# 2017 Conference Summary and Thanks to Partners and Sponsors

ARC 2017 was a 4-day international research conference for academics and industry scientists interested in basic and translational research in the ataxias. It was hosted by Italy's GoFAR, Ataxia UK and US-based FARA (Friedreich's Ataxia Research Alliance), and held in Pisa, Italy. The focus of the meeting is a comprehensive scientific review of new research from disease definition to therapeutic treatments. The conference will include **Friedreich's ataxia** and other recessive ataxias (eg: ataxia with oculomotor apraxia), **dominant ataxias** (eg: spinocerebellar ataxias, DRPLA, episodic ataxias) and **autoimmune ataxias**.

I. Molecular basis of disease:
- Diagnosis and identification of disease
- Pathways associated with disease

II. Translational models of disease:
- Cell and animal models of ataxias
- Drug Discovery
    - Tools for discovery
    - Recent compounds

III. Natural history, biomarkers, and endpoints
- Defining disease measures for use in advancing treatment development

IV. Therapeutics, interventions and clinical development
- Therapeutic approaches to ataxias, including gene therapy
- Regulatory considerations in treatment trials

**Many thanks to the scientific Steering Committee, Organizing Committee and Sponsors!**

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**About ARC-2017**

The International Ataxia Research Conference 2017 took place in Pisa, Italy on the 27th- 30th of September, 2017. FARA was able to partner with International Ataxia Research Organizations to co-host the largest ataxia research conference to date. IARC2017 was co-organized by goFAR, Ataxia UK and FARA and simply by the numbers included:

- over 400 attendees- researchers, clinicians, patients, and government, regulatory, and industry representatives.
- Representatives from 22 countries
- >15 pharma/ biotech companies
- >30 platform presentations on FA
- Over 95 poster presentations on FA!!!

In addition to the impressive engagement numbers, the IARC had many meaningful research and collaboration take-aways such as:

**Young Investigators**
The IARC2017 welcomed an increasing number of young investigators, demonstrating growing interest in research for the ataxias. The host organizations believe in developing and mentoring the next generation of researchers and were honored to give nine scholarships to graduate students and post-doctoral fellows who submitted competitive abstracts. In order to help these young investigators make contacts in the field and develop their careers in ataxia research, FARA provided a lunch at the start of the meeting to help young investigators meet more senior investigators and learn about options for career development within the field. In addition, among more than 50 competitive Young Investigator Posters, Nesli Ece Sen and Saba Saqlain earned honors for best Young Investigator Posters for their work in ataxia and Friedreich's ataxia respectively.

**International Collaboration**
Ataxia is not just a US or a European disease. It is a global disease, requiring international collaboration on all fronts. These collaborations are the power behind research advancement and treatments for FA. The research advocacy organizations held a meeting at IARC2017 and discussed the importance of the FA Global Patient Registry as an essential resource for both clinical trial readiness and advancing research. The partner organizations committed to further collaboration in expanding and growing the patient registry. https://curefa.net/registry/

**Exercise is Good**
Several researchers presented data on the benefits of exercise for people living with FA. Dr. Zen Yan from University of Virginia presented data in mice showing that long term aerobic exercise is seen to slow FA onset (Click here for more information) Dr. Sarah Milne from Monash University in Australia also presented a poster demonstrating that physical rehabilitation improved physical and emotional health. There were also several other poster and platform presentations drawing attention to the need to treat symptoms of visual dysfunction, hearing loss, speech and swallowing and depression.

**Patient & Clinical Trial Design Roundtables**
Individuals with ataxia and parents and caregivers joined the meeting for a special patient roundtable discussion to share their personal stories and need for treatment. This roundtable made clear statements about the need to not only focus on stopping and curing the disease, but also asserted that treatments which target symptoms are meaningful. If therapies could improve symptoms such as vision and hearing loss or speech impairment, this would be significant for people living with ataxia.

(Click here for more information) There was also informative roundtable on clinical trial design for ataxias that featured representation from the European Medical Association, ataxia patient community, clinical neurologists and pharmaceutical companies. This allowed for important discussion among all stakeholders on good clinical trial design. Some themes that arose from this discussion included the need for better communication between study sponsors and study participants and the need for objective outcome measures.

**Advancing Therapies**
The conference included more than 20 platform and poster presentations focused on drug discovery, preclinical and clinical outcomes in FA. These presentations demonstrated progress in many aspects of the treatment pipeline. Encouraging results of recent clinical trials were reported including Reata's Phase 2 MOXIe study, as well as a number of talks on new therapeutic options that may reach the clinic soon. There has been significant growth and advancement in drug discovery and development for therapies that target frataxin (ie the root cause of FA). This was most notable in the number of presentations on genetic based therapies including oligonucleotide and gene replacement strategies. To read more about some of the research presented at the conference, click one of the links below:

- Omaveloxolone Can Aid Neurological Function in FA Patients, MOXIe Trial Shows
- Synthetic Molecules Seen to Restore Frataxin Protein Levels in Cells from FA Patients
- Gene Therapy Seen to Reverse Sensory Ataxia in FA Mouse Model
- Protein-producing Frataxin Gene Delivered to Brain, Heart of Primates in Gene Therapy Study
- Small Molecules Mimicking Brain Factors Raise Frataxin Protein Levels in FA Mice in Study

Thank you to all who made IARC2017 a success- the host organization- goFAR, steering committee, presenters, and participants. You've shown once again that Together We Will Cure FA!

Click here to view the abstracts from the meeting