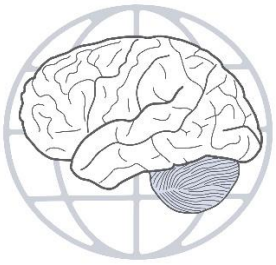

1st ARCA Global Conference, Denver, March 2-3, 2020

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

Confirmed speakers: Brent Fogel (Los Angeles, USA), Pierre-Gilles Henry (Michigan, USA), Thomas Klockgether (Bonn, Germany), Stephan Zuchner (Miami, USA), Bernard Brais (Montreal, Canada), Rebecca Schüle (Tübingen, Germany), Osamu Onodera (Niigata, Japan), Matthis Synofzik (Tübingen), Jose Luis Pedroso (Sao Paolo, Brazil), Davide Szmulewicz (Melbourne, Australia).

DAY1 - MONDAY , March 2, 2020

Welcome & Opening Remarks		
8:30-8:40 AM	Welcome & Introduction	*ARCA Steering Committee*
8:40-9:00 AM	ARCA Global idea	*Matthis Synofzik, Tübingen*
9:00-9:15 AM	Lessons learned from SCA Global & possible infrastructure connections	*Thomas Klockgether, Bonn*
Session 1: Existing cohorts and natural history of ARCAs as the basis for trial-readiness		
9:15-9:40 AM	**Keynote:** Towards trial-equivalent natural history studies in ARCAs	
9:40-10:00 AM	**Keynote:** Clinical standards, integrating cohorts, and combining registries: the need and successful implementation in multicenter settings	
10:00-12:00 AM	ARCAs around the world	Several investigators

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?
12:20-1:00 PM	*LUNCH*

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

Genes

1:00-1:20 PM	**Keynote:** ARCA genomics: overview and novel genes
1:20-1:50 PM	Platforms for collaborative ARCA gene hunting: (i) GENESIS (ii) GPAP/RD-Connect (iii) CCAG
1:50-2:05 PM	**Round table:** NGS platforms: how to collaborate within ARCA GLOBAL?
2:05-2:25 PM	Talk#1+2 from Abstract Submission (new genes/ mechanisms) *t.b.d*

Mechanisms and targets

2:25-2:45 PM	**Keynote:** ARCA mechanisms, model systems and compound screening
2:45-3:05 PM	**Keynote:** AAVs and ASOs: systematic requirements for candidate ARCA targets
3:05-3:15 PM	Talk#1 from Abstract Submission (targets/gene therapy/treatments) *t.b.d*
3:15-3:25 PM	Talk#2 from Abstract Submission (targets/gene therapy/treatments) *t.b.d*
3:25-3:40 PM	**Round table:** What are the most promising approaches & next steps to make selected ARCAs ready for gene therapies?
3:40-3:55 PM	*COFFEE BREAK*

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

3:55-4:15 PM	**Keynote:** FDA perspective on outcome measures: standards of biomarker and COA qualification in trials in ultra-rare diseases
4:15-4:35 PM	Magnetic resonance biomarkers in ARCAs: lessons learned
4:35-4:50 PM	ENIGMA: lessons learned from multicenter ataxia MRI studies & a platform for ARCAs
4:50-5:10 PM	Clinical scales and functional tests: requirements and examples for multi-center trial readiness in ARCAs

5:10-5:30 PM	Fluid biomarker discovery and confirmation in rare diseases
5:30-5:45 PM	*COFFEE BREAK*
5:45-6:05 PM	Wearable Sensors for multicenter clinical trials
6:05-6:15 PM	Talk#1 from Abstract Submission *t.b.d*
6:15-6:25 PM	Talk#2 from Abstract Submission *t.b.d*

DAY2 - TUESDAY , March 3, 2020

Session 4: Enhancing clinical trial readiness	
8:15-8:35 AM	**Keynote**: From trial-ready cohorts to first clinical trials in global multicenter studies of rare diseases: learning from DIAN-TU
8:35-08:50 AM	Which criteria have to be met to consider a certain ultra-rare disease cohort to be trial- ready? The pharma perspective
08:50-09:10 AM	Natural History Trial designs for ultra-rare diseases
09:10-09:30 AM	Treatment Trials design for ultra-rare diseases
09:30-09:45 AM	Regulatory requirements for transatlantic resource sharing *NIH representative* Alternatively: C-Path: pre-competitive standards, data aggregation and outcome measure standards paving the way for regulatory approval.
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
10:35-10:45 AM	Charging each working group for specific goals
10:45-12:15 AM	Working Group discussion sessions
Plenary session: presentation of working group results *(8 min each)*	
12:15-12:23 AM	#1 Clinical outcomes & registry
12:23-12:31 AM	#2 next-generation genomics & platforms
12:31-12:39 AM	#3 molecular biomarkers & biosampling

12:39-12:47 AM	#4 MRI biomarkers
12:47-12:55 AM	#5 digital-motor biomarkers
12:55-1:05 PM	#6 model systems & preclinical trials
1:05-1:13 PM	#7 policies & patient organization engagement

Session3: Clinical, imaging and fluid biomarker outcomes: standards for discovery and and assessment in multi-center settings
Chair: t.b.d

1:15-1:25 PM	Action plans and closing remarks
	END OF MEETING
1:30-2:30 PM	Internal board meeting of ARCA GLOBAL steering committee: wrap of results and next steps (includes working lunch)
5:00 PM	**National Ataxia Foundation Ataxia Investigator's meeting reception** (for AIM attendees)