



THE
FA GCC
GLOBAL CLINICAL CONSORTIUM
JULY 2026 NEWSLETTER

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FA GCC SITE SPOTLIGHT

July Spotlight: Prague site

FARA Director of Global Relations & Initiatives Myriam Rai visited the FA GCC site at the **Centre of Hereditary Ataxias, Motol and Homolka University Hospitals, and the Second Faculty of Medicine, Charles University.**



Associate Professor Martin Vyhnálek, Doctor Lucie Šťovíčková, and the multidisciplinary team provided an overview of the Czech national FA program, which integrates adult and pediatric neurological care, translational research, biomarker development, and participation in UNIFAI and interventional studies.

The Prague site has established **one of the largest FA cohorts in Central Eastern Europe**, with nearly 50 patients enrolled in longitudinal follow-up. Since 2021, the team has contributed clinical data to international FA registries through EFACTS and, more recently, the global UNIFAI natural history study. The visit highlighted ongoing research on genotype-phenotype correlations, disease progression, digital and functional outcome measures, neurofilament light chain (NfL) biomarkers, mitochondrial dysfunction, and real-world evaluation of omaveloxolone treatment. Through its participation in FA-GCC and UNIFAI, the Prague site contributes valuable clinical expertise, biomarker data, and longitudinal assessments that support the development of future therapies and outcome measures for Friedreich's ataxia.

The team also showcased its comprehensive approach to patient care, including advanced neurological assessments, functional testing, speech and digital biomarker programs, cardiological evaluation, retinal imaging, neuropsychological assessment, and collaborative research initiatives aimed at improving our understanding of disease progression and treatment response in FA.



Pictured left to right:
Martin Vyhnálek,
Pavel Hrubý,
Jaroslava Paulasová -
Schwabová, Myriam
Rai, Lucie Šťovíčková,
Alena Zumrová



FA GCC SITE SPOTLIGHT

Introduction of our new site: Argentina

We are excited to welcome the Neurogenetics Clinic and Laboratory within the Neurology Division at **Hospital J.M. Ramos Mejía** in Buenos Aires, Argentina.

Led by **Dr. Marcelo Kauffman** and **Dra. Dolores González Morón**, the team is internationally recognized for its expertise in hereditary ataxias and other rare neurogenetic disorders. Through pioneering work in genetic diagnosis, precision medicine, and patient care, they have helped build one of Argentina's leading neurogenetics programs, including a significant body of work focused on Friedreich's ataxia, the most common recessive ataxia seen in their national cohort. Their commitment to advancing research and improving access to care has made a meaningful impact for individuals and families affected by ataxia across the region.



Meet the Argentina Team



Top row, from left: Marcelo Kauffman, MD, PhD, Dolores González Morón, MD, PhD, Sergio Rodríguez, MD; Bottom row, from left: Valentín Selvaggi, MD, María José Miere, MD, Nancy Medina, B.Sc. in Genetics

“ We are the Neurogenetics Clinic and Laboratory at Hospital J.M. Ramos Mejía in Buenos Aires, Argentina. Our team brings together neurologists, and genetics specialists dedicated to the diagnosis, care, and research of hereditary and neurogenetic disorders. We combine specialized clinical evaluation with in-house molecular and genomic testing to provide accurate diagnoses and personalized guidance for patients and their families. ”



NEW CLINICAL OPERATIONS MANAGERS



Mackenzi Coker earned her bachelor's degree in Communication Sciences and Disorders from the University of South Florida and her master's degree in Speech-Language Pathology from Florida Atlantic University.

As a licensed Speech-Language Pathologist, Mackenzi's clinical focus in aerodigestive health and orofacial myology brings a patient-centered perspective to advancing clinical research and supporting the Friedreich's ataxia community.

Before joining FARA, Mackenzi spent six years in neuromuscular clinical research, including five years as a Clinical Research Coordinator at the University of Florida specializing in Friedreich's ataxia. During her time at UF, she coordinated multiple FA clinical trials and natural history studies, working closely with individuals and families throughout every stage of study participation while supporting protocol implementation, regulatory oversight, site operations, and publication development. She is excited to continue collaborating with the FA community to advance research and support clinical trial success.

Outside of work, Mackenzi enjoys spending time on the water and volunteers alongside her Goldendoodle, Bailey, as a pet therapy team.



Aliza Naqvi graduated from New York University with a focus on pre-medical sciences and Psychology. She began her career as an In-House CRA, completing extensive training across all aspects of clinical research, and has worked for 10 years across Biotech, CRO, and vendor organizations throughout the clinical development lifecycle.

For the past six years, she has focused on rare disease research, contributing to Data Review Committees, Investigator-Initiated Research, and Expanded Access Programs. Through this work, she collaborated closely with patients and physicians to help provide access to critical therapies through compassionate use pathways, deepening her commitment to patient advocacy and patient-centered care.

Aliza is excited to join an organization where the patient remains at the heart of the mission and looks forward to supporting efforts that improve the lives of patients and their families. In her free time, you can find her exploring new places with her husband, toddler and mini-goldendoodle.

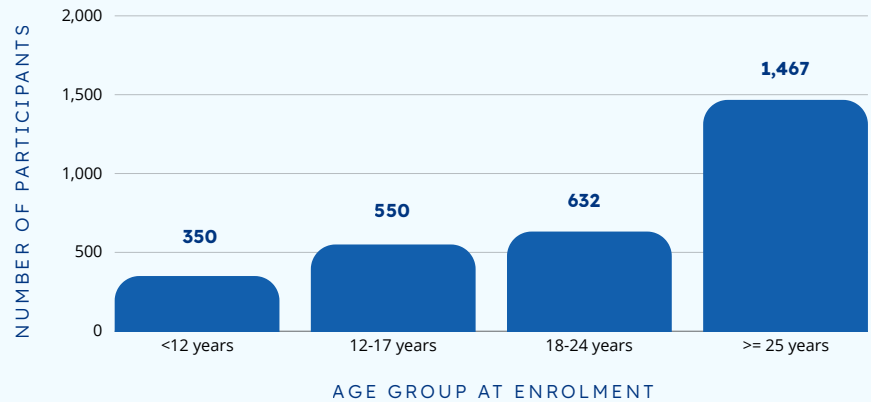




UNIFAI DATA

Real-Time Dashboard Visualization

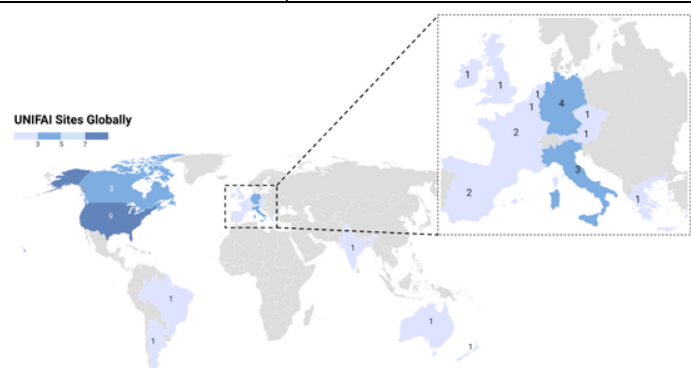
This dashboard, powered by Medrio's real-time reporting capabilities, presents a comparative visualization of participant age distribution. The bar chart categorizes participants by age at the time of enrollment into four distinct groups: < 12 years, 12–17 years, 18–24 years, ≥ 25 years.



Continents represented	Countries with UNIFAI sites	UNIFAI sites
5	18	35
Total visits in 2026 to date	Total baseline visits	Cumulative visits
464	3,008	15,576
% participants with at least 2 visits	% participants with at least 3 visits	% participants with at least 5 visits
77%	64%	45%
% participants with at least 10 visits	Average number of visits completed by participants	Highest number of visits completed by a participant
16%	5	23

35 UNIFAI Sites

With the addition of the newest FA GCC site, Argentina, there are now 35 institutions with clinical research teams devoted to FA.





THE FA GCC GLOBAL CLINICAL CONSORTIUM

ENROLLING STUDIES AND TRIALS

Improve Mitochondrial Function & Reduce Oxidative Stress

CLINICAL TRIAL | AGES 13+
**Effects of Aerobic Training on
Friedreich's Ataxia**

Columbia University
In-person

[VIEW DETAILS](#)

Frataxin Replacement, Stabilizers, or Enhancers

CLINICAL TRIAL | AGES 2-60
**An Open Label Study of Nomlabofusp in
Subjects With Friedreich's Ataxia (Jive)**

Larimar Therapeutics
Phase II | In-person

[VIEW DETAILS](#)

Gene Addition

CLINICAL TRIAL | AGES 12-50
**Phase 1 Clinical Trial of an
Investigational Gene Therapy for
Cardiac Disease Associated with
Friedreich's Ataxia -
AAVrh.10hFXN**

Weill Medical College of
Cornell University
Phase I | In-person

[VIEW DETAILS](#)

Improve Mitochondrial Function & Reduce Oxidative Stress

CLINICAL STUDY | AGES 16+
**A Study to Learn More About the Long-
Term Safety of BIIB141
(Omaveloxolone) in Participants With
Friedreich's Ataxia Who Are Prescribed
It by Their Own Doctors**

Biogen
Phase IV, Postmarketing
Study | In-person

[VIEW DETAILS](#)

Improve Mitochondrial Function & Reduce Oxidative Stress

CLINICAL STUDY | AGES 16+
**SKYCLARYS (Omaveloxolone) Pregnancy
and Lactation Surveillance Program**

Biogen
Phase IV, Postmarketing
Study | Remote

[VIEW DETAILS](#)

Non-Interventional Study

Clinical Study | Ages 8-70
Biomarkers in Friedreich's Ataxia

University of Florida
In-person

[VIEW DETAILS](#)

Non-Interventional Study

CLINICAL STUDY | AGES 16+
**Mechanisms, Markers, and Risk Factors in
Friedreich's Ataxia Heart Disease (FA-HD)**

USF Health
In-person

[VIEW DETAILS](#)

Non-Interventional Study

CLINICAL STUDY | AGES 8-30
**Neurobooth - Digital Phenotyping for
Better Measures**

Massachusetts General
Hospital
In-person

[VIEW DETAILS](#)

Non-Interventional Study

CLINICAL STUDY | AGES 0+
Friedreich Ataxia Global Clinical Consortium UNIFIED Natural History Study (UNIFAI)

Friedreich's Ataxia Research Alliance
In-person

[VIEW DETAILS](#)



THE FA GCC GLOBAL CLINICAL CONSORTIUM

PUBLICATIONS

- **Peripheral frataxin levels govern long-term clinical progression in Friedreich ataxia**
- **Analysis of a Modified Version of the Inventory of Non-Ataxia Signs Over 12 Years in Patients with Friedreich's Ataxia in the EFACTS Study**

[CLICK HERE TO VIEW](#)

[CLICK HERE TO VIEW](#)

ICAR 2026

Register for ICAR 2026 today!
Registration prices increase after September 1



ICAR 2026
November 10-13, 2026
Atlanta, Georgia, U.S.
Renaissance Atlanta Waverly Hotel & Convention Center

International Congress for Ataxia Research
ICAR
ATAxia FARA NAF

ICAR 2026

The 2026 International Congress for Ataxia Research (ICAR) will be held November 10-13, 2026 in Atlanta, Georgia, USA.

Hosted by the National Ataxia Foundation (NAF), the Friedreich's Ataxia Research Alliance (FARA), Ataxia Global Initiative (AGI), and Ataxia UK, ICAR will cover aspects of cerebellar function, neurodegeneration, and treatment of ataxias, and provide ample opportunities to network with scientists, clinicians, and representatives from industry and patient groups.

[LEARN MORE AND REGISTER](#)



PLEASE JOIN US AT THE
FA GCC Quarterly Investigator Meeting
in person before ICAR 2026
NOV 10 | 8:30am-12:30pm
Renaissance Atlanta Waverly Hotel & Convention Center



2026 EVENTS OF INTEREST

AUG

- Aug 2** rideATAXIA Hometown Boston (Lexington, MA)
- Aug 8** Swing for a Cure: Softball Tournament (Uniontown, OH)
- Aug 9** Rock 'N' Jock Charities: Night of Entertainment (Warwick, RI)
- Aug 9** Rock 'N' Jock Charities: Golf Tournament (East Greenwich, RI)
- Aug 11** rideATAXIA Hometown: rideWILD Haleakala (Maui, HI)
- Aug 15** Saddle Up for FARA (Dade City, FL)
- Aug 22** Go Hog Wild for FARA! (Thompson, CT)
- Aug 22** Sweet Caroline's 21 for FARA (Auburn, AL)
- Aug 23** rideATAXIA Hometown Cleveland (Berea, OH)

SEPT

- Sept 3** Living Life Happily: Access to a Cure (Branford, CT)
- Sept 11** GolfATAXIA (White Lake, MI)
- Sept 19** rideATAXIA Hometown St. Louis (St. Louis, MO)
- Sept 26** Race for Matt & Grace (Providence, RI)

OCT

- Oct 11** rideATAXIA Philly 2026 (Blue Bell, PA)
- Oct 12** FARA & CHOP FA Symposium (King of Prussia, PA and virtual)
- Oct 17** The FARA Energy Ball (Tampa, FL)
- Oct 24** Mattoberfest 2026 (Providence, RI)

NOV

- Nov 1** Team FARA: TCS New York City Marathon (New York, NY)
- Nov 7** rideATAXIA Dallas 2026 (Lewisville, TX)
- Nov 10-13** International Congress for Ataxia Research (ICAR) (Atlanta, GA)
- Nov 14** Epic Game Day (Henderson, NV)



GRANT/FUNDING OPPORTUNITIES

Clinician Scientist Development Award

LOI Due Aug 15

The Clinician Scientist Development Award provides funding for mentored clinical research to provide protected research time to early-career physician faculty to enable career development and transition to independent investigators as clinician scientists. FARA's objective is to promote training and retention of clinicians who wish to develop expertise in diagnosis, management and/or treatment of Friedreich ataxia as part of their clinical practice and who want to engage in Friedreich ataxia (FA)-focused clinical and/or translational research.

General Research Grant

LOI Due Aug 15

The General Research Grant is open to qualified investigators proposing projects that align with FARA Research Priorities.

Postdoctoral Fellowship

LOI Due Aug 15

FARA offers a Postdoctoral Fellowship to support young investigators in the FA biomedical research space. For this application type, the PI of the grant proposal may be either the trainee or the trainee's faculty advisor; however, both the trainee and advisor must be named in the application.

Postdoctoral Research Award

LOI Due Aug 15

FARA offers a Postdoctoral Research Award as a mechanism to expand and retain young investigators in the FA biomedical research space.