

# Monthly Newsletter

June 2026

CTNNB1 FOUNDATION

Issue #12

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## Dear CTNNB1 community,

As we prepare to gather once again for our annual conference, this month's newsletter is filled with exciting updates from across our global community. From major research milestones and clinical trial progress to preparations for the upcoming conference in Barcelona, there is much to celebrate and look forward to.

Inside, you'll find highlights from recent scientific meetings, important conference information, updates on our Natural History Study and clinical trial programs, and opportunities to connect with fellow families, researchers, and clinicians. We also share ways you can help support the future of CTNNB1 research and ensure that our work continues to move forward.

Thank you for being part of this journey. Every family, researcher, clinician, donor, and supporter plays an important role in bringing us closer to a better future for individuals living with CTNNB1 syndrome.

We look forward to seeing many of you in Barcelona very soon.

*The CTNNB1 Team*

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## Community News

### **ASGCT 2026**

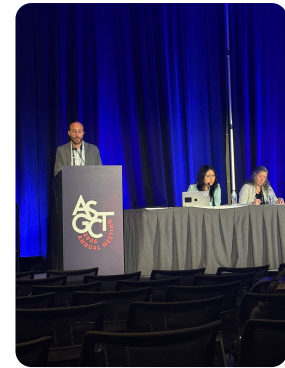
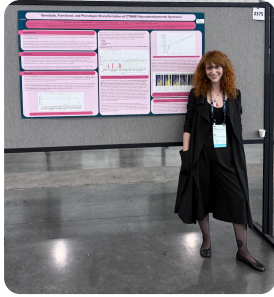
In May, members of the CTNNB1 Foundation team traveled to Boston, USA, to attend the [American Society of Gene & Cell Therapy \(ASGCT\) Annual Meeting](#), one of the world's leading conferences in gene and cell therapy.

Our team delivered four oral presentations, presented by **Dr. Špela Mirošević**, **Prof. Damjan Osredkar**, and **Assoc. Prof. Duško Lainšček**, alongside three scientific poster presentations highlighting ongoing CTNNB1 research (also presented by **Dr. Mirošević**, **Prof. Damjan Osredkar**, and **Nina Žakelj**).

For a small team from a small country, it was an incredible honor to share our work on such a prestigious international stage.

The conference provided a valuable opportunity to raise awareness of CTNNB1 syndrome, showcase the progress being made toward developing treatments, and connect with researchers, clinicians, industry leaders, and advocates from around the world. 🙌

We are proud of what our team accomplished and look forward to hopefully sharing even more progress and discoveries at next year's ASGCT meeting in Philadelphia.



### **NHS Clinical Examinations in Boston**

We are pleased to share that we have successfully completed this year's round of Dragonfly Natural History Study clinical examinations in Boston.

It was wonderful to reconnect with many familiar faces and welcome new participants into our growing community. Seeing families come together from across the United States and beyond continues to remind us how strong and connected the CTNNB1 community has become.

Thank you to all the families who participated and helped make these examinations a success.



## !! Final Conference Updates

The **4th International CTNNB1 Syndrome Conference** is now fully booked, and registration has officially closed.

See the final conference program [here](#).

We are excited to welcome families, researchers, clinicians, and industry partners from around the world to Barcelona later this month. We have also prepared a few special surprises for attendees, including some conference merchandise. 👁️ Quantities are limited this year as we test interest, but if you like what you see, we hope to expand the collection in the future.

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### 🍴 Conference Dinner

On the **evening of June 18**, following the first day of the conference, we will host a community dinner for families, researchers, clinicians, and supporters attending the event.

This informal gathering is a wonderful opportunity to connect, share experiences, and strengthen relationships within the CTNNB1 community.

**Dinner tickets are still available**, although spaces are limited.

Please note:

- One ticket is required per adult attendee.
- Children may attend free of charge.

Purchase tickets [here](#).

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## **Submit Questions in Advance for the Family–Doctor Round Table**

At the conclusion of the first conference day, families and caregivers will have the opportunity to participate in an open **"Ask Me Anything" round table discussion** with a panel of clinicians experienced in CTNNB1 syndrome.

To help us organize the session and address the topics most important to families, we kindly ask that questions be submitted in advance.

The discussion will focus on:

- Clinical characteristics of CTNNB1 syndrome
- Symptoms and disease progression
- Medical management and care
- Everyday clinical challenges

Please note that **questions related to the GAIN clinical trial will not be addressed during this session.**

Submit your questions [here](#).

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## **Hotel Accommodation – Hotel SB Diagonal Zero Barcelona**

If you have not yet arranged accommodation, rooms are still available at Hotel SB Diagonal Zero Barcelona, where both the NHS examinations and the CTNNB1 conference will take place.

Participants must enter the following code on the [hotel website](#) to receive the discounted reservation rate: **D4-CTNNB26**

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## **Experience Barcelona**

We are delighted to share that [Moco Museum Barcelona](#) is offering conference attendees an exclusive **2-for-1 ticket promotion**.

Use code: **2x1CTNNB1**

The code is **valid from June 12 through June 28** and may be used both online and at the museum ticket desk. When purchasing tickets on-site, attendees should present proof of conference registration or accreditation.

Moco Museum also offers:

- Private guided tours for groups

- Exclusive experiences upon request, including refreshments and special arrangements

We hope this provides a fun and memorable activity for families visiting Barcelona.

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## Research & Clinical Trial Updates


### Dragonfly NHS Updates

Examination schedules for the upcoming assessments at **Hotel SB Diagonal Zero Barcelona** have now been distributed.

Families can locate their child's participant ID within the schedule and review their assigned examination times. 

The schedule has been carefully developed based on the travel information previously provided. Due to the complexity of coordinating assessments across multiple disciplines and limited staff availability, we are unfortunately unable to accommodate major scheduling changes. Exceptions may be considered only in cases of significant travel changes, such as modified flight arrangements or delays.

**! Room locations for individual assessments will be communicated as soon as they are confirmed by the hotel.**

A final version of the schedule will be distributed shortly before examinations begin (expect an email on the weekend of June 13-14). 

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### GAIN Clinical Trial Updates

We share an update from Mafalda's parents 4 weeks after her gene therapy treatment:

Mafalda is doing well after surgery and continues to recover. 🍊 Overall, she is in good spirits, but the immunosuppressive medication, especially prednisolone, has brought some real challenges.

Her sleep has been up and down, and her behaviour can change very quickly, with intense tantrums and very little patience when things don't go her way. We know this is most likely related to the medication, so for now we are taking it day by day and doing our best to endure this part of the journey.

At the same time, we have already noticed something that feels very meaningful to us. Mafalda's hands and fingers feel much softer and less tense than before. It may still be too early to say what this means, but for us, even these small changes feel incredibly hopeful.

She also lost a tooth while at the hospital for exams, which gave us a little unexpected moment in the middle of everything. We suddenly saw blood on the bed and worried she had hurt herself — only to realise there was now a little gap in her smile. 🦷

So, overall, Mafalda is doing well. There are difficult moments, especially with sleep and behaviour, but we are also seeing small signs that give us hope and remind us why this journey matters so much. 🧡

## !! Help our next gene therapy candidate

Harper has been selected as the **third child in the world** to participate in the CTNNB1 gene therapy clinical trial.

At just 8 years old, she faces the daily challenges that so many of our children know all too well—communication difficulties, mobility challenges, developmental delays, and countless hours of therapy. **Yet Harper continues to meet each day with courage, determination, and hope.**

Her family is now fundraising to cover costs associated with participation in the trial, including travel, accomodation, rehabilitation, follow-up assessments, and other costs over the coming years. Their goal is to raise funds to ensure Harper can complete this historic journey.

But this is about more than one child.

Every family who participates in this trial is helping write the future of treatment for CTNNB1 syndrome. Every assessment, every follow-up visit, every piece of data brings us closer to understanding how gene therapy can transform the lives of our children. Harper's participation will contribute to knowledge that could benefit children across our entire community for years to come.

We have always said that no family should have to walk this journey alone.

**Now it is our turn to stand beside Harper and her family.**

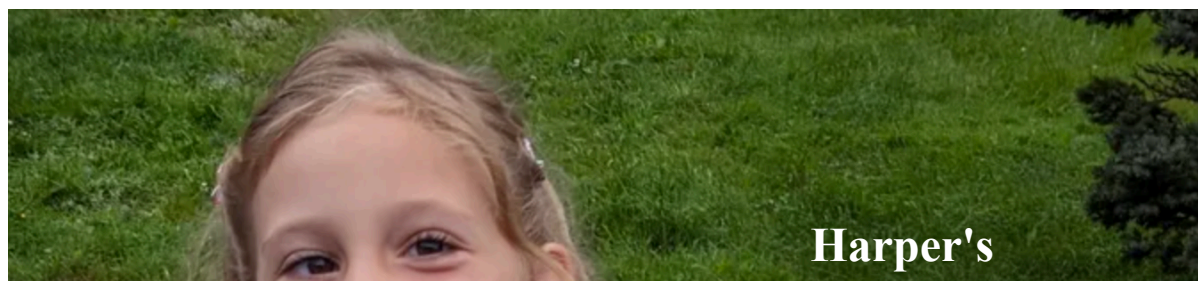
Whether you can donate \$5, \$50, or simply share her story with your network, every action matters. The strength of our community has never come from any single family—it comes from thousands of people choosing to help one another.

Let's show Harper's family what the CTNNB1 community can do when we come together.

Let's help the third child in this trial take her place in history.

Let's help create a future where every child with CTNNB1 syndrome has access to treatment.

Please donate, share, and help us spread Harper's story.



Donate here

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## Fundraising Updates

### ♥ Support the Future of CTNNB1 Research

Over the past few years, together we have achieved what many thought was impossible. We have built a global community, launched the Dragonfly Natural History Study, organized international conferences, developed the world's first gene therapy for CTNNB1 syndrome, and treated the first children in a clinical trial.

But none of this happened by chance.

Behind every research study, clinical examination, family conference, educational resource, and treatment milestone is a small team working tirelessly to keep everything moving forward. As a non-profit organization, we do our best to fund these activities through grants, sponsorships, and partnerships.

However, the reality is that advancing research, supporting families, organizing international events, and maintaining clinical programs requires substantial resources.

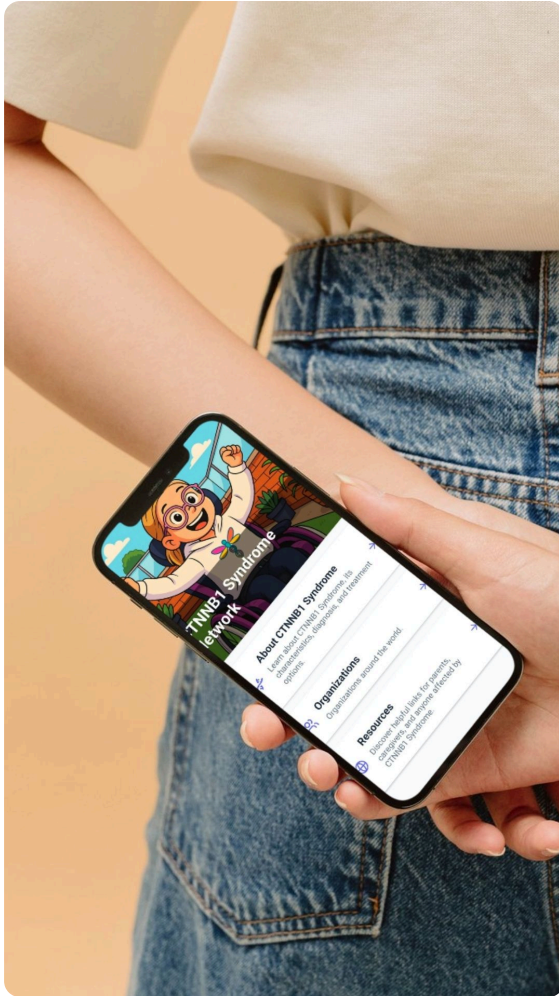
Today, we need your help more than ever.

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](mailto:contact@ctnnb1-foundation.org)

Donate



## 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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