TAYSHA TIMES

Taysha's Quarterly Advocacy Newsletter

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"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 here.

We asked Greg Gara, Senior Vice President of Manufacturing, what this means for patients more broadly:

"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 wall-breaking 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."

Meet Mishima "Mish" Gerhart

A Q&A with our Chief Regulatory Officer and Head of Quality

Mish Gerhart, Chief Regulatory Officer and Head of Quality

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 approval process, and how these collaborations enable speed and 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. This is where the education starts, and why every piece of feedback gathered from these groups is so valuable as we develop our gene therapies.

Taysha's pipeline includes numerous gene therapy candidates for ultra-rare 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.

Continuing the Journey

#RareAlly

#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 diagnosis journey.

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

"Six years ago, I was diagnosed 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, co-workers and other extra 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 us in welcoming our new advocacy team member and **#RareAlly,** Christina Vail, Senior Manager of Patient Experience and Education.

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

"Each interaction I have with a patient or caregiver is a reminder to be grateful to do this work and support the rare disease community. That inspires and motivates me, every day."

Christina Vail
Senior Manager, Patient Experience & Education

You can also check out what being an **ally** in rare means to the rest of our team by viewing our digital mural.

The Latest at Taysha

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

Acquiring TSHA-120 for the treatment of GAN: We acquired exclusive worldwide rights to a clinical-stage gene therapy program, now known as TSHA-120, for the treatment of giant axonal neuropathy (GAN) from Hannah's Hope Fund and the National Institutes of Health (NIH). Learn more here.

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.

TSHA-102 data published in *Brain*: Preclinical data for TSHA-102, our gene therapy for Rett Syndrome, was recently published in the highly esteemed, neurological science peer-reviewed journal, *Brain*. Learn more about it in our video summary of the findings here.

Supporting GM2 gangliosidosis (Tay-Sachs disease and Sandhoff 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.

Where We've Been

We spoke and listened at a number of conferences and events this spring. Check out where we've been - over Zoom, of course!

SXSW 2021: Our President, Founder and CEO, RA Session II, spoke at this year's SXSW 2021 Conference alongside Kasey Woleben of the Cure SURF1 Foundation and Rare Village Foundation, as well as other notable leaders in the space, regarding advancements being made in gene therapy today. Listen to the full panel discussion here.

NTSAD Family Conference: 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: Dr. Prasad also gave an interactive presentation on gene therapy to Lab Rascals, a virtual camp for kids to learn more about the exciting world of STEM.

Rett Syndrome International Society for Autism Research (INSAR) Annual Meeting: Dr. Prasad participated in a panel, "Gene-Based Therapies in Development for Rett Syndrome," facilitated by Rett Syndrome Research Trust (RSRT) and Rettsyndrome.org at the INSAR Annual Meeting on May 4.

During the panel, Dr. Prasad shared 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 annual meeting.

Upcoming Events

SURF1 Leigh Syndrome
June 3: During the "Cure 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 15-17: 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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