Open House

15 MAR 2025

Agenda

Welcome

Meet the Board

Key 2024 Focus Areas

2025 - Exciting Events Planned

Question and Answer

Closing

Meet the Board

2024 Strategic Planning: refresh Foundation organization

- Why we did this
  - At 5 years old, it was time to re-examine ways of working and chart a path for growth
  - Foundations this age risk getting stale and faltering without a periodic strategic "refresh"

- What we did, supported by a facilitator
  - We solicited community input on foundation strengths & weaknesses; community priorities
  - We met in person in March for all-day work session
  - Guided by community surveys and brainstorming, we re-imagined what we should be doing and how

- What we achieved
  - Strategic planning session helped us refocus our efforts
  - We defined three key "pillars": Communications, Community, Research
  - We identified two cross-cutting support functions: Fundraising, Administrative

2024 Strategic Planning: what we're doing going forward

- Budget and team is organized to the Pillars
  - Fundraising for specific Community and Research activities
  - Communications mounts campaigns explain purpose of fundraising efforts
  - Community and Research execute funded activities
  - Administrative defines budget and oversees operations

- Strategic planning also identified needs for
  - Paid staff: part-time accountant identified; Executive Director recruiting underway
  - Volunteers are also being sought and aligned to support major Pillar projects
  - Succession planning: volunteers ⇨ at-large board members ⇨ board officers

- Follow this link for the Strategic Plan

Communications Team

- Organized campaigns on major social channels
  - 508 Facebook followers
  - 1700 Friends and Family of FOXP1 Facebook members
  - 350 Instagram followers
  - 180 LinkedIn followers
- Website refresh https://www.foxp1.org
- **Family Meetups** - City, State or Region - share your plans with the Foundation and we will do our best to help with event funding - food or location expenses. KC group in the photo.
- Overview **information sheets** for families, caregivers, therapists for newly diagnosed individuals https://www.foxp1.org/copy-of-resources-1
- Continued our monthly newsletters and Zoom calls
- **Watch for an Interest survey on Foundation Programs and Resources**
- **Volunteers - We need to grow our Communications Team**

Global Community

Build a global community that empowers and supports families and individuals

- ➢ Census 2025 ~ 652 individuals with FOXP1 in 50 countries
- ➢ 24 Ambassadors from 20 countries are building a global FOXP1 Community
- ➢ Ambassadors and Regional Ambassadors organize local family gatherings,help parents understand the complexities and unknowns of this genetic disorder, and connect researchers with FOXP1 families,
- ➢ If you would like to support families in your country, or would like to connect with someone in your local area please reach out to those on the Ambassador’s webpage or email info@foxp1.org

Global Community

Build a global community that empowers and supports families and individuals

- How you can Connect with other families:
  - Monthly Zoom Chats with parents from around the globe ~ continue to be a success
  - Sibling and Grandparent Support Chats ~ are happening
  - Monthly Newsletter ~ Keeps you informed
  - FaceBook or WhatsApp Groups - different languages, English, French, German, Spanish, Italian, Portuguese
  - Attend the 2025 Conference in The Netherlands ~ Esther Kloosterman of The Netherlands

Global Community

How you can contribute to research:

Join the Foundation

~Be counted ~ Sign up

Rare-X Data Collection

~ Register ~ Upload genetic report ~ Answer survey questions

Citizen Health program

~ Register 5 minutes

FOXP1 Worldwide Research conference and Family Gathering 2025

June 19 – 21 2025

Location

AMSTERDAM area

IJMUIDEN

Netherlands

Europe

35 minutes/kilometers
from Airport Schiphol/AMS

40 minutes/kilometers
from Amsterdam City Centre

2 minutes walk from the beach

120 people registered for 21 FOXP1 family members and still counting!

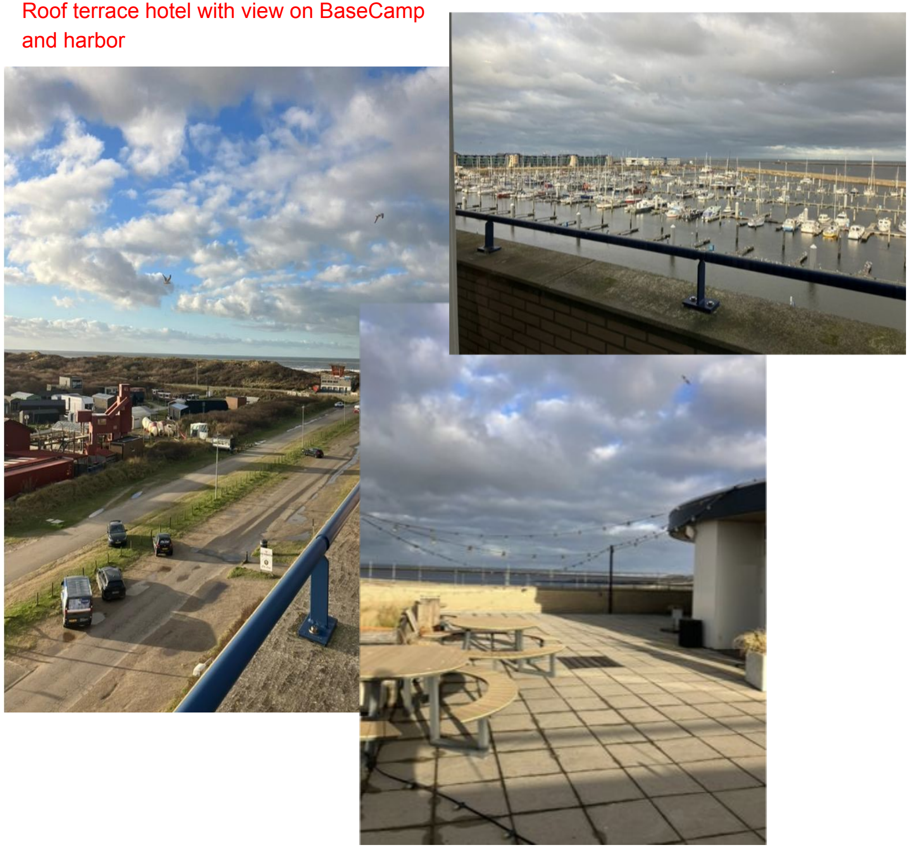
FOXP1 Worldwide Research conference and Family Gathering 2025

Thursday June 19	**Research Conference**	09.00	Research conference with different speakers on FOXP1 related research topics
		17.00	End of research conference
	Family Gathering	16.00	Arrival and meet & greet Hotel Leonardo topfloor
		18.00	Dinner at Hotel Leonardo topfloor researchers and families
Friday June 20	**Family Gathering**	08.45	Drop off of kids at Basecamp where a group of 20 caretakers will be waiting to give them a beautiful morning
		09.00	Morning conference for adults featuring key speakers discussing various FOXP1 research topics in hotel Leonardo
		12.00	Lunch for everyone (adults, caretakers, kids, speakers) in the hotel
		13.15	Bring back the kids to Basecamp for a fun afternoon
		13.3	Breakout sessions on different symptoms, languages and interests, moderated by a specialist on the topic in the hotel
		18.00	Dinner in hotel Leonardo
		20.00	Drinks and Bites for everyone who wants to at Basecamp until …!
Saturday June 21	**Family Gathering**	10.30	Walk on the beach, after-talks/sharing thoughts over morning coffee
		12.00	End of conference (leaving with a lot of Foxy insights)

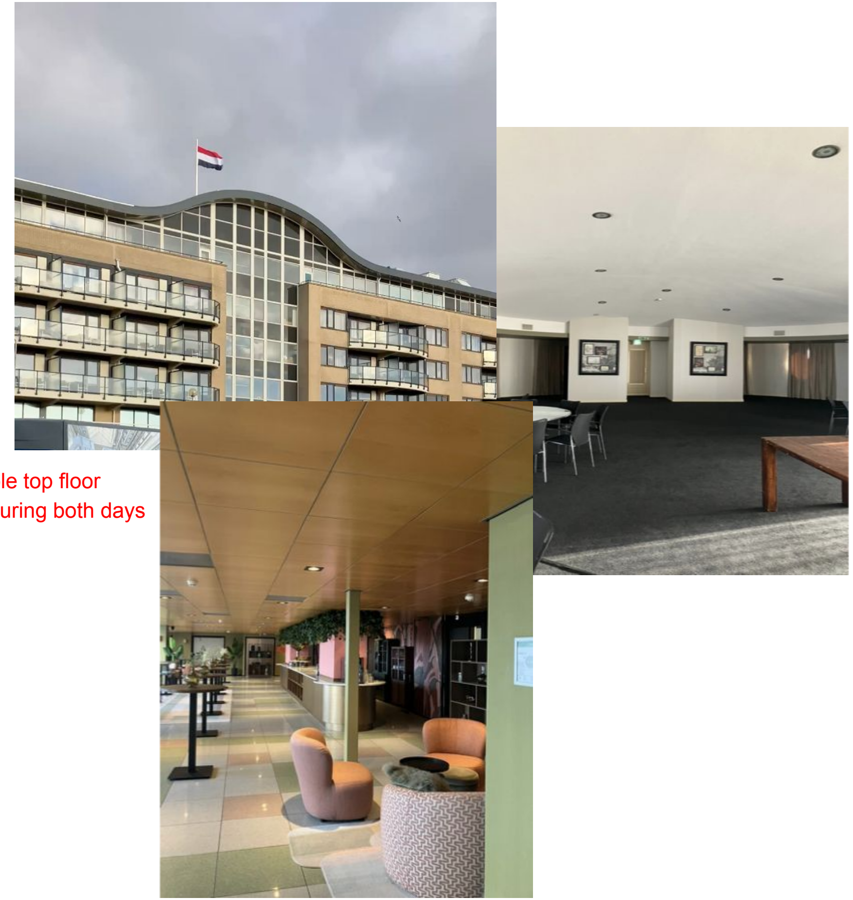
FOXP1 Worldwide Research conference and Family Gathering 2025

Research conference June 19 and Family Gathering June 20 in Hotel Leonardo (topfloor)

Roof terrace hotel with view on BaseCamp and harbor



The whole top floor is ours during both days



FOXP1 Worldwide Research conference and Family Gathering 2025

Activities children and young adults in BaseCamp

Playground next to Refter on BaseCamp

Refter in BaseCamp for kids activities

citizen health® X INTERNATIONAL FOXP1 FOUNDATION

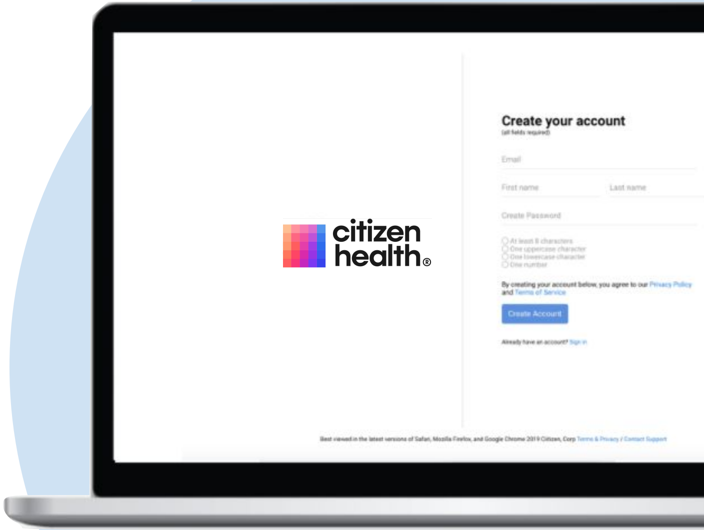
Join the FOXP1 community on Citizen

International FOXP1 Foundation has partnered with Citizen Health to help patients and families use their health data to improve care and drive groundbreaking treatments.

What is Citizen Health?

- Citizen Health is a platform that enables patients with rare conditions to get access to all their medical data in one place and advance critical research efforts with minimal effort. Working with over 80 advocacy partners and 12,000 patients, Citizen has helped enable rare disease patients to use their own health data to improve care and accelerate groundbreaking treatments

Empowering patients to drive their care and research through their own health data



Create your account
(all fields required)

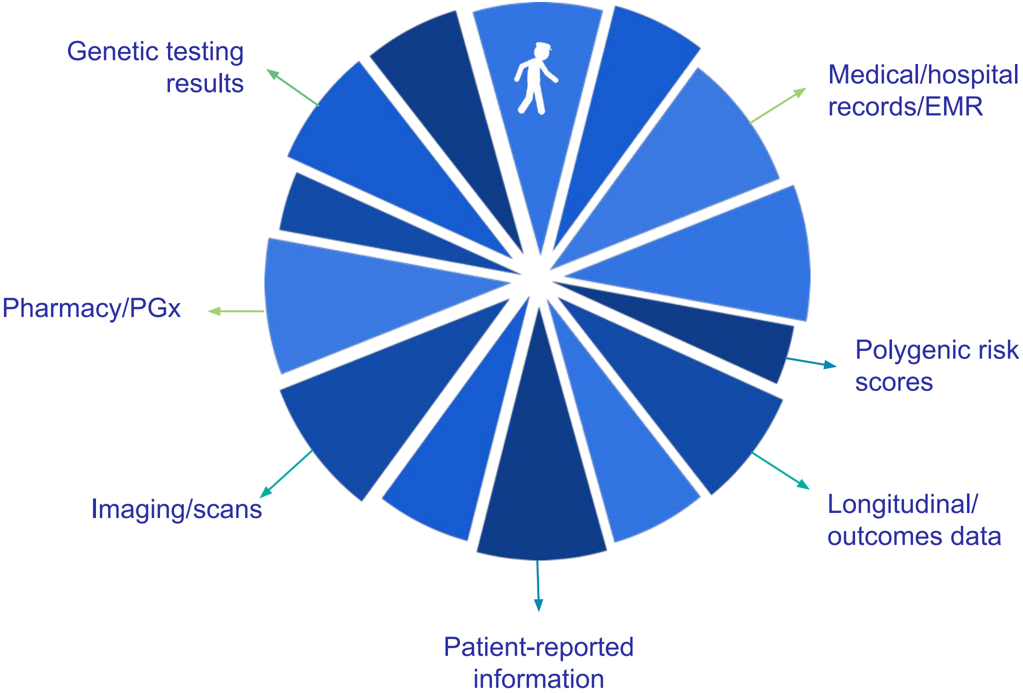
○ At least 8 characters
○ One uppercase character
○ One lowercase character
○ One number

By creating your account below, you agree to our Privacy Policy and Terms of Service

Already have an account? Sign in

Best viewed in the latest versions of Safari, Mozilla Firefox, and Google Chrome 2019 Citizen, Corp Terms & Privacy / Contact Support

Valuable patient data is trapped



There is immense value in the medical record, but those are trapped in various institutions

There is no single longitudinal view of the patient's care

Difficult to get access to all of your medical records to help inform your own care

How do we solve this?

1) Create an account: Simple, five-minute onboarding process
   - Join our community on Citizen Health

2) Citizen will collect your medical data from your providers and give you access to your data in your **secure, digital** profile

3) **Choose** what to do with your data:
   - Share information with your care team to help inform your healthcare decisions
   - Share your **de-identified data** stripped of any personal identifiers to help power research and advance cures

Benefits to you

- ✓ Get access to all your medical records in one place
- ✓ Easily power and accelerate research efforts without leaving your home
- ✓ Gain more control and understanding of health
- ✓ Support your community in their vital step to build natural history studies
- ✓ **Five minutes, one sign up - we do the rest**

citizen health®

Is my data safe?

Privacy and Security with Citizen Health

✓ **Secure, Encrypted, and HIPAA-Compliant Systems**
Citizen Health uses HIPAA-compliant cloud services, including Amazon Web Services (AWS), to securely maintain patient information. All data is encrypted locally and on AWS, a platform trusted worldwide for its commitment to data security.

✓ **Privacy Protection with Minimal Data Sharing**
Citizen Health follows the HIPAA Privacy Rule's Expert Determination process to minimize the risk of patient re-identification in data shared with research partners. Studies meet strict IRB requirements, with tightly controlled access to all data.

✓ **Rigorous Controls and Training**
Citizen Health complies with stringent protocols, including Title 21 CFR Part 11. Team members undergo regular training on security policies to ensure patient data is protected at all times. Data is never shared with third parties without explicit patient consent.

Why should you sign-up? – Community Benefits

Research

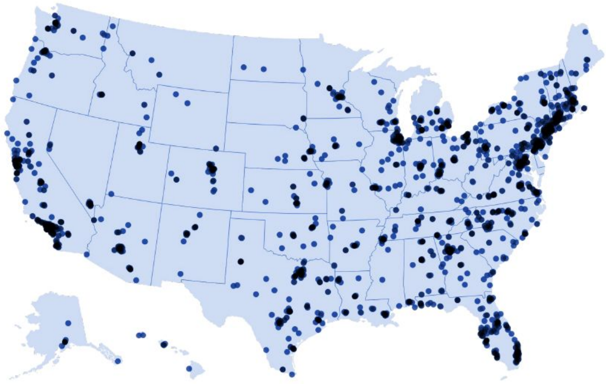
De-identified data can accelerate research into a cure for FOXP1 Syndrome

Citizen enables patient access to novel therapies, **faster**

How does it work?

OPTIONAL

Collect
EHR



Map generated using Datawrapper[1]

- Established relationships with over **4,000** unique health care institutions across the United States
- Launching international onboarding this year, focused on genetic and patient reports
- Records populated in **30-60 days**
- Health records collected from multiple providers at multiple institutes to follow patient journey
    - Includes all electronic health records (imaging, recordings, physician notes)

Why does this matter?

Citizen enables patient access to novel therapies, **faster**

- Recent partnership with Praxis resulted in NHS within 6 months
- FDA approved use of Citizen generated NHS to completely replace in-person NHS for first-ever time in rare diseases and granted Investigational New Drug clearance
- Paved the way for other rare cohorts to put their RWD to use to compliment and accelerate drug development efforts
- Reduced time to treatment by **5-10 years** for individuals living with *SCN2A*-related disorders


power research
find cures
proud
partner
2025
citizen
health®

citizen
health®

Connecting patients.
Accelerating cures.

Join the community
at citizen.health

Help us reach our goal:

50 sign-ups in 50 days

www.citizen.health/partners/foxp1

Join us for live sign-up event with Citizen on Sunday, April 27th

mcowen@foxp1.org

Goals of Research

To accelerate the development of effective targeted treatments for individuals with FOXP1 Syndrome.

- Hosting research conferences (alternating stand-alone, and in conjunction with family meeting).
- **Direct funding of research projects.**
- Outreach to companies and FDA.
- Working together with community pillar on building robust patient data (RARE-X, Citizen, Census).

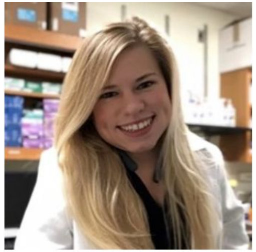

David Amaral
UC-Davis


Cora Taylor
Geisinger


Siddharth Srivastava
Harvard


Paige Siper
Seaver


Genevieve Konopka
UTSW


Jennifer Klein
Odylia Therapeutics


Gudrun Rappold
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Annette Schenck
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Simon Fisher
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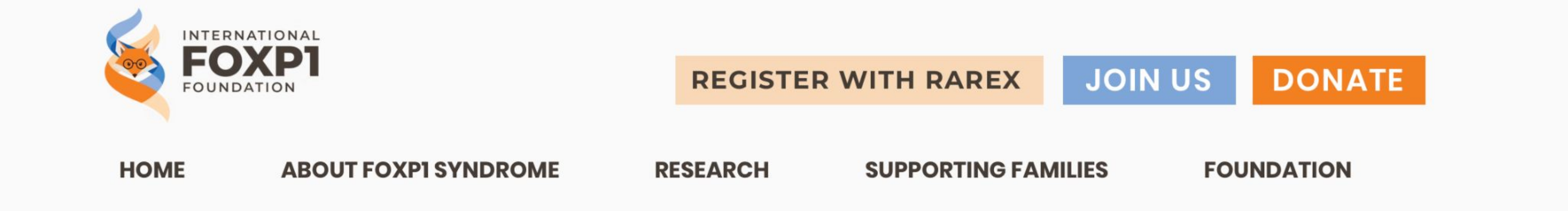
Jacqueline Crawley
UC-Davis


Jozef Gécz
University of Adelaide

Scientific Advisory Board of the International FOXP1 Foundation

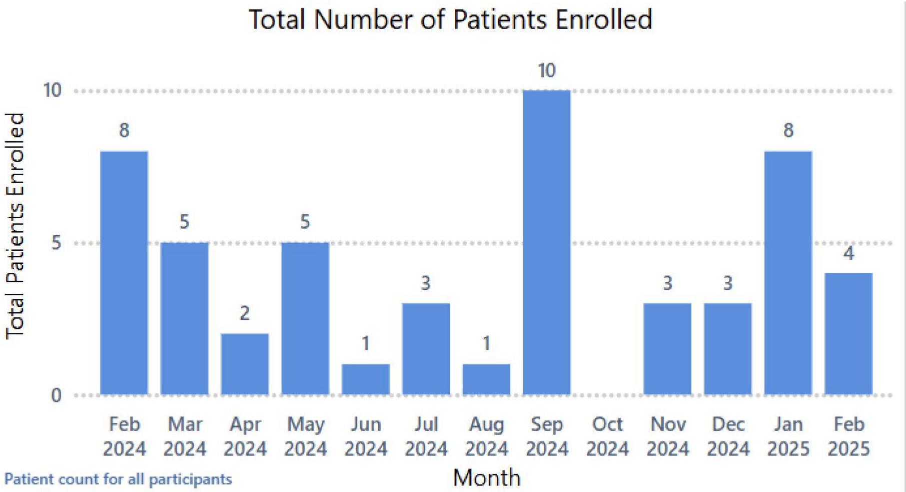
RAREX
A Research Program of Global Genes

To sign up, visit foxp1.org:



Thank you to everyone who signed up and participated in surveys about your loved ones with FOXP1 syndrome!

Country	Patient Count	Percent of Global Patients
United States	118	49.2%
United Kingdom	21	8.8%
Australia	14	5.8%
Brazil	14	5.8%
Germany	12	5.0%
Netherlands	10	4.2%
Italy	7	2.9%
Canada	6	2.5%
France	6	2.5%
Spain	5	2.1%
Denmark	4	1.7%
Switzerland	4	1.7%
Argentina	2	0.8%
Belgium	2	0.8%
Cyprus	2	0.8%
Ireland	2	0.8%
Israel	2	0.8%
Portugal	2	0.8%
Austria	1	0.4%
Mexico	1	0.4%
Norway	1	0.4%
Romania	1	0.4%
Serbia	1	0.4%
Slovenia	1	0.4%
South Africa	1	0.4%
Total	240	100.0%



Total Number of Patients Enrolled

Patient count for all participants

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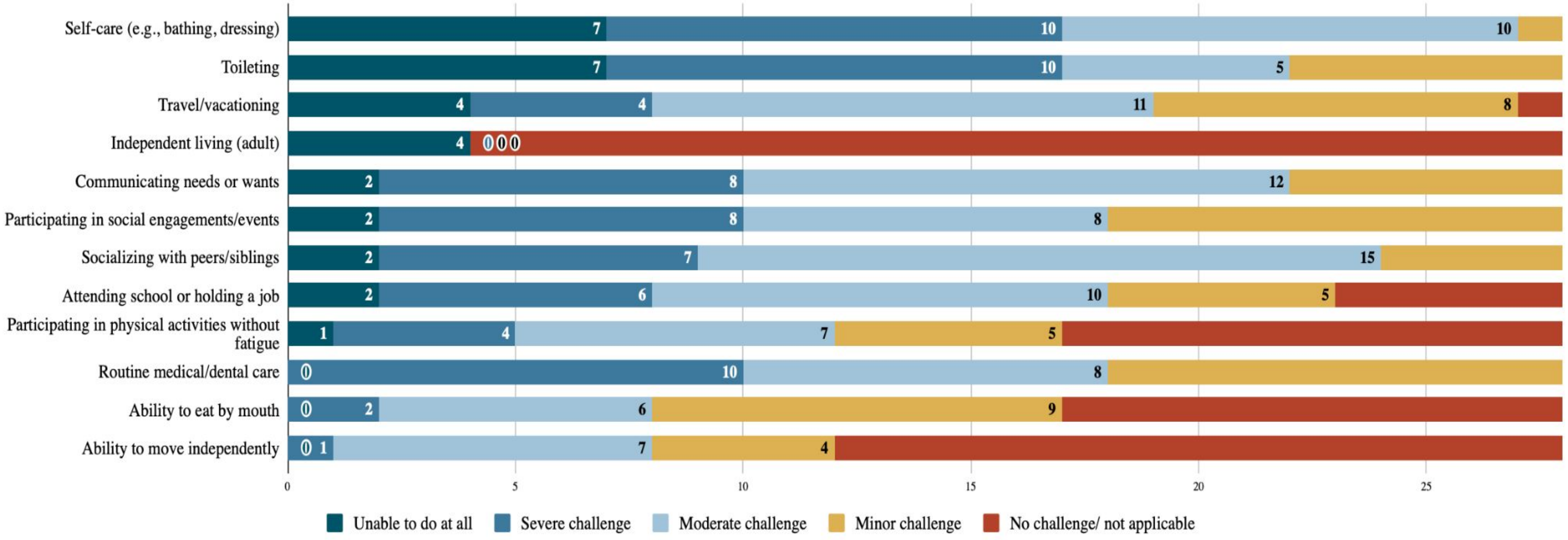
RARE-X Burden of Illness Survey


Burden of Illness Activity Challenges Reported by 34 FOXP1 Patients under 9 years old

RARE-X team is working with International FOXP1 Foundation and leading FOXP1 researchers to write a journal article based on this data.

RARE-X

- Voice of the patient—critical data for the community, researchers, FDA regulators, and pharma companies.

- Can eventually inform clinical trial design.

- **Essential to upload genetics report.**

- RARE-X team working to “map” FOXP1 and better understand correlations between particular mutations and outcomes.

2024 Activities

- Hosted research conference at Duke on FOXP1 Syndrome. (Over 30 researchers + 4 family presentations.)
- Funded 4 research projects.
- Released Request for Proposals, received and reviewed proposals.
- Began discussions with Mahzi Therapeutics about ASO treatment.

2024 Research Projects Funded ($274,000)

- Dr. Joe Buxbaum, Seaver Autism Institute
  Development of FOXP1 organoids for drug screening.
- Dr. Genevieve Konopka and Dr. Jay Gibson, UTSW Medical School
  Mouse experiment testing gene therapy treatment of FOXP1 syndrome.
- Dr. Gudrun Rappold and Dr. Henning Frohlich
  Testing PDE10A inhibitor on FOXP1 organoids.
- Dr. Paige Siper, Seaver Autism Institute
  Development of CGI scale for FOXP1 Syndrome.

2025 RFP projects selected ($296,000 over 2 years)

- Dr. Yann Herault, Institute of Genetics, Cellular, and Molecular Biology
  Development of a FOXP1 mouse where the gene can be corrected after birth.
  Goal is to demonstrate that FOXP1 can be treated.

- Dr. Bennett Novitch, UCLA
  Development of network of FOXP1 organoids to understand effects on the brain and possible treatments.

- Dr. Rujuta Wilson and Dr. Catherine Lord, UCLA
  Neurodevelopmental phenotyping in FOXP1 Syndrome: moving toward clinical trial readiness

More Goals for 2025

- Develop basic science behind ASO upregulation therapy with Mahzi Therapeutics ($150,000+).
- Show phenotype in FOXP1 neurons, which demonstrates that FOXP1 can be treated in adults ($90,000+).
- Continue to develop PDE10A inhibitor as possible small molecule treatment. (Support research and engage with pharma)
- Continue to develop AAV gene therapy.
- Continue to expand clinical trial readiness.

Be on the lookout!

Important meetings and events are announced on:

- Our monthly newsletter
- foxp1.org
- International FOXP1 Foundation Facebook page
- Friends and Family of FOXP1 Facebook page

Next up: **April 27, 2:00-3:30pm ET.** Zoom call with families, ***"Toward Clinical Trial Readiness."***

- Dr. Mara Cowen and Mequel Bunch on Citizen (Webinar and live sign-up demonstration)
- Dr. Paige Siper
- Dr. Rujuta Wilson and Dr. Catherine Lord

Other foundation infrastructure work for sustainability

- Established multiple reliably successful annual fundraising activities (eg, Annual Run-Walk)
- Secured international nonprofit certifications to enhance ability to attract and land grant monies by new grant team
- New donations system (GiveButter) for better donation experience, better reporting, automated end-of-year letters
- Sound balance sheet; as of end 2024:
  - General fund - $215,100
  - Research fund - $76,100
  - Number of donations last year - 453
  - Average donation size - $736.15
- 2025 saw first annual planning and budgeting process
  - As noted above, anticipate >$300,000 in revenue and program activities this year
  - Now have first paid staff (accountant; exec dir) - a huge milestone for the foundation!

All of this made possible by your consistent involvement and money/time support
THANK YOU!

Looking ahead - 2025 and beyond

- **Continue implementing priority actions from strategic planning session -** driving force
- Family Meetings Netherlands in June! 🇳🇱 It’s not too late to register! (click here)
- **“How to Plan a Fundraiser” campaign - featuring the Targon Family. Planning events can be one of the most successful ways to make a difference, spread awareness, educate and raise research funding**
- Strengthen ties within our community - Support Local Meet-Ups - Expand Peer Mentoring Programs such as our chat groups, local and regional ambassadors, educational and legislative advocacy efforts
- Focus on building our Citizen and RAREX - Data is critical
- Increase *Ask the Experts* program and foster our relationships with our Scientific Advisory Board members
- We strongly encourage you to consider Volunteering - Calling all friends, relatives and parents.

Question and Answer

We are all connected

Listen, learn, and share

Every effort counts