the full

NTSAD Family Conference:

panel discussion here.

SXSW 2021: Our President,

Where We've Been

Dr. Suyash Prasad, our Chief Medical Officer & Head of R&D, presented at the NTSAD Family Conference. He provided an overview of TSHA-101 and how the GM2 gangliosidosis (Tay-Sachs disease and Sandhoff disease) community plays a critical role in our development of a meaningful clinical trial. Beryl Institute Patient Experience Week: In honor of Patient

Experience Week, our Chief Patient

Officer, Emily McGinnis, shared perspective on creating an optimal clinical trial experience for patients. Check out her responses here.



Lab Rascals Virtual STEM Camp:

a presentation entitled, "An Innovative Regulated Gene Replacement Therapy Approach for Rett Syndrome," highlighting information about Taysha's gene therapy product candidate, TSHA-102, for Rett syndrome. ASGCT Virtual Annual Meeting 2021: RA Session II presented a keynote address at this year's ASGCT pre-meeting, while Dr.

Steven Gray provided insight into

patient advocates' roles in fostering gene therapy research, as well as

challenges and opportunities faced

in treatment development during the

Development for Rett Syndrome,"

Rettsyndrome.org at the INSAR

facilitated by Rett Syndrome

Research Trust (RSRT) and

Annual Meeting on May 4.

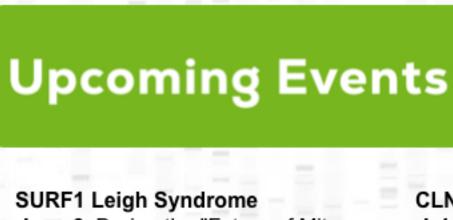
annual meeting.

During the panel, Dr. Prasad shared

TAYSHA

We spoke and listened at a number of conferences and events this spring.

Check out where we've been - over Zoom, of course!



June 3: During the "Future of Mito Disease Research: Powered by YOUR Data" webinar, Dr. Suyash Prasad will share how natural history studies are important in developing potential therapies for rare diseases. We invite you to join

this hour-long webinar to learn about

how data is important for Mitochondrial diseases, but applies to all diseases. Register here. **Global Genes** June 10-11: Taysha is a proud supporter of the Rare Drug Development Symposium virtual event. We invite patient advocates to join this symposium to learn about various ways to engage and drive research in preclinical, translational and clinical pathways in rare

diseases. Register here.

**CLN1** Disease July 22-25: The BDSRA Family Virtual Conference supports families and research for Batten disease, including CLN1 disease. As Taysha

is an ally to the CLN1 disease community, we will provide an update on engagement with CLN1 caregivers and insight on the clinical trial design of TSHA-118. Register here. Angelman Syndrome August 2-6: The Angelman Syndrome Foundation will hold its virtual ASF Research Symposium

and Family Conference, where Taysha will provide an overview of TSHA-106, our gene therapy program for Angelman syndrome. Register here.

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