

# **Ataxia Global Conference 2022**

**Addison, Dallas (TX), USA**

# Welcome!

**TETSUO ASHIZAWA**



**BERNARD BRAIS**



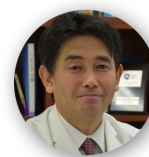
**ROSALIND CHUANG**



**THOMAS KLOCKGETHER (CHAIR)**



**OSAMU ONODERA**



**JOSÉ LUIZ PEDROSO**



**ALEXANDRA DURR**



**BRENT FOGEL**



**JULIE GREENFIELD**



**BING-WEN SOONG**



**MATTHIS SYNOFZIK (VICE-CHAIR)**



**DAVID SZMULEWICZ**



**SUE HAGEN**



**LAURA JARDIM**



**HONG JIANG**

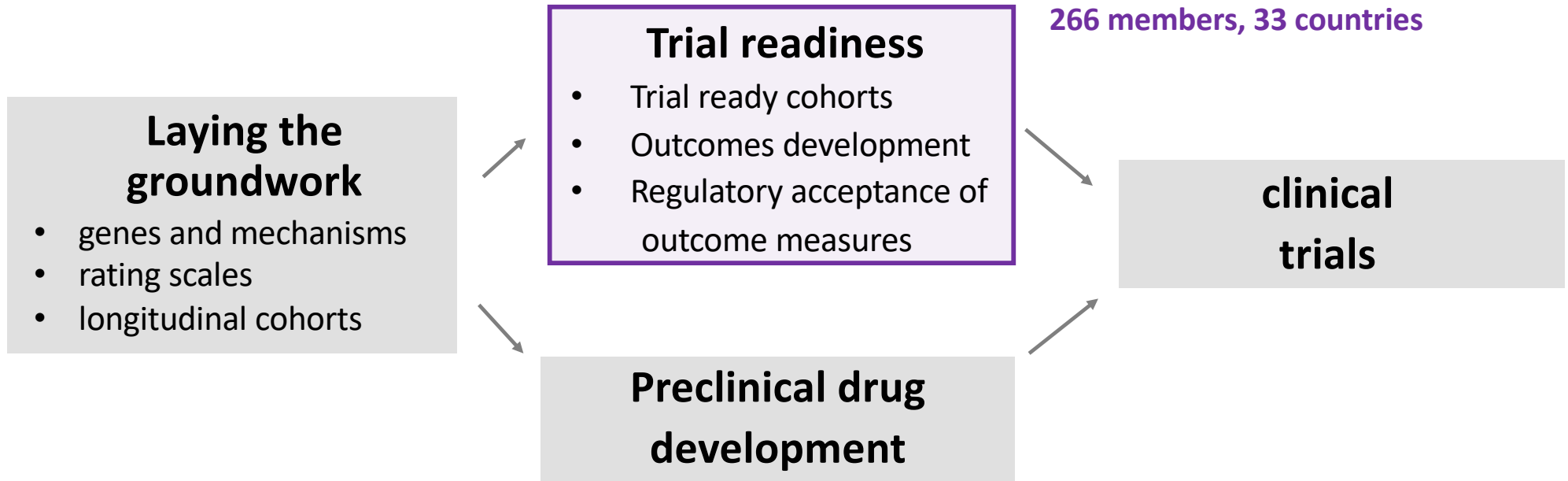


# The road towards molecular therapies for ataxias



- global
- academia, industry, PAOs together

266 members, 33 countries





- aggregate & harmonize natural history data
- standardize assessments (clinical, biosamples, imaging, digital-motor, etc)
- harmonize & optimize outcome measures
- education and training
- multicentre trial readiness services

coordination,  
standardization &  
joint projects

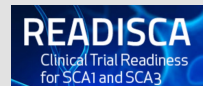
expertise &  
knowledge

content  
input

## Patient Data

cohorts, registries & natural history studies

study networks



NAF CRC-  
SCA

BIGPRO  
Brazil



TREAT-  
ARCA

single site studies



anonymized  
data










- Regulatory qualification of outcome measures and biomarkers

# AGI formats

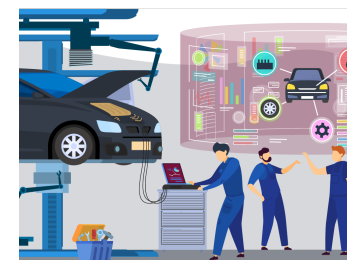
<https://ataxia-global-initiative.net>

- working groups
- projects
- ressources
- Young Investigator Initiative

#1	<u>Clinical outcomes &amp; registries</u>	
#2	<u>Molecular biomarkers &amp; biosampling</u>	
#3	<u>MR biomarkers</u>	
#4	<u>Digital-motor biomarkers</u>	
#5	<u>Model systems &amp; preclinical trials</u>	
#6	<u>Next-generation genomics and platforms</u>	
#7	<u>Policy and patient organization engagement</u>	

→ consensus & standards: special issue in Cerebellum 2023

- tools & methods studios



**Session 1**  
Date: 15th of July 2022, 5-6 pm CEST  
Speaker: Christian Rummeny (Clinical Data Science GmbH, Basel, Switzerland)

[Register here](#)



**Session 2**  
Date: 22th of July 2022, 5-6 pm CEST  
Speaker: Dieter Hilgers (University of Aachen, Aachen, Germany)

[Register here](#)



**NEW! Session 3**  
Date: 29th of July 2022, 5-6 pm CEST  
Speaker: Sophie Tezenas du Montcel (Sorbonne University, Paris, France)

[Register here](#)



= joint **work studios** with the aim to **discuss and improve trial-readiness tools and methods** with regard to **their novel (or improved) application in the ataxia field**



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