TAYSHA TIMES

Introducing Taysha's Quarterly Advocacy Newsletter

February 2021
Vol. 1

At Taysha, values like **collaboration** and **transparency** are the foundations that our company was built upon. It's through close partnership and open communication that we can bring life-changing therapies to the people who need them most.

The rare disease community is an essential part of who we are, and because of that, we intend to keep you as up-to-date as possible on how we're driving our mission forward. This is why we are pleased to introduce *Taysha Times*: a quarterly newsletter for us to share recent updates and information about Taysha.

On behalf of the entire Taysha team, thank you for being an integral part of the work that we do!

WE ARE AN ALLY IN RARE

In the Caddo native American language, Taysha means ally.

Ally. It's engrained in our name and encompasses who we are to our very core. We strive to be a trusted ally to the patient community, employees, researchers, physicians and the many partners we work with – further fueling the work we do every day.

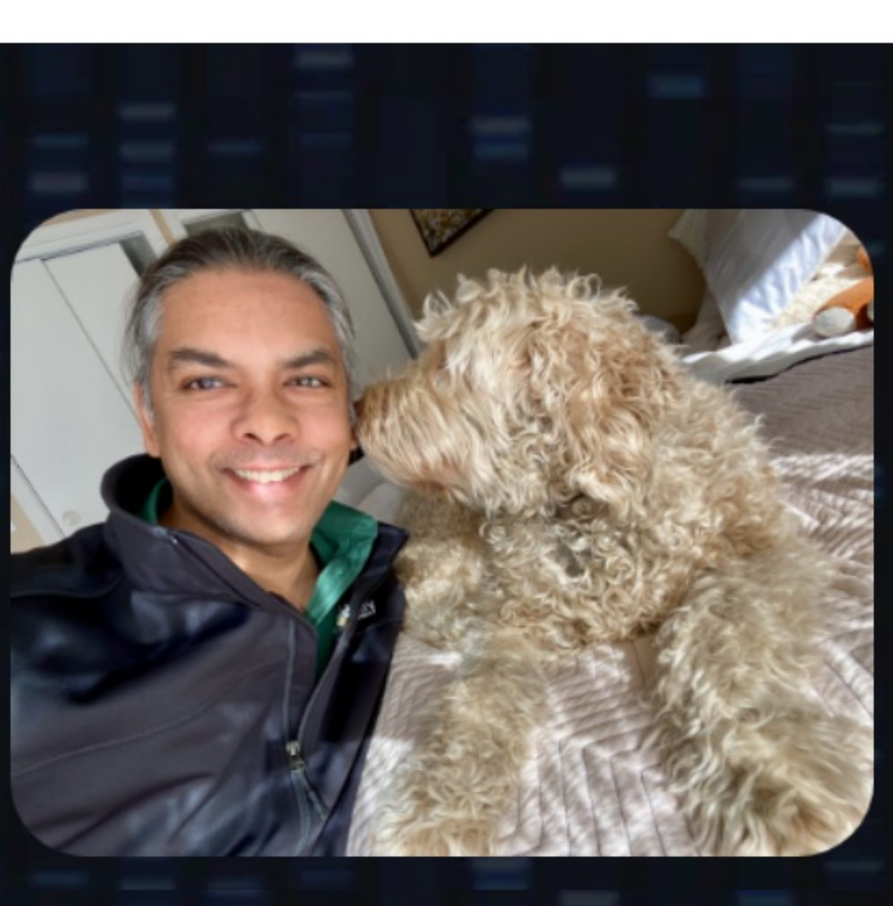
THE TAYSHA APPROACH

Our unique approach to gene therapy consists of three key components: AAV9 gene therapy programs that focus exclusively on monogenic CNS diseases, our experienced team with a track record for developing and commercializing gene therapies, and our collaboration with UT Southwestern (UTSW) that serves as an engine for innovation.

OUR COLLABORATION WITH UTSW

Through our close-knit partnership with UTSW, a renowned gene therapy powerhouse, we are able to leverage the collective expertise of the university's researchers, clinicians and investigators with decades of experience in conducting cutting-edge research and providing clinical care.

Learn more about our approach.



A Q&A with our Chief Medical Officer

Suyash Prasad, MBBS, MSc., MRCP, MRCPCH, FFPM
Chief Medical Officer and Head of Research and Development

We sat down with Suyash Prasad, MBBS, MSc., MRCP, MRCPCH, FFPM, chief medical officer and head of research and development, to discuss the Taysha approach and learn more about the crucial work he does on behalf of patients.

What inspired you to pursue a focus on rare diseases?

> Before I moved into a medical role within industry, I trained and worked as a pediatrician, with a specific focus on pediatric intensive care. Since leaving full-time clinical practice, the majority of my time in industry has been in medicines development for children. Thus, the rights and well-being of children have been woven into everything I do. Children with severe and devastating rare diseases are a vulnerable group within our society. A large part of my role is to simply be an advocate for such children and represent their interests – as physicians may be unfamiliar with the diagnosis, management and general knowledge about the condition.

How would you describe your role at Taysha?

> As the CMO and head of research and development, I oversee a wide array of functions including clinical sciences, clinical operations, pharmacovigilance, biometrics, preclinical science, bioanalytics, medical affairs and health outcomes. I work to ensure each contributes to Taysha's goals and our aim of serving children and families affected by serious, rare, neurological disease. In addition, I have a role as an executive leader at the company – where it is important to have the medical, scientific and patient perspective represented.

I believe it is important the senior physician at Taysha reflects the voice of the patient and the family by ensuring we carefully consider their perspective in clinical trial design, endpoints and study conduct, as well as how we communicate with regulators. As the senior physician, I also represent the ethical and medical voice of the company, both internally, and externally among key opinion leaders, regulators and patient organizations. Part of that involves helping to encourage a culture of scientific and medical rigor and using that as a basis for strategic decision-making across our portfolio of programs.

What is the role of patient advocacy groups in supporting gene therapy research?

> At Taysha, we are deeply committed to working with patient advocacy groups. I believe that the individuals and families affected by the rare and devastating diseases we serve are our partners, collaborators and teachers. Accordingly, the perspective of patients, families and patient groups is of critical importance to our mission of developing new therapies. We accomplish this through activities such as patient focus groups led by Patient Affairs, attendance at patient advocacy meetings and one-on-one meetings with patient advocacy leaders.

What is next for gene therapy? What excites you about the future for this field?

> Throughout my career, both in industry and clinical practice, it has been evident the brain is the hardest organ to treat. It is the organ where disease is the most uncompromising, and is reflected in early mortality, or a profoundly sad and relentlessly progressive clinical course. Given Taysha's focus, it is very rewarding for me to be given an opportunity to meaningfully help children with grave neurological disease. The future is bright. We are on the cusp of a paradigm shift in how we can treat such diseases with the advent of gene therapy and genetic medicines, and I am immensely proud that Taysha is one of the organizations that is leading the way.

#RareAlly TAYSHA

#RAREALLY FOR RARE DISEASE DAY

Rare Disease Day, which falls on February 28 each year, helps raise awareness of the significant impact rare diseases have on the entire world. To build upon this annual recognition of the rare disease community, we also introduced a new Taysha initiative – **#RareAlly**. #RareAlly is all about coming together as one. It's our unique 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 their support or expertise. Visit our #RareAlly page.

MEET OUR ADVOCACY TEAM

In each newsletter, we'll be highlighting individuals who truly embody what a #RareAlly means to us and to the community more broadly. For our first newsletter, we would like to introduce the members of our patient advocacy team – a dedicated group of people who make all of our work with you possible.

Emily McGinnis: Chief Patient Officer and Head of Government Affairs

#RareAlly means being a partner, ensuring that you listen and learn, and demonstrate collaboration in a meaningful way.

Katie Hogan: Senior Director, Patient Advocacy and Government Affairs

There's nothing more inspiring to me than seeing a family come together to provide the best life possible for their loved one with a rare condition.

Kristin Phillips: Senior Director, Community Engagement

I am inspired by – and try to emulate in my work – the determination, compassion and hope of the rare disease community.

Jennifer VanHoutan: Associate Director Global Patient Advocacy

My children are the reason I work on potential treatments so that one day a family won't have to hear "there is no treatment."

THE LATEST FROM TAYSHA

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

> **Launching a New Gene Therapy Manufacturing Facility:** We acquired a new 187,000-square-foot facility in Durham, NC to help support preclinical through commercial current Good Manufacturing Practices (cGMP) manufacturing. This will enable us to more quickly deliver on our broad pipeline of gene therapies.

> **Collaboration with AllStripes:** We announced a multi-year collaboration with AllStripes (formerly RDMD), a healthcare technology company dedicated to accelerating research for patients with rare diseases. We'll leverage AllStripes' platform to inform our understanding of SURF1-associated Leigh syndrome natural history, burden of disease and patients' diagnostic journeys.

> **Six Programs Received Orphan Drug and Rare Pediatric Disease Designations:** Several of our gene therapy programs have received Rare Pediatric Disease and Orphan Drug Designations from the FDA. These designations are important as they offer incentives that may provide opportunities for Taysha to work with the FDA and potentially expedite development of our gene therapies.

Our programs with these designations include:
- •TSHA-101 for GM2 gangliosidosis (Tay-Sachs disease and Sandhoff disease)
- •TSHA-102 for Rett syndrome
- •TSHA-103 for SLC6A1 haploinsufficiency disorder
- •TSHA-104 for SURF1-associated Leigh syndrome
- •TSHA-105 for SLC13A5-related epilepsy
- •TSHA-118 for CLN1 Batten disease

> **Advancing Our Gene Therapy Programs:** To prepare for potential meetings with health agencies, Taysha is meeting with physicians and parents to gain an understanding of the patient journey, including diagnosis, symptoms and management. We expect these discussions to provide keen insight in the development of our clinical trial protocols. We plan to work closely with health agencies in the hopes of submitting investigational new drug (IND) applications and starting clinical trials for certain gene therapy programs in 2021. More information about our programs and forward-looking statements are available in our press release.

WHAT ELSE HAVE WE BEEN UP TO?

Presentation at the WORLDSymposium Virtual Scientific Meeting 2021: This month, we attended the conference to deepen our knowledge about lysosomal storage disorders, including CLN1 Batten disease and GM2. We also presented findings from our study that gathered critical insights from the GM2 gangliosidosis caregiver community, who we would like to sincerely thank for participating in this initiative.

Rett Syndrome Awareness Month: By supporting the Rett Syndrome community in silence, the Taysha team participated in Rett Syndrome Awareness Month in October to raise awareness of the rare neurogenetic disorder that impacts a child's ability to speak: **view here**.

Season of Hope for Tay-Sachs & Sandhoff Disease: During NTSAD's Season of Hope in October 2020, our team showed support for the Tay-Sachs & Sandhoff Disease patient community: **view here**.

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