



1st ARCA Global Conference, Denver, March 2-3, 2020
 Sheraton Denver Downtown, 1550 Court Place, Denver, USA

Conference Schedule

- Session 1: Existing cohorts and natural history of ARCAs as the basis for trial-readiness
- Session 2: Genes & mechanisms as bridgeheads towards molecular treatment targets
- Session 3: Clinical, imaging, digital-motor and fluid biomarker outcomes: standards for discovery and assessment in multi-center settings
- Session 4: Enhancing clinical trial readiness
- Session 5: Working groups

DAY1 - MONDAY , March 2, 2020

Welcome & Opening Remarks

8:00 AM	Breakfast	
8:30-8:40 AM	Welcome & Introduction	<i>ARCA Steering Committee (Sue Hagen/Brent Fogel)</i>
8:40-9:00 AM	ARCA Global: the new global network	<i>Matthis Synofzik, Tübingen</i>
9:00-9:15 AM	Lessons learned from SCA Global & possible infrastructure connections	<i>Thomas Klockgether, Bonn</i>

Session 1: Existing cohorts and natural history of ARCAs as the basis for trial-readiness
Chair: Holm Graessner

9:15-9:40 AM	Keynote: Towards trial-equivalent natural history studies in ARCAs: standards and criteria for trial-readiness <i>Melissa Beiner, Biohaven, New Haven, USA</i>	
9:40-10:00 AM	Keynote: Clinical standards, integrating cohorts, and combining registries: the need and successful implementation in multicenter settings <i>Rebecca Schüle, Tübingen, Germany</i>	
10:00-10:15 AM	ARCAs in Europe: PREPARE	<i>Matthis Synofzik, Tübingen</i>
10:15-10:30 AM	ARCAs in USA	<i>Brent Fogel, Los Angeles</i>
10:30-10:45 AM	ARCAs in Central and South America	<i>Jose Luiz Pedroso, Sao Paolo</i>

10:45-11:00 AM	<i>Coffee break</i>	
11:00-11:15 AM	ARCAs in Australia	David Szmulewicz, Melbourne
11:15-11:30 AM	ARCAs in Asia	Osamu Onodera, Niigata
11:30-11:45 AM	ARCAs in Canada	Bernard Brais, Montreal
11:45-12:00 AM	ARCAs in Europe: SPATAX	Alexandra Durr, Paris
12:00-12:20 AM	Round table: How can we integrate ARCA cohorts across the world and how will we be able to run trial-equivalent natural history studies? <i>moderator: Thomas Klockgether; discussants: all continent representatives (Synofzik, Dürr, Brais, Fogel, Pedroso, Onodera, Szmulewicz)</i>	
12:20-1:00 PM	<i>Lunch</i>	

Session 2: Genes & mechanisms as bridgeheads towards molecular treatment targets
Chair: Bernard Brais

Genes

1:00-1:20 PM	Keynote: ARCA genomics: overview and novel genes <i>Matthis Synofzik, Tübingen, Germany</i>	
1:20-1:50 PM	Platforms for collaborative ARCA gene hunting:	
	(i)	GENESIS <i>Stephan Zuchner, Miami</i>
	(ii)	GPAP/RD-Connect <i>Sergi Beltran, Barcelona</i>
	(iii)	CAGC <i>Brent Fogel, Los Angeles</i>
1:50-2:05 PM	Round table: NGS platforms: how to collaborate within ARCA GLOBAL? <i>moderator: Synofzik, discussants: Zuchner, Beltran, Fogel</i>	
2:05-2:15 PM	Feature evolution, disease progression and discriminative symptoms of multisystemic RFC1 disease: a multi-center study of 61 patients <i>Selina Reich, Tübingen, Germany (selected abstract)</i>	
2:15-2:25 PM	Prevalence of RFC1-Mediated Spinocerebellar Ataxia in a North American Ataxia Cohort <i>Brent Fogel, Los Angeles, USA (selected abstract)</i>	

Mechanisms and targets

2:25-2:45 PM	Keynote: ARCA mechanisms, model systems and compound screening <i>Francesca Maltecca, Milan, Italy</i>	
2:45-3:05 PM	Keynote: Developing ASO treatments for recessive ataxias: systematic requirements for candidate ARCAs <i>David Corey, Dallas, USA</i>	
3:05-3:15 PM	Towards AAV treatments in ARCAs: lessons learned from autosomal-dominant ataxias and systematic requirements for recessive ataxias	

Melvin Evers, Unicure, Amsterdam, Netherlands

3:15-3:30 PM **Round table:** What are the most promising approaches & next steps to make selected ARCAs ready for gene therapies?

moderator: Pandolfo; discussants: Maltecca, Corey, Evers, Beiner, Larkindale

3:30-3:50 PM *Coffee break*

Session 3: Clinical, imaging and fluid biomarker outcomes: standards for discovery and assessment in multi-center settings

Chair: David Szmulewicz

3:50-4:10 PM **Keynote:** 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

4:10-4:30 PM Clinical scales and functional tests: requirements and examples for multi-center trial readiness in ARCAs

Massimo Pandolfo, Brussels, Belgium

4:30-4:50 PM Magnetic resonance biomarkers in ARCAs: lessons learned

Pierre-Gilles Henry, Minnesota, USA

4:50-5:05 PM ENIGMA: lessons learned from multicenter ataxia MRI studies & a platform for ARCAs

Ian Harding, Melbourne, Australia

5:05-5:15 PM Fully-automated pipeline for whole-brain MRI volumetry in clinical trials in ataxia

Kirsi Kinnunen, IXICO, London, UK (selected abstract)

5:15-5:35 PM Fluid biomarker discovery in ultra-rare diseases: successful approaches and lessons learned

Nicholas Ashton, Gothenburg, Sweden

5:35-5:50 PM *Afternoon break*

5:50-6:10 PM Wearable Sensors for multicenter clinical trials in:

(i) Clinics/lab-based *Christopher Gomez, Chicago*

(ii) real-life *Winfried Ilg, Tübingen*

6:10-6:20 PM Gait Laboratory in a Box: Objective gait assessment via smartphone analysis

Andrea Nemeth, Oxford, UK (selected abstract)

6:20-6:30 PM Wearable sensorized device to assess upper-limb motor function in patients with ataxia

Susanna Summa, Rome, Italy (selected abstract)

- 6:30-6:40 PM Speech as a biomarker in ataxia: What can it tell us and how should we use it?
Adam Vogel, Melbourne, Australia (selected abstract)
- 7:30 PM *Dinner with patient perspective by Cindy Demint (AOA2 ataxia)*
 - *ballroom Sheraton Downtown-*

DAY2 - TUESDAY , March 3, 2020

Session 4: Enhancing clinical trial readiness

Chair: Holm Graessner

- 7:00 AM Breakfast
- 8:00-8:05 AM Introduction to Day 2
Holm Graessner, Tuebingen, Germany
- 8:05-8:25 AM **Keynote:** From trial-ready cohorts to first clinical trials in global multicenter studies of rare diseases: learning from DIAN-TU
David Clifford, St. Louis, USA
- 8:25-8:35 AM Establishing a global consortium for rigorous multi-center trial-readiness natural history studies: lessons from TREAT-NMD and ENROLL-HD
Richard Roxburgh, Auckland, Australia
- 8:35-08:55 AM Natural History Trial designs for ultra-rare diseases
Sophie Tezenas du Montcel, Paris, France
- 08:55-09:15 AM Treatment Trials design for ultra-rare diseases
Ralf-Dieter Hilgers, Aachen, Germany
- 09:15-09:45 AM Project proposals for ARCA GLOBAL: speed dating flash talks (each 180 sec)
 - *see seperate proposal overview sheet -*
- 09:45-10:25 AM Guided poster session
 +COFFEE BREAK

Session 5: Working Groups

- 10:25-10:35 AM Defining tasks and Introduction of working groups
Holm Graessner, Tübingen, Germany
- 10:35-10:45 AM Charging each working group for specific goals
Holm Graessner, Tübingen, Germany
- 10:45-12:15 AM Working Group discussion sessions

Plenary session: presentation of working group results & next steps of ARCA GLOBAL

Chair: Holm Graessner

- 12:15-12:23 AM WG #1 Clinical outcomes & registry

12:23-12:31 AM WG #2 next-generation genomics & platforms
12:31-12:39 AM WG #3 molecular biomarkers & biosampling
12:39-12:47 AM WG #4 MRI biomarkers
12:47-12:55 AM WG #5 digital-motor biomarkers
12:55-1:05 PM WG #6 model systems & preclinical trials
1:05-1:13 PM WG #7 policies & patient organization engagement

1:15-1:25 PM **Action plans and closing remarks** *Matthis Synofzik*

END OF MEETING

- posters must be removed at this time –

1:30-2:30 PM Internal board meeting of ARCA GLOBAL steering committee: wrap of results and next steps (includes working lunch)

5:00 -6:30 PM **National Ataxia Foundation Ataxia Investigator's meeting reception**
- all ARCA GLOBAL attendees welcome! -