

# ICAR 2024 Final Remarks



ATAXIA

ATAXIA GLOBAL  
INITIATIVE

FARA  
Friedrich's  
Ataxia  
Research  
Alliance

NAF  
National Ataxia  
Foundation

# ICAR 2024 by the numbers

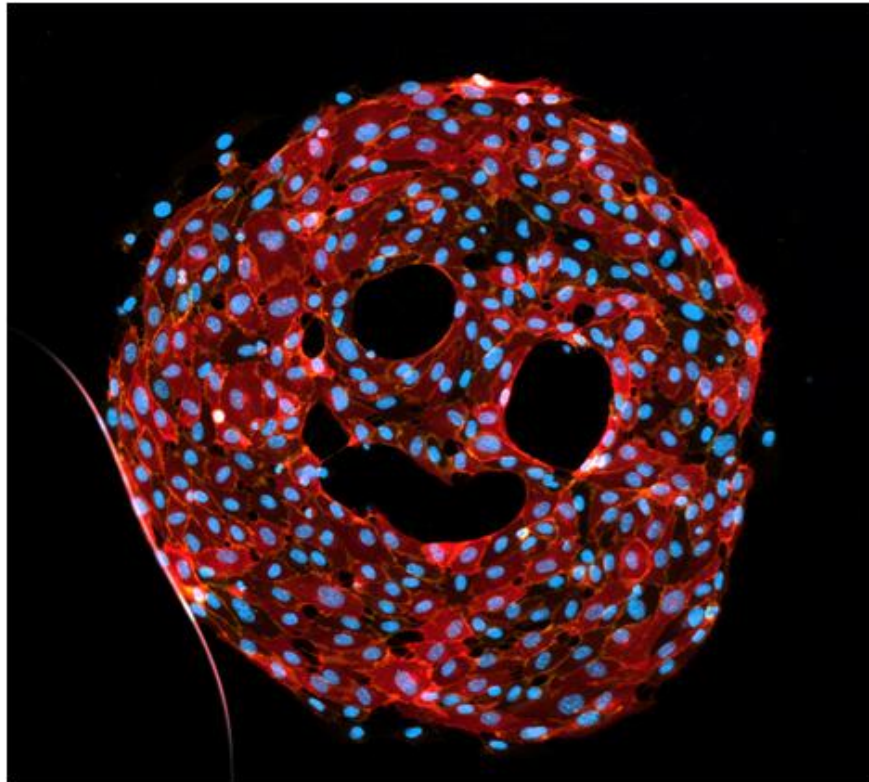
- Registrants: 600
- Submitted Abstracts: ~470
- Sessions: 11 + Late Breaking Session
- Workshops: 8 (basic science → patient engagement)
- Invited Talks: > 80

# Notable

- Novel ataxia genes discovered – more and more patients are being diagnosed and this is a first step to therapy (SCA4, 27B CANVAS)
- Long read sequencing technology – interruption of the repeats and their role; interruptions as therapy
- Somatic instability (SCA27B, SCA3, FA)
- Developmental component of ataxias
  - spatial and temporal regulation of gene expression using single cell approaches

# We did not achieve a consensus.....

## So, for now let's keep both!



<https://blogs.illinois.edu/view/9030/1192575282>



[https://www.freepik.com/premium-ai-image/cute-smiling-mouse-with-big-ears-holding-piece-cheese-grey-background-funny-character\\_137566372.htm](https://www.freepik.com/premium-ai-image/cute-smiling-mouse-with-big-ears-holding-piece-cheese-grey-background-funny-character_137566372.htm)

# Presentations

Shift of balance from:

Disease Mechanisms/Cell and Animal Models to  
Emerging Therapeutics, Pre-clinical and Clinical Research

# Progress in Clinical Research

- Spinocerebellar ataxia types 1 and 3 and Huntington's disease
  - ASO VO659 (CAG repeat-targeting)
- Friedreich's ataxia:
  - Skyclarys (Omaveloxolone)
  - Vatiquinone
  - Nomlabofusp
  - GeneTACs
- SCA27B
  - 4-aminopyridine
- Other
  - Genome editing
  - New capsids for gene therapy
  - Role of rehabilitation/exercise