



2024 IMPACT REPORT

CTNNB1 Connect and Cure is a nonprofit organization run by parents of children diagnosed with CTNNB1 Syndrome.

Our vision is a world with safe and effective treatments and cures for all patients with CTNNB1 Syndrome.

Our mission is to find treatments and a cure for CTNNB1 Syndrome while raising awareness and connecting families.



WWW.CURECTNNB1.ORG



Dear friends,

2024 marked 7 years since the first patient advocacy organization for CTNNB1 Syndrome and 5 years since we started funding research for treatments and a cure.

As you will read in the pages of this report, CTNNB1 Connect and Cure is driving incredible progress.

This year, we experienced immense organizational growth and refined our internal governance to ensure our actions speak to the voice of the patient community. My main goals coming in as the new President were increasing transparency, building collaboration, and ensuring sustainability. I am so proud of the steps we have taken in these areas and am so excited for the projects we have planned for 2025.

Our progress never feels fast enough and there is so much more to do. We are grateful for your investment in our big goals to build a better future for everyone affected by CTNNB1 Syndrome.

With Gratitude,

Emily Amerson

*President, CTNNB1 Connect and Cure
Mom to Savannah*



TOP HIGHLIGHTS



CTNNB1 Connect and Cure was invited to participate in our first Million Dollar Bike Ride, raising \$33,312 and receiving an additional \$30,000 in matching funds for research from the Orphan Disease Center/Penn Medicine. This grant was awarded to Jeff Collier at Johns Hopkins University for mRNA Therapeutic Development.



Dr. Michele Jacob from Tufts University and Dr. Michel Weiwer from the Broad Institute received a Prestigious NIH Collaboration from the National Center for Advancing Translational Sciences (NCATS) to optimize a lead compound into a drug candidate for human clinical trials.



CTNNB1 Connect and Cure has launched a comprehensive Drug Repurposing Project aimed at identifying existing medications with the potential to treat CTNNB1 Syndrome. This initiative will complement ongoing efforts in novel therapeutic development, creating a multifaceted approach to maximize our chances of finding safe and effective treatment options for all CTNNB1 patients as soon as possible.



BY THE NUMBERS

61.7K

Social Media
accounts
reached

273

Patients
participating in
research

13

Grants applied for
(7 awarded)

8

Conferences
attended

3

CTNNB1 research
grants funded

2

New publications



COMMUNITY

In 2024, CTNNB1 Connect and Cure continued to place a strong emphasis on building a supportive and connected community through **newly diagnosed resources**, our **family connections database**, and much more.

1

Virtual SibShops

We launched a virtual SibShop program aimed at fostering a sense of belonging while equipping siblings with valuable knowledge and support. Thanks to the dedication of our Director of Engagement, Annie Wood, and our fantastic volunteer, Abby Turnwald,—both certified in SibShop facilitation—we successfully hosted seven engaging sessions, welcoming participants from various locations.

2

Family Meetup

In October, two dedicated CTNNB1 families hosted a memorable meetup at the Sam Noble Museum in Norman, Oklahoma. The gathering, organized by the Paes and Robinson families, offered a perfect blend of fellowship, fun, and shared experiences. Families enjoyed exploring the museum's exhibits, sharing meals, and creating lasting connections. This special event fostered a sense of unity and support among those brought together by their CTNNB1 journey.

3

Parent Webinars

To foster open communication between CTNNB1 Connect and Cure and the community, we introduced virtual parent webinars. These sessions provided a platform to inform parents about exciting ongoing projects, gather valuable feedback, and create a welcoming space for families to connect and engage with one another.



RESEARCH

In addition to supporting the **mRNA Therapeutic Program**, continuing the **GSK3 Inhibitor Program (now in collaboration with the NIH)**, and the launch of our brand new **Drug Repurposing Program**, CTNNB1 Connect and Cure has made several other significant strides in advancing research efforts at understanding CTNNB1 Syndrome and developing potential treatments.

1

Patient Participation

Research relies on patient participation, which is why CTNNB1 Connect and Cure is proud to announce that there are now: 273 CTNNB1 patients registered in SimonsSearchlight, 106 patients registered in Citizen Health, 34 patients registered in Boston Children's Hospital's in-person clinical assessments, 46 samples with SimonsSearchlight, 52 samples with COMBINEDBrain, and 13 participants in the TIGER Study.

2

Incredible Progress

In 2024, CTNNB1 Connect and Cure awarded over \$260,000 in research grants focused on treatment development, understanding disease progression, and identifying biomarkers for clinical trials. New patient-derived models were created to test treatments across multiple variants, ensuring broader treatment effectiveness. There are now 8 patient-derived iPSCs via SimonsSearchlight and 3 CTNNB1 mouse models available to researchers.

3

Building Capacity

One large focus in 2024 was growing our capacity to advance research and support families. By applying to grants, creating fundraising campaigns, recruiting more volunteers, and building direct connections with key stakeholders and collaborators, CTNNB1 Connect and Cure is on track for even more growth and progress in the coming year.



In 2024, CTNNB1 Connect and Cure **applied for an ICD-10 Code**, facilitated several **outreach projects**, and organized key initiatives to **spread awareness and educate the public** about CTNNB1 Syndrome.

EDUCATION & AWARENESS

1

CTNNB1 Awareness Day

On July 25, 2024, CTNNB1 Connect and Cure hosted the 5th annual CTNNB1 Awareness Day, a global event aimed at increasing recognition and understanding of CTNNB1 syndrome. Families, researchers, and advocates participated in local and virtual activities across multiple countries. Social media campaigns and online discussions reached over 500,000 people worldwide, helping to spread crucial information about the disorder.

2

Animated Videos Launch

To make scientific information about CTNNB1 syndrome more accessible, we produced a series of animated educational videos. These videos covered various topics such as our organization, therapeutic treatment updates, and genetic cerebral palsy. By using engaging visuals and simple language, these videos have become valuable resources for CTNNB1 families.

3

Engaging with the Larger Rare Disease Community

CTNNB1 Connect and Cure strengthened its presence in the rare disease community by participating in several rare disease conferences and educational courses. Some key highlights include COMBINEDBrain's Annual Meeting, where our President, Emily Amerson, received the "Rare Advocate Award," and Global Genes' Week in Rare, where our Board Member, Effie Parks, received her 3rd Champion in Advocacy nomination and our clinical partner, Dr. Wendy Chung, received the Champion in Health Equity award.

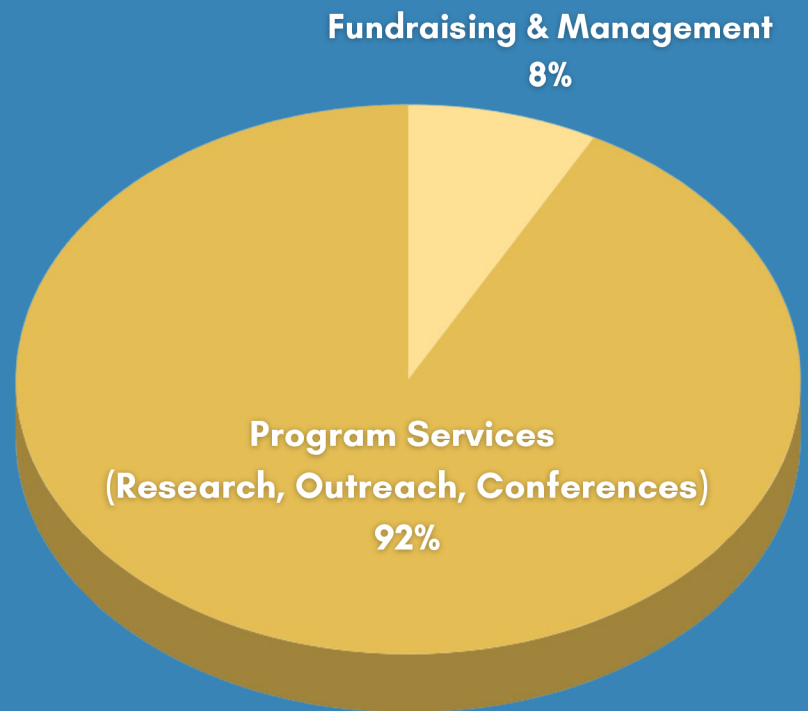


FINANCIALS & TRANSPARENCY

CTNNB1 Connect and Cure is dedicated to driving science toward new therapeutics and ultimately a cure for every person affected by CTNNB1 Syndrome. In 2024, we raised over \$329,000 (nearly 30% more than the prior year!) through grassroots fundraising, grants and sponsorships.

Our work would not be possible without the generous support of our donors. We are an all-volunteer board and donations support our research, family support, and advocacy programs. Thank you for joining us on our mission to a cure.

HOW FUNDS WERE SPENT



Platinum
Transparency
2024

Candid.

CTNNB1 Connect and Cure is proud to be a Platinum Level Participant on the GuideStar Exchange, demonstrating our commitment to financial transparency.



MEET OUR TEAM

Board and Leadership Team



Emily Amerson
President & CEO



Ashley Swift
Chief Communications Officer



Sean Cochran
Chief Financial Officer



Nicole Dashiell
Secretary



Effie Parks
Director of Partnerships



Annie Wood
Director of Engagement



Bruno Ramalho
Director of Technology



Lauren Cochran
Co-Director of Research



Alex Dashiell
Director of Finance



Lindsay Stevens
Director of Fundraising



Mariana Parks
President Emeritus, CSAW

Scientific Advisory Board



Wendy Chung
MD, PhD
Boston Children's Hospital



Gerald Cox
MD, PhD
Boston Children's Hospital



Jennifer Bain
MD, PhD
Columbia University



Drew Scoles
MD, PhD
Children's Hospital of Philadelphia



Rachel Heilmann
PharmD, BCPS

Scientific Consultants



George Stratigopoulos
PhD
Columbia University



Kellan Weston
PhD
COMBINEDBrain

Support Team

Allen Jun	Jill Haile
Anita Jones-Garza	Julie Anderson
Ashley Hamic	Kassaundra Pierson
Brenton McWilliams	Louis Anetrella
Caroline Kettering	Luke Dreckman
Crawford McWilliams	Natasha King
Dan Stevens	Nicole Fimple
Diana O'Mara	Paetyn Dreckman
Jennifer Hubert	Susanna Barnes
Jessica Norbut	Toni Paes
Jessica Robinson	



PARTNERS

Global CTNNB1 Patient Organizations



Rare Disease Partners



NORD®
National Organization
for Rare Disorders



Global Genes®
Allies in Rare Disease



**citizen
health®**



Tufts
UNIVERSITY



Epilepsies
Action Network



**Boston
Children's
Hospital**

COLUMBIA
UNIVERSITY



BROAD
INSTITUTE



JOHNS HOPKINS
UNIVERSITY



the
orphan
disease center

probably
genetic

SIMONS
SEARCHLIGHT



Project
FIND-OUT



COURAGEOUS
PARENTS
NETWORK

UW Medicine
DEPARTMENT OF PSYCHIATRY
AND BEHAVIORAL SCIENCES

WELLCAST
SUPPORTING WELLBEING OF
CAREGIVERS VIA TELEHEALTH



LOOKING AHEAD TO 2025

EVENTS

**Rare Disease Day
(February 28th)**

**2025 Conference in
Boston, MA
(July 10-12th)**

Family Meetup

**1st Annual Community-
Wide Fundraising Event**

RESEARCH

Disease Concept Model

Drug Repurposing Project

CTNNB1 Patient Priority Survey

Optimize small molecule drug with NIH

Continue development of mRNA therapeutic

Grow CTNNB1 Biorepository

Publish Proteomics Analysis

**Increase enrollment in patient-
driven research studies**

FDA Listening Session

SUPPORT

**Standards of Care
Launch a Centers of
Excellence Program**

**Regional Ambassadors
Program**

More educational resources

**Family connection
opportunities**

JOIN US



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Do you have skills or interests
you want to share? We have a
variety of projects for which
we could use your help!

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THANK YOU FOR YOUR CONTINUED SUPPORT



CTNNB1 Connect and Cure relies on the generous support of our donors to advance our mission. Whether you are making your first donation or are a major donor, we are honored to have your support. We can't do this work without it! Please make a donation to support the cause today!



