December 2019

**1st ARCA Global Conference, Denver, March 2nd-3rd, 2020 : CALL FOR ABSTRACTS**

The **1st conference of the ARCA Global network, held in Denver, USA, March 2nd-3rd, 2020**, aims to advance a global consortium for translational studies in autosomal-recessive ataxias (ARCAs), ***ARCA Global*** (<http://ataxia-global-initiatives.net/arca-global/>). ARCA Global will aggregate ARCA patients and expertise from centers worldwide in close collaboration with patient organizations to facilitate all crucial translational steps on this path towards trial-readiness. It will:

* aggregate registry-inventoried ARCA cohorts from across the world
* perform genetic fingerprinting of unsolved ARCA patients, including defining novel ARCA genes and pathways
* lead standardized preclinical compound trials in ARCA model systems
* chart the natural history of ARCAs
* establish trial-ready clinical, imaging and molecular outcome measures for ARCAs
* implement first-in-human drug trials and prepare phase II and III studies in ARCAs.

Our goal for the **1st ARCA GLOBAL conference** is to advance a global consortium for this translational path, and to initiate first infrastructure for each of these aims in a cross-center harmonized global fashion.

Abstract submissions are encouraged from both senior and junior ataxia investigators. Women and under-represented minorities are encouraged to submit abstracts in line with the mission of the NIH. We further encourage submission of work that focuses on other degenerative diseases (e.g. autosomal-dominant ataxias, other rare recessive neurogenetic diseases) but has implications on recessive ataxias. The major review criteria will be new cutting-edge and translational trial-readiness research that focuses on one of the four meeting themes:

**·       Theme #1 Existing cohorts and natural history of ARCAs as the basis for trial-readiness.** Comprehensive studies that analyze the frequencies, clinical and genetic characteristics of ARCA cohorts in different racial, ethnic, social and regional populations across the world, with a clear aim to prepare the path for registry-inventoried ARCA cohorts. Innovative studies (including conceptual and technological ideas) on registries, registry trial design and between-registry interfaces, allowing to establish registry-inventoried ARCA cohorts and possibly also bringing together different ARCA cohorts and registry from the world.

 **·       Theme #2: Genes and Mechanisms: bridgeheads towards molecular treatment target identification.** Gene hunting and mechanistic studies that unravel novel recessive ataxia genes and/or molecular mechanisms underlying recessive ataxias, in particular if amendable to treatment ideas. Functional or pharmacological preclinical studies on pathways and targets in ARCAs amendable to targeted molecular treatments.

**·       Theme 3: Clinical, imaging, digital-motor and fluid biomarker outcomes: standards for discovery and assessment in multi-center trials.** Studies on validating existing or identifying novel ARCA biomarker candidates and outcome measures, in particular suitable for use in global multi-center treatment trials. On the discovery stage, this might include innovative studies on discovery approaches for fluid biomarkers in recessive ataxias and other rare neurogenetic diseases which might serve as a methodological role model for several rare recessive conditions. Intriguing proposals for standards for biomarkers (clinical, imaging, digital-motor, fluid) specifically suited and geared for global multicenter ARCA studies.

 **·       Theme 4: Enhancing clinical trial readiness. Studies on first innovative, targeted** preclinical trials, first-in-human trials and possibly even phase II and III multi-center clinical trials in ARCAs. Intriguing concepts and first success stories on pathways of regulatory approval of ARCA outcome measures or even treatments; these pathways should ideally be paradigmatic and applicable to several ARCAs alike.

**Abstract submission guidelines:**

• Abstracts should consist of sections covering background, methods, results, discussion, and conclusions. The maximum length is limited to 2500 characters. Please indicate the theme under which your abstract should be reviewed. Please note that the above themes are meant to be guidelines.

* Submit abstracts through google docs at: <https://docs.google.com/forms/d/e/1FAIpQLSf4nA1UXpY6YaeyzIIFS5WCbFJp6ScPJBrIA82RMqd0IpsFZg/viewform?usp=sf_link> by **Jan 6th, 2020**.
* Decisions on acceptance of the abstracts by the ARCA GLOBAL steering committee will be announced January 21th, 2020. Submitters must accept the invitation for presentation at the ARCA GLOBAL conference by January 25th, 2020.

**Selected presenters must be able to attend the entire ARCA GLOBAL meeting.**

**Presentations**

**Six** (6) speaking slots have been reserved by the ARCA GLOBAL Steering Committee for oral presentations . In addition, twelve (12) high quality abstracts will be selected for the guided poster session and be given four minutes for oral presentation during this session.

**Registration for ARCA GLOBAL** has already opened. Please register early since space is limited. Register via the following link:

 <http://weblink.donorperfect.com/2020ARCA_Global>

The AIM registration fees will be Junior Investigator - $150, Senior Investigator - $250, Industry - $1,000.

Thank you to the ARCA GLOBAL Steering Committee members: Matthis Synofzik, MD; Bernard Brais, MD, PhD; Brent Fogel, MD, PhD, Jose Luis Pedroso, MD, PhD; Osamu Onodera, MD PhD; David Szmulewicz MD; Holm Graessner PhD, Sue Hagen, Julie Greenfield PhD

Questions may be directed to Sue Hagen at NAF at susan@ataxia.org or 763-231-2742.