THE FA GCC GLOBAL CLINICAL CONSORTIUM

JULY 2025 NEWSLETTER

TABLE OF CONTENTS

FARA Friedreich's Ataxia Research Alliance

THE FA GCC GLOBAL CLINICAL CONSORTIUM

FA GCC SITE SPOTLIGHT

Our July spotlight is the RWTH Aachen University Team in Aachen, Germany

We are pleased to officially welcome Timo Reiß to the UNIFAI Study Management Team!

Timo holds a Ph.D. in Biology and brings extensive experience in clinical research, data management, and academic leadership, most recently at RWTH Aachen University. He will join the Neurology team at the Aachen site and will be taking over many of the study monitoring and data management responsibilities.

FA GCC management team representatives Cait Monette and Myriam Rai visited the RWTH Aachen University in June of 2025 to welcome Timo to the UNIFAI Study. Principal Investigators, Jörg B Schulz and Kathrin Reetz, gave the team an update on the RWTH Centralized BioMaterial Bank with a brand-new state-of-the-art automatic system and semi-automated storage options.

Left to right, **Aachen team in blue: Timo Reiß, PhD - FA GCC Monitor & Data Manager, Dr. rer. medic. Imis Dogan, Dipl.-Psych, Prof Kathrin Reetz - Investigator,** Cait Monette – FARA Director of Clinical Operations, **Ankica Grgic - Study Nurse Coordinator, Sara Schawohl, M.Sc. - Research Associate, Dorothea Peitz, M.Sc - Research Associate**

ANNOUNCMENTS

Transition of mFARS/ADL Scales to MAPI

To streamline the management of FARS, mFARS, FDS, ADL, and mFARS USS scales—including version control, standardization, translations, and licensing—FARA will transfer distribution and licensing responsibilities to MAPI Trust, effective June 2025.

- Ongoing studies (e.g., UNIFAI) are not affected and may continue using the scales as before.
- New studies and clinicians must request access through MAPI. For clinical trials, this is typically handled by the sponsor. Investigator-initiated research should also follow this process.
- The scales will remain free for unfunded academic users and clinicians. Funded users will pay a modest fee to cover administrative costs.

FARA has removed the scales from its website and now directs users to obtain them via MAPI.

FARA Clinician Scientist Development Award – LOI Due August 15

FARA is now accepting Letters of Intent for the Clinician Scientist Development Award, with a deadline of August 15. This award supports early-career physician faculty by providing protected time for mentored clinical or translational research in Friedreich's ataxia (FA), with the goal of fostering the next generation of clinician-scientists.

Award Highlights:

- Funding: $100,000/year for up to 2 years (direct costs)
- Focus: Patient-oriented or translational research aimed at improving diagnosis, treatment, or understanding of FA
- Eligibility: MD or MD/PhD within 7 years of completing residency (fellowship time excluded from this count)
- Not Eligible: Current/former PIs of NIH R-level or equivalent grants

Requirements:

- Mentor support letter and mentoring plan
- 50% effort commitment from the applicant
- Department Chair letter confirming protected time

Research areas may include natural history, clinical trials, biomarkers, disease mechanisms, and more. Projects supporting degrees like MSCI or MPH are encouraged.

Find out more here.
https://www.curefa.org/research/grant-program/grant-type/#scientist

THE FA GCC GLOBAL CLINICAL CONSORTIUM

UNIFAI NEWS

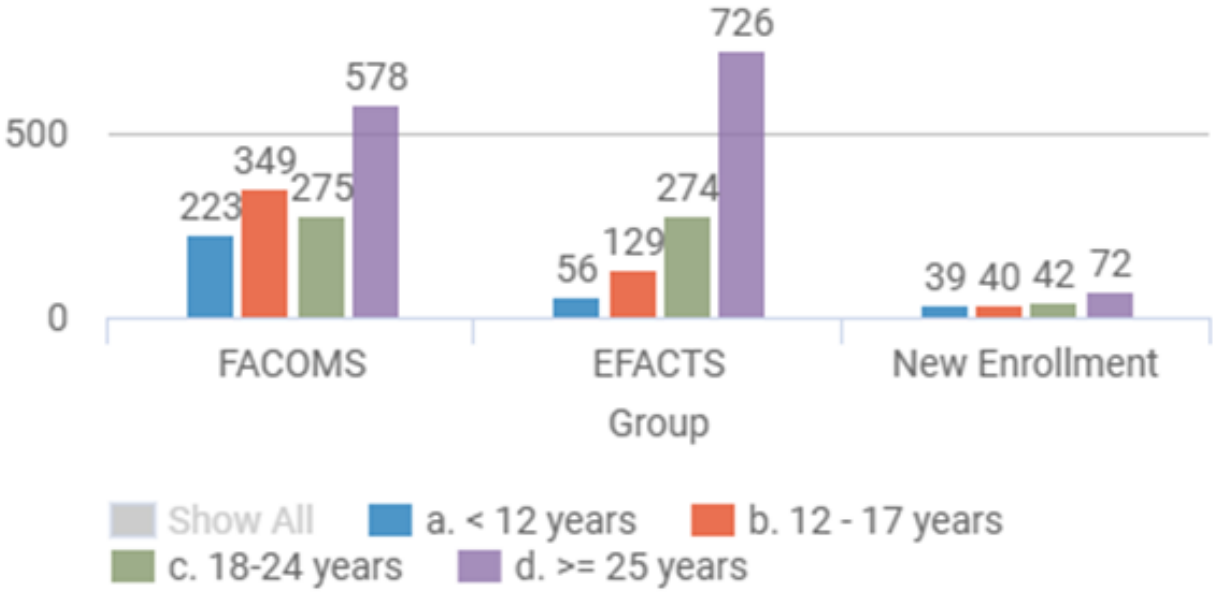
MILESTONE: First UNIFAI Patient Visit at Radboud

Dr. Bart van de Warrenburg shared that in May of 2025 his Nijmegen had completed their first UNIFAI visit! This is a milestone to celebrate for the site and for the UNIFAI effort. Thank you to Marike and the Radboud team for their contributions to FA Research!

Photo: (Back) Maaike Lamers - Study Coordinator and the Radboud Clinical Team; (front) 🐾 Vello, Marieke van Driel

Real-Time Dashboard Visualization

This dashboard, powered by Medrio's real-time reporting capabilities, presents a comparative visualization of participant age distribution across three study groups: FACOMS, EFACTS, and New Enrollment. The bar chart categorizes participants by age at the time of enrollment into four distinct groups: < 12 years, 12–17 years, 18–24 years, ≥ 25 years.
Each bar represents the number of participants within a specific age group for each study cohort.



UNIFAI Participant Age Distribution at Enrollment

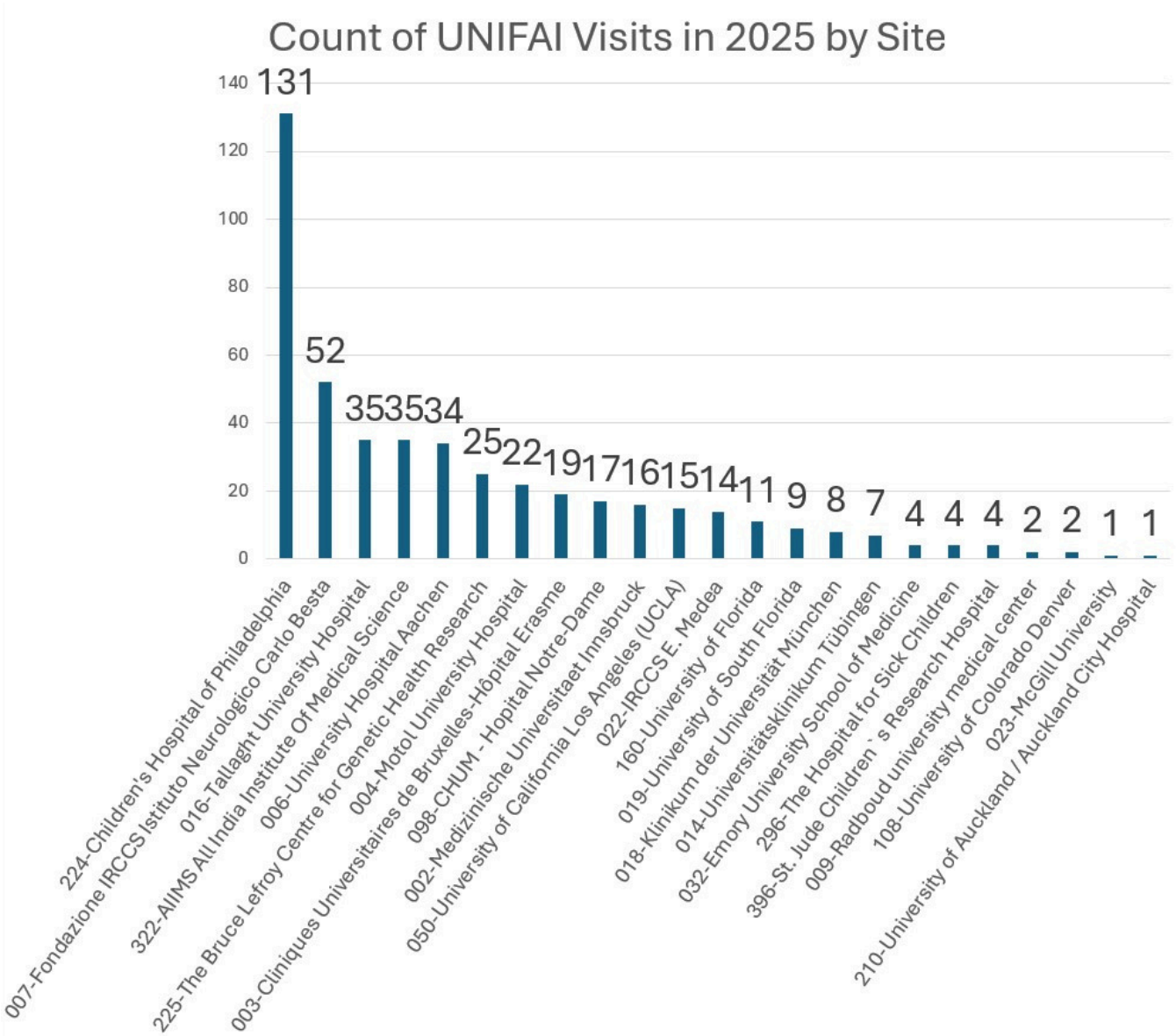
THE FA GCC GLOBAL CLINICAL CONSORTIUM

SITE TRANSITION STATUS

current as of 7/1/25

UNIFAI VISITS IN MEDRIO	1405
NEW ENROLLMENTS UNDER UNIFAI	124

IRB/ EC SUBMISSIONS	85%
IRB/ EC APPROVAL	83%
NEW LEGAL AGREEMENTS REPONSES RECIEVED	89%
FULLY EXECUTED NEW LEGAL AGREEMENTS	77%
ENROLLING UNDER UNIFAI	77%



PHARMA NEWS

Press Release and Community Statement: Biogen Initiates Phase 3 Pediatric Study of Omaveloxolone for the Treatment of Friedreich Ataxia | June 18, 2025
Read the full press release & community statement

Press Release: Larimar Therapeutics Announces FDA Recommendations on Safety Database, and Other Details of Nomlabofusp BLA Submission for Friedreich's Ataxia Program | June 23, 2025
Read the full press release

Press Release: Lexeo Therapeutics Announces FDA Breakthrough Therapy Designation for LX2006 in Friedreich Ataxia | July 7, 2025
Read the full press release and community statement

ENROLLING STUDIES

FARA posts information about clinical trials and studies on our Clinical Trial Finder. Here is a list of actively enrolling studies. Please consider sharing this information with any of your patients who are interested in participating in clinical research:

Clinical trials

- A Multiple Ascending Dose Study of DT-216P2 in Patients with Friedreich's Ataxia, Design Therapeutics
- A Study to Learn More About the Effects and Long-Term Safety of BIIB141 (Omaveloxolone) in Participants with Friedreich's Ataxia Aged 2 to 15 Years Old (BRAVE) - Friedreich's Ataxia Research Alliance, Biogen
- Phase 1 Clinical Trial of an Investigational Gene Therapy for Cardiac Disease Associated with Friedreich's Ataxia - AAVrh10hFXN, Weill Medical College of Cornell University

Non-interventional studies

- Clinical Course of Disease in Participants With FA Cardiomyopathy (CLARITY-FA), Lexeo Therapeutics
- Mechanisms, Markers, and Risk Factors in Friedreich's Ataxia Heart Disease, University of Southern Florida
- Biomarkers in Friedreich's Ataxia, University of Florida
- Remote Monitoring Study for Friedreich's Ataxia, Massachusetts General Hospital
- Neurobooth - Digital Phenotyping for Better Measures, Massachusetts General Hospital
- SKYCLARYS (Omaveloxolone) Pregnancy and Lactation Surveillance Program - Friedreich's Ataxia Research Alliance, Biogen
- A Study to Learn More About the Long-Term Safety of BIIB141 (Omaveloxolone) in Participants With Friedreich's Ataxia Who Are Prescribed it for the First Time - Friedreich's Ataxia Research Alliance, Biogen

RESEARCH RESOURCES

Clinical Trials 101 - A Guide for Participants

Clinical Trials 101 Guide for Participants and Informed Consent worksheet are available as resources to patients considering clinical study participation and can be found on FARA's website and are available translated into 9 languages. curefa.org/research/understanding-clinical-trials/

mFARs Training video

There is now a mFARS training video available to consortium members for educational purposes. Please do not distribute outside of consortium collaborators.
https://youtu.be/OI1xvE_qvh0

Tissue Bank Access for Researchers

NIH NeuroBioBank at University of Maryland
To request tissues that have been donated to the University of Maryland Brain and Tissue Bank, all requests must be submitted to the NIH NeuroBioBank.
Click on the "Researchers" tab at the NIH NeuroBioBank website. Information is provided for tissue availability from hundreds of disorders along with demographics and RIN, a measure of RNA quality. Additional information about the tissue request process is available at their website. If you have any questions, please contact FARA at info@curefa.org.

Surgical Tissue at the Children's Hospital of Philadelphia
To access the FA tissues that have been donated during surgical procedures, please contact the study coordinator at CHOP according to the details on this flyer.

End-of-Life Tissue Donation
When a person dies, a rapid tissue collection will allow researchers to collect important tissues for detailed study. Previous donations of tissues upon death from individuals with FA have provided us with a much greater understanding of FA and how the condition affects various tissues, including the brain, spinal cord, eyes and heart. Additionally, donations from individuals who do not have FA provide tissues that serve as valuable comparisons in research. Learn more about tissue donation at curefa.org/research/tissue-donation-program/

Process Update! How to facilitate the donation process

Please refer families directly to the University of Maryland Brain and Tissue Bank for all tissue donations made after death. If anyone is interested in donating, please email btbdonors@som.umaryland.edu for the link to online registration forms or by calling (410) 706-1755.

Selected Recent Publications

CALL for VOLUNTEERS

If your site has a junior investigator or fellow interested in providing a short editorial of a "recent publication" for the next quarterly newsletter, please reach out to fagcc-info@curefa.org.

Naeije G, Georgiev C, Cabaraux P, Bourguignon M. Cerebellar grey matter volume predicts cerebellar tDCS efficacy in individuals with Friedreich ataxia. Clin Neurophysiol. 2025 May 16:2110744. doi: 10.1016/j.clinph.2025.2110744. Epub ahead of print. PMID: 40399205.

Liu H, Fan D, Tao H, Shen Z, Yao K. Characteristics of Adverse Events and Clinical Risks of Omaveloxolone Based on FAERS Data. Cerebellum. 2025 Jun 18;24(4):119. doi: 10.1007/s12311-025-01873-4. PMID: 40533692.

Gunther K, Profeta V, Keita M, Park C, Wells M, Sharma S, Schadt K, Lynch DR. Safety Monitoring of Omaveloxolone in Friedreich Ataxia: Results from One Year of Clinical Treatment. Neurol Ther. 2025 Jun;14(3):1105-1114. doi: 10.1007/s40120-025-00749-3. Epub 2025 Apr 30. PMID: 40304846; PMCID: PMC12089633.

Rummey C, Thomas-Black G, Garcia-Moreno H, Lynch DR, Abeti R, Arisoy H, Heslegrave A, Zetterberg H, Giunti P; European Friedreich's Ataxia Consortium for Translational Studies (EFACTS). Increase of Plasma Biomarkers in Friedreich's Ataxia: Potential Insights into Disease Pathology. Mov Disord. 2025 Jun 11. doi: 10.1002/mds.30250. Epub ahead of print. PMID: 40498047.

Reetz K, Lischewski SA, Dogan I, Didszun C, Pishnamaz M, Konrad K, Marx-Schütt K, Farmer J, Lynch DR, Corben LA, Pandolfo M, Schulz JB; FACROSS study group. Friedreich's ataxia-a rare multisystem disease. Lancet Neurol. 2025 Jul;24(7):614-624. doi: 10.1016/S1474-4422(25)00175-9. PMID: 40541211.

Visani E, Canafoglia L, Nanetti L, Rossi Sebastiano D, Duran D, Anversa P, Bonfoco D, Dotta S, Tabarelli D, Castaldo A, Marchini G, Mongelli A, Mariotti C. Neuromagnetic responses to multimodal stimuli in Friedreich's ataxia. Clin Neurophysiol. 2025 Jul;175:2110738. doi: 10.1016/j.clinph.2025.2110738.

Heleven E, Vyhnalek M, Karamazovová S, Van Overwalle F, Naeije G. False Beliefs, True Deficits: Investigating Social Cognition in Friedreich Ataxia. Cerebellum. 2025 Jul 11;24(5):128. doi: 10.1007/s12311-025-01886-z. PMID: 40643773.

Perlman S, Anheim M, Boesch S, Lewis JH, Lynch DR. Managing Aminotransferase Elevations in Patients with Friedreich Ataxia Treated with Omaveloxolone: A Review and Expert Opinion on Use Considerations. Neurol Ther. 2025 Aug;14(4):1209-1227. doi: 10.1007/s40120-025-00752-8. Epub 2025 Jun 2. PMID: 40455368; PMCID: PMC12255605.

EVENTS OF INTEREST

FA Community Conversation on Wednesday, August 6, at 12 p.m. ET.

https://us02web.zoom.us/webinar/register/WN_BlvduAzETtyU0tYCXA4QMw#/registration

FARA CEO Jen Farmer and Chief Scientific Officer Barbara Tate will focus on the FA Drug Development Pipeline and cover the following topics:

- How FARA-funded research leads to treatment approaches in the pipeline
- A high-level overview of current programs in the pipeline
- What to consider when you're thinking about participating in a clinical trial

SAVE THE DATE: ICAR 2026 Atlanta, Georgia, U.S.

International Congress for Ataxia Research

Ataxia UK, National Ataxia Foundation (NAF), Friedreich's Ataxia Research Alliance (FARA), and Ataxia Global Initiative (AGI) are pleased to announce the date for the next International Congress for Ataxia Research (ICAR).

ICAR 2026 will take place at the Renaissance Atlanta Waverly Hotel & Convention Center in Atlanta, Georgia, U.S. Please save the date for November 10-13, 2026.

ICAR 2026 will be the place to share the latest ataxia research, including updates on Friedreich's ataxia and SCAs 1, 2, 3, 6, 7, and more. Attendees will hear developments in novel treatment approaches, clinical trial results, and scientific debates from leading ataxia researchers. There will also be the opportunity to network with academic and industry leaders. Special sessions and events are planned for junior researchers.

UPCOMING MEETINGS

02 September 2025
FA GCC Quarterly Investigator Meeting
8am ET| 2pm CET

23/24 September 2025
FA GCC Coordinator Call
(Option 1) 23 Sept
5pm ET| 7AM AET (24th)
(Option 2) 24 Sept
9am ET | 3 pm CET

27-28 October 2025
Clinical Trials in Hereditary Ataxias (CTAX) in Amsterdam

17 November 2025
FA GCC Quarterly Investigator Meeting 12pm ET| 6pm CET

01/02 December 2025
FA GCC Coordinator Call
(Option 1) 01 Dec
4pm ET| 8 am AET (2nd)
(Option 2) 02 Dec
9am ET| 3 pm CET

10-13 November 2026
ICAR 2026 Atlanta, Georgia, U.S.

GRANT/FUNDING OPPORTUNITIES

- **FARA research grants** General Research Grants, Postdoctoral Fellowships, and Research Awards –LOI deadline is August 15, 2025. See FARA grant program priorities here.
- **ASGCT** Career Development Awards and Fellowships
- **The Warren Alpert Distinguished Scholars Fellowship** enrollment period will run from July 2025 to November 1, 2025. The Award will support individual postdoctoral scientists of exceptional creativity in the field of neurosciences.
- **Innovation Grants to Nurture Initial Translational Efforts** (IGNITE Program) | National Institute of Neurological Disorders and Stroke
- The **National Ataxia Foundation's 2026 Grant Cycle** is now open. Submissions will be accepted until August 15, 2025
- FDA is establishing a **Rare Disease Endpoint Advancement (RDEA) Pilot Program** to support novel endpoint efficacy development for drugs that treat rare diseases.
- The **Patient-Centered Outcomes Research Institute** (PCORI) intends to issue a PCORI Funding Announcement on August 12, 2025, seeking to fund high-quality, patient-centered comparative clinical effectiveness research studies that focus on rare diseases. LOI Deadline: September 23, 2025 | 5 pm ET
- **Funding opportunities for rare disease research** FDA launched the FDA Rare Neurodegenerative Disease Grant Program. The agency awards grants and contracts to public and private entities to cover costs of research and development of interventions intended to prevent, diagnose, mitigate, treat or cure ALS and other rare neurodegenerative diseases in adults and children.
- **ARPA-H funding opportunities** The Advanced Research Projects Agency for Health (ARPA-H) funds transformative health breakthroughs using diverse funding mechanisms.
- Grant opportunities from CIRM can be found **here**.
- Various **MRC Funding Opportunities**