Monthly Newsletter

August 2026 **CTNNB1 FOUNDATION** **Issue #13**

Dear CTNNB1 community,

We hope you have all had a wonderful summer so far! 🏖️

After a short break in July to recharge following an incredibly busy first half of the year, our team is back with a new edition of the newsletter—and there is a lot to share.

Inside, we look back at our unforgettable conference and Natural History Study assessments in Barcelona, celebrate the incredible efforts of everyone who helped make the event a success, and share updates on the Dragonfly Natural History Study and our ongoing clinical trial.

You'll also find an opportunity to contribute to new CTNNB1 research focused on caregivers, learn how you can support the future of CTNNB1 research, and read an inspiring story about Selim, whose determination reminds us that every child has the potential to achieve extraordinary things.

Thank you for being part of this incredible community. Every family, volunteer, researcher, clinician, donor, and supporter plays an important role in bringing us closer to a future where children with CTNNB1 syndrome have access to effective treatments and the support they deserve.

We hope you enjoy this month's newsletter!

The CTNNB1 Team

Community News

🇪🇸 Looking Back: A Successful Conference and Natural History Study Visit in Barcelona

From June 15–19, the CTNNB1 community came together in beautiful Barcelona, Spain, for several inspiring days of research, learning, and connection. Families, researchers, clinicians, and advocates from around the world gathered to contribute to ongoing studies, attend educational presentations and discussions, and share experiences with others who understand the unique journey of CTNNB1 syndrome.

The Natural History Study (NHS) assessments, held at **Hotel SB Diagonal Zero Barcelona**, welcomed approximately 50 participants from across the globe. An additional 35 participants completed their evaluations at **Vall d'Hebron Hospital**, helping accommodate many of our Spanish-speaking families. We are deeply grateful to the hospital team for their support and collaboration. 🙌

Over two packed conference days, attendees enjoyed a diverse program featuring both scientific and family-focused presentations. Highlights included an update on the first participant enrolled in the GAIN gene therapy trial, insights from families participating in the study, research updates, and discussions on the future of CTNNB1 syndrome care and treatment.



A special thank you goes to **Asociación CTNNB1 España** for organizing childcare throughout the conference, allowing parents to fully participate in the sessions and discussions. ❤️

Presentation recordings are already available on our YouTube 🎥 , and a post-conference booklet containing lecture abstracts and key takeaways is also in preparation.

Most importantly, we hope everyone returned home with new knowledge, renewed hope, meaningful connections, and wonderful memories.

A heartfelt thank you to the entire organizing committee, physicians, researchers, psychologists, physiotherapists, volunteers, and family representatives whose dedication made this event possible. We would also like to express our sincere gratitude to our partners and sponsors for their continued support, and to the **ERDERA Networking Support Scheme 2025**, whose co-funding helped make this conference a reality.

We are already looking forward to seeing you next year in **Portugal**. 🇵🇹 More information will be shared in the coming months!











Research & Clinical Trial Updates

🩺 Dragonfly NHS Update

With the successful completion of our clinical examinations in Ljubljana, Barcelona, and Boston, we have now concluded this year's Dragonfly Natural History Study (NHS) assessments.

Thank you to all the families who participated and contributed their time and effort to this important research. Your continued involvement is helping us better understand CTNNB1 syndrome and move future treatments forward.

📅 What happens next?

We will begin planning the next round of Dragonfly NHS examinations in **early 2027** (approximately January–February).

🌱 Interested in joining the study?

If your child is not yet enrolled in the Dragonfly NHS, you can express your interest by completing our short waitlist form:

👉 **Join NHS Waitlist Here**

Please note: Completing this form **does not mean your child has been enrolled in the study.** It simply lets us know that you are interested in participating.

All information submitted through the form will be kept strictly confidential and will only be accessed by designated members of the study team for the purpose of managing the study waitlist and enrollment.

Because the number of study staff and trained assessors is limited, we are only able to enroll a certain number of new participants each year. As soon as a place becomes available, a member of our study team will contact you to begin the enrollment process.

👨‍👩‍👧 For families already enrolled

If your child is already participating in the Dragonfly NHS, there is no need to complete the waitlist form again.

At the beginning of next year, we will contact you by email and invite you to register for your child's annual clinical examinations.

Please keep an eye on our website and social media channels for future updates.

If you have any questions about the Dragonfly Natural History Study, please contact us at **nhs@ctnnb1-foundation.org** 📩

🧬 Clinical Trial Update

We are pleased to share that the GAIN clinical trial continues to move forward successfully. We have now enrolled our third participant, who has arrived safely in Slovenia and will soon be undergoing pre-treatment assessments in preparation for dosing.

The gene therapy procedure is scheduled to take place in **early September.**

We are incredibly grateful to this family for their trust and courage in taking part in this groundbreaking clinical trial. Please join us in wishing them a smooth procedure, a successful recovery, and all the very best in the weeks and months ahead. 💙

Opportunity to participate in new CTNNB1 research

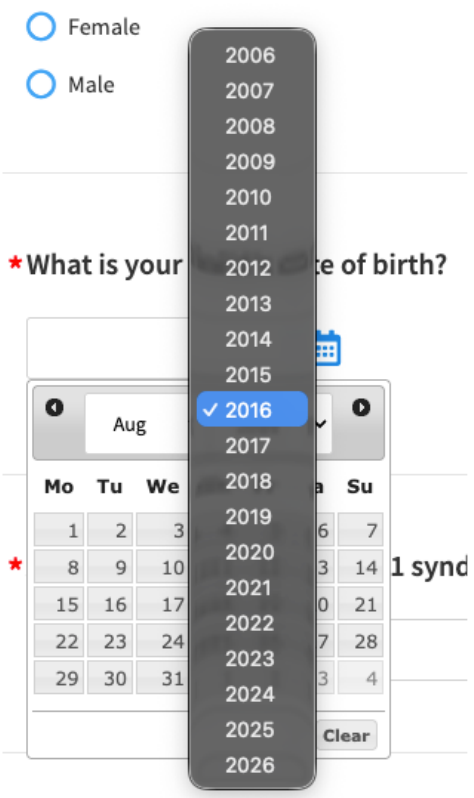
We invite all **primary caregivers of children with CTNNB1 syndrome** to participate in an international research study exploring caregiver burden, mental well-being, resilience, social support, and quality of life.

While many studies focus on our children, very little research has examined the experiences of the people who care for them every day. Your participation will help us better understand the challenges caregivers face and identify factors that may protect caregiver well-being. We hope these findings will contribute to improving family-centered care and support services for CTNNB1 families in the future.

What does participation involve?

Complete an online questionnaire (approximately **25–30 minutes**). **Both caregivers** of the child can fill out the survey.

Fill out survey



‼️ **Please note:** When selecting your child's date of birth, the date picker may initially display years from 2016 onwards. If your child was born before 2016, simply click on "2016" at the top of the year list, then click it once more to access earlier years and scroll to the correct birth year. There is no age limit set in the date selector.

If your child has already participated in the Dragonfly Natural History Study in 2026, you **do not** need to complete the Vineland questionnaire again—we will use the information you have already provided, with your consent.

If your child has not participated in Dragonfly this year, you will also receive a separate Vineland-3 questionnaire by email (approximately **20 minutes**), which can be completed at a later time.

Participation is completely voluntary, and all responses will be handled confidentially.

Thank you for helping us advance CTNNB1 research!

Fundraising Updates

💙 Support the Future of CTNNB1 Research

Everything you've read in this newsletter—from the international conference and Natural History Study to our clinical trial—has only been possible because of the generosity of our community and supporters. As we continue to grow, so do the resources needed to keep this work moving forward.

Today, we need your help more than ever.

Your donation helps us continue critical research, support clinical trial activities, organize community events, provide resources to families, and ensure that promising treatments continue moving toward approval and accessibility. Every contribution—regardless of size—makes a meaningful difference.

If you are unable to donate, you can still help by sharing our mission. Many of the opportunities that have brought us this far started with a simple introduction or referral.

🔍 If you know of any organizations, companies, foundations, or individuals who may be interested in supporting a meaningful cause, we would be incredibly grateful if you shared our contact information with them or invited them to reach out to us directly at **contact@ctnnb1-foundation.org**

Donate

Stories of Impact: Selim

When people hear about CTNNB1 syndrome, they often focus on the challenges: delays in speech and communication, difficulties with motor skills, and cognitive impairment. But every child is more than a diagnosis, and Selim's story is a beautiful reminder of what can be achieved when determination, patience, and love come together.

Recently, Selim took on one of the most demanding roles imaginable for a young actor: Hamlet. For nearly an hour, he performed his lines independently, without prompts or assistance, captivating the audience with his focus, confidence, and energy. It was a remarkable achievement—one that left his family, teachers, and community filled with pride.

What makes this accomplishment even more extraordinary is the journey that led to it.

Like many children with CTNNB1 syndrome, Selim faced significant developmental challenges. Just three years ago, he struggled with fine motor skills and could not properly hold a pencil. Today, he can write, learn scripts, and perform on stage. His progress did not happen overnight. It was built through years of hard work, perseverance, therapy, encouragement, and unwavering support from those who believed in him.

His family never accepted limitations as permanent barriers. Instead, they focused on possibilities. Step by step, challenge by challenge, Selim continued to grow.



His success on stage reflects not only his own determination, but also the power of a supportive environment. When children are surrounded by patience, understanding, and belief in their potential, they often exceed expectations and surprise us in extraordinary ways.

Selim's performance has already been showcased in five different venues, and his journey may soon reach an even bigger stage. His family recently received exciting news that the play could be performed in Ankara for the President of Turkey—an opportunity that would further highlight the abilities and potential of children with special needs.

For families across our community, Selim's story is a powerful message of hope. Every child with CTNNB1 syndrome follows their own unique path, but progress is possible. Achievements may look different for each child, yet every step forward matters.

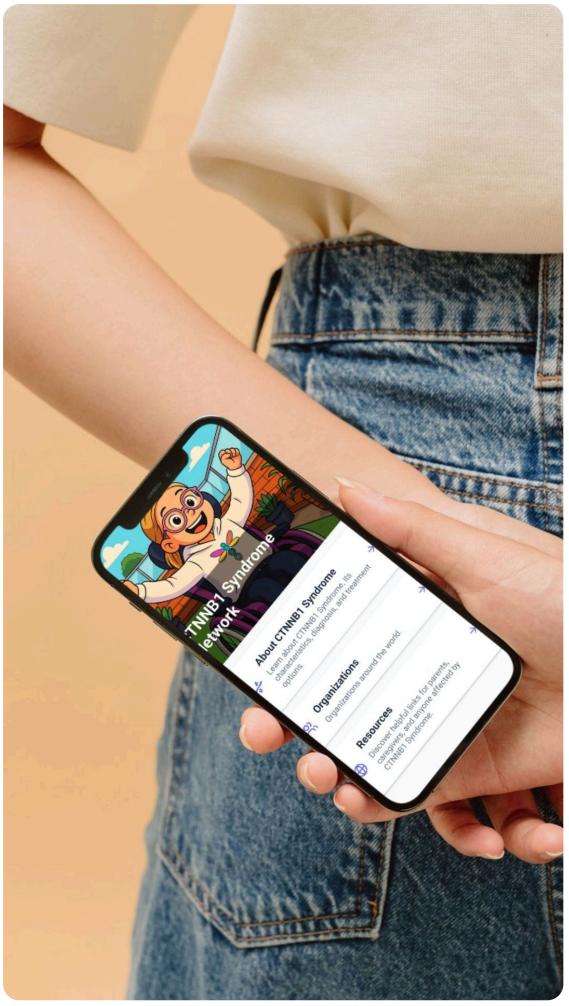
Today, we celebrate Selim—not only for his outstanding performance as Hamlet, but for showing all of us what can happen when a child is given the opportunity to shine.

"Selim proved that limits are often far smaller than we imagine. With love, patience, and belief, children can accomplish extraordinary things."

- story by Selim's parents







Don't forget to download the CTNNB1 app!

This app is your central hub for **connection, support, and information-sharing** within the CTNNB1 community.

Available in **English, Deutsch, Español, Français, Italiano, Português, Slovenščina,** and **Polski**.

Through the app, you can:

- Stay up to date with the latest news and research
- Discover community events and meetups
- Join support group discussions
- Access educational resources
- Get informational support through a CTNNB1-focused AI assistant

Download for Android

Download for iOS



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