

# Agenda | SCA & ARCA Global Conference 2020

19-21 October 2020 | online, Berlin time (CEST)

## Monday, 19 October 2020

### 15:00 SCA & ARCA Global: setting the stage

15:00 Welcome

Thomas Klockgether (Bonn, Germany), Matthis Synofzik (Tübingen, Germany)

15:15 Keynote lecture

Towards trial-equivalent natural history studies in SCAs and ARCAs: standards and criteria for trial-readiness

Massimo Pandolfo (Brussels, Belgium)

### 15:50 Session 1: Clinical, patient-reported and digital biomarkers

15:50 Biomarkers and prospects for interventional trials at the preataxic stage

*tbc*

16:10 Patient-reported outcome measures for ataxia (PROM-Ataxia): ready for trials?

*tbc*

16:30 Speech as a biomarker in ataxia: What can it tell us and how should we use it?

Adam Vogel (Melbourne, Australia)

16:40 Wearable Sensors for multicentre clinical ataxia trials: assessment in clinics/lab

Fay Horak (Chicago, USA)

16:50 Wearable Sensors for multicentre clinical ataxia trials: assessment in real life

Winfried Ilg (Tübingen, Germany)

### 17:00 Break

#### Virtual coffee rooms

Meet the speakers of Session 1

### 17:30 Session 2: Fluid and imaging biomarkers

17:30 Neurofilaments as translational fluid biomarkers for SCA: cross-species evidence from SCA3 cohorts and mice

Matthis Synofzik (Tübingen, Germany)

17:50 Ataxin-3 proteins in biofluids

Leonard Petrucelli (Jacksonville, USA)

18:10 MRI in SCA3

Marcondes França Jr. (Campinas, Brazil)

18:30 Magnetic resonance biomarkers in ARCAs: lessons learned

Pierre-Gilles Henry (Minnesota, USA)

**18:50 Flash talks I**

6 flash talks à 5 minutes will be selected from submitted abstracts

**Flash talks II**

6 flash talks à 5 minutes will be selected from submitted abstracts

**19:20 Break**

**Virtual coffee rooms**

Meet the speakers of Session 2 and Flash Talks I & II

**19:50 SCA Working Group Results**

19:50 Clinical outcome assessments  
Thomas Klockgether (Bonn, Germany)

20:20 MR biomarkers  
Gülin Öz (Minneapolis, USA)

20:50 Biosampling standards  
Puneet Opal (Chicago, USA)

21:20 SCA Global policy  
Holm Graessner (Tübingen, Germany)

**21:50 SCA Global – Young Investigator Initiative**

- Introduction
- Ataxia scales training
- Further steps

Heike Jacobi (Heidelberg, Germany) & Jennifer Faber (Bonn, Germany)

**22:30 End meeting day 1**

**Tuesday, 20 October 2020**

**15:00 Session 3: Trial-readiness**

15:00 From trial-ready cohorts to first clinical trials in global multicenter studies of rare diseases: learning from DIAN-TU  
David Clifford (St. Louis, USA)

15:20 Natural history and treatment trial design for ultra-rare diseases  
Ralf-Dieter Hilgers (Aachen, Germany)

15:40 Making outcome measures ready for regulatory acceptance: FDA qualification standards for outcome measures, biomarker and data aggregation in ultra-rare diseases  
Jane Larkindale (C-Path Institute, Tucson, USA)

16:00 Neurodegenerative Disorders: regulatory aspects  
Karl Broich (BfArM, Bonn, Germany)

**16:20 Session 4: Industry panel / presentations on planned trials**

**17:20 Break**

**Virtual coffee rooms**

Meet the speakers of Session 3 and 4

**17:50 Flash talks III**

6 flash talks à 5 minutes will be selected from submitted abstracts

**Flash talks IV**

6 flash talks à 5 minutes will be selected from submitted abstracts

**18:20 Session 5: Existing ARCA cohorts**

18:20 ARCAs in Europe: PREPARE  
Matthis Synofzik (Tübingen, Germany)

18:35 ARCAs in Europe: SPATAX  
Alexandra Dürr (Paris, France)

18:50 ARCAs in USA  
Brent Fogel (Los Angeles, USA)

19:05 ARCAs in Canada  
Bernard Brais (Montreal, Canada)

19:20 ARCAs in Central and South America  
Jose Luiz Pedroso (Sao Paolo, Brazil)

19:35 ARCAs in Australia  
David Szmulewicz (Melbourne, Australia)

19:50 ARCAs in Asia  
Osamu Onodera (Niigata, Japan)

**20:05 Break**

**Virtual coffee rooms**

Meet the speakers of Session 5 and Flash Talks III & IV

**20:35 Session 6: Genes & Mechanisms**

20:35 SCAs: mechanisms, model systems and compound screening  
*tbc*

20:55 ARCAs: mechanisms, model systems and compound screening  
Helene Puccio (Lyon, France)

**21:15 Genetic platforms for collaborative ARCA and SCA gene hunting**

21:15 GENESIS  
Stephan Zuchner (Miami, USA)

21:25 GPAP / RD-Connect  
Sergi Beltran (Barcelona, Spain)

21:35 CAGC  
Brent Fogel (Los Angeles, USA)

21:45 Round table: NGS platforms – how to collaborate within ARCA and SCA Global?  
All speakers; moderator: Matthis Synofzik (Tübingen, Germany)

**22:00 End meeting day 2**

## Wednesday, 21 October 2020

### 15:00 ARCA Working Group Sessions

15:00 Defining tasks and introduction of working groups  
Holm Graessner (Tübingen, Germany)

15:10 ARCA Working Group Discussions:

- #1 Clinical outcomes & registry
- #2 next-generation genomics & platforms
- #3 molecular biomarkers & biosampling
- #4 MRI biomarkers
- #5 digital-motor biomarkers
- #6 model systems & preclinical trials
- #7 policies & patient organization engagement

### 16:30 Break

Virtual coffee rooms

### 17:00 ARCA Working Group Results

17:00 #1 Clinical outcomes & registry

17:10 #2 next-generation genomics & platforms

17:20 #3 molecular biomarkers & biosampling

17:30 #4 MRI biomarkers

17:40 #5 digital-motor biomarkers

17:50 #6 model systems & preclinical trials

18:00 #7 policies & patient organization engagement

18:10 ARCA Wrap-up

Matthis Synofzik (Tübingen, Germany)

### 18:30 End of SCA & ARCA Global Conference 2020