

Monthly Newsletter

December, 2025

CTNNB1 FOUNDATION

Issue #8

Dear CTNNB1 community,

As we enter the heart of the holiday season, we hope you're able to slow down, find moments of warmth, and enjoy time with the people you love. This is a busy and meaningful month for our community, and we are grateful for every family, researcher, clinician, and supporter who continues to walk this journey with us.

In this edition, we share reminders for families in the Dragonfly NHS regarding clinical examinations, along with fundraising news. You'll also find research and clinical milestones, and updates on clinical trials. Additionally, we provide details on the now-open clinical trial enrollment process and a short statement on the process of selecting clinical trial participants.

As we close out the year, we feel deep gratitude for this community and the shared hope driving every step forward. Thank you for your trust, your patience, and your unwavering support.

Wishing you a peaceful holiday season and a bright start to the new year. 🌲 ✨

Best,
The CTNNB1 Team

Community News

Clinical Examinations

For families participating in the Dragonfly NHS study: If you have not done so yet, please [complete this form](#) indicating where you would prefer to have your examination. This will help us plan the examinations more efficiently.

If you have already submitted the form, please do not fill it out again to avoid duplicate responses. Thank you!

Research & Clinical Trial Updates

QP Release

We are pleased to share that the first batch of URBAGEN has successfully received QP release. The doses for the first two patients have already been safely delivered to the clinical pharmacy, marking an important milestone as we move closer to initiating treatment.

Clinical Trial

Our first patient is scheduled to receive treatment in the middle of December. We have high hopes for the outcomes and are grateful to have an exceptionally dedicated and experienced team of clinicians and support staff who will be supporting this clinical trial at the University Children's Hospital, University Medical Centre Ljubljana. We wish our first participant the very best - may the procedure go smoothly and the recovery be safe and peaceful. ❤️

Registration for CT

Enrollment for the clinical trial is now officially open, and eligible patients are invited to apply. You can find all details on the newly added [Clinical Trials section](#) of our website, where we provide comprehensive information about the study, eligibility, procedures, and next steps.

To enroll, please first contact gain@ctnbn1-foundation.org. You will then receive an official application form to complete.

Process of Selecting Participants in the GAIN Clinical Trial

The first four participants in the GAIN clinical trial have now been selected. For this initial cohort, the medical team recommended sending the pre-screening survey only to families who took part in the Dragonfly Study. This approach allowed the team to begin with patients they already knew, which helps ensure safety in the very first phase of a first-in-human trial.

Participants were chosen based on several criteria. One of the key factors was **clinical trial readiness** - how prepared a family is if complications arise. Eligibility and age (6-12 years) were also essential. The medical team decided to prioritise children closest to six years old. This is the age group that most closely matches the animals used in pre-clinical safety studies and is considered the safest population in which to begin dosing.

The medical team then conducted interviews with the shortlisted families, and an independent ethics committee reviewed all applications. This committee, which is working **entirely separately from the clinical investigators, the Sponsor, and families**, has selected the first four children and determined their order. Their decisions were based strictly on **eligibility, safety considerations, and documented conversations with families about their readiness to participate**.

Clinical trial applications remain open. The medical team will select the next four participants in **March-April 2026**, and this cohort will be open to any child aged 2-12 years. Inclusion of these additional patients is expected between September 2026 and March 2027.

We are with you, rooting for every child to be given this chance. At the same time, we are exploring options and are hoping to expand the clinical trial in Australia, Spain, Brasil and the US in 2026!

Fundraising Updates

Fundraiser Update: Help Us Cross the Finish Line

It's the season of giving, and once again, your generosity shines. Since our last update, we've raised an additional **2,100€** on Givebutter!

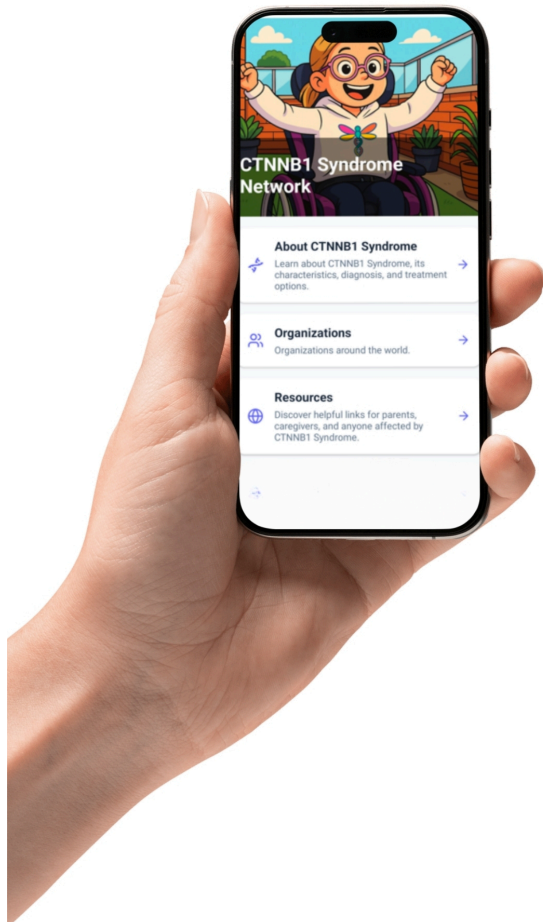
A heartfelt thank you to the Spanish association Asociación CTNNB1 España for their extraordinary **60,000€** contribution and for standing with us every step of the way.

We now still need 338,809 € to reach our goal of 500,000 €. The remaining unpaid amount includes 97,000 € for the 2024/2025 work at the Children's Medical Research Institute, as well as 103,430 € for QP release, storage and distribution, and the remaining clinical trial expenses for each patient.

As you know, we are running this clinical trial without any pharmaceutical or investor partner, which means the financial responsibility falls entirely on us. We are actively seeking funding partners to help move this program forward and ease the burden on families, and we are applying for grants to cover part of the remaining costs.

🌟 **At this moment, funding is still critical. So we kindly ask you to help us support this program and our children.**

Donate on Givebutter



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
- Connect with experts through live chat assistance

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