

## Agenda | Ataxia Global Conference 2022

4-5 November 2022 | Dallas, TX, USA & online | Dallas time (CDT)

### Friday, 4 November 2022

|              |   |                                  |
|--------------|---|----------------------------------|
| <b>12:00</b> | <b>Registration</b>   | Foyer in front of Laliq Ballroom |
| <b>13:00</b> | <b>Session 1: Cohort updates</b><br><i>Chair: Alexandra Dürr, Paris, France</i>   | Laliq Ballroom                   |
| 13:00        | Welcome<br><i>Thomas Klockgether, Bonn, Germany &amp; Matthis Synofzik, Tübingen, Germany</i>   |                                  |
| 13:05        | Cohort update – ESMI<br><i>Jennifer Faber, DZNE Bonn, Germany</i>   |                                  |
| 13:20        | The ARCA cohort: a global collaborative longitudinal cohort for advancing trial readiness in autosomal recessive cerebellar ataxias<br><i>Matthis Synofzik, University of Tübingen, Germany</i>   |                                  |
| 13:35        | BIGPRO: the SCA3/MJD cohort from South Brazil. An update<br><i>Laura Jardim, Universidade Federal do Rio Grande do Sul, Brazil</i>  |                                  |
| 13:50        | Cohort update – China. The Natural History of Spinocerebellar Ataxia Type 3 in Mainland China<br><i>Hong Jiang, Central South University, China, <a href="#">online</a></i>   |                                  |
| 14:05        | READISCA – An NIH-funded transatlantic observational study for SCA1 and SCA3<br><i>Tetsuo Ashizawa, Houston Methodist Research Institute, USA</i>   |                                  |
| 14:20        | Clinical phenotype & biomarkers in sporadic degenerative ataxia: longitudinal data from the SPORTAX registry<br><i>Thomas Klockgether, DZNE Bonn, Germany</i>   |                                  |
| 14:30        | Mapping SPG7 natural history: cross-sectional progression rates in 97 patients from the PROSPAX study<br><i>Rebecca Schüle, University of Heidelberg, Germany, <a href="#">online</a></i>   |                                  |
| 14:40        | Data sharing success stories <ul style="list-style-type: none"> <li>▪ ESMI: Clinical phenotype and biomarkers in sporadic degenerative ataxia: longitudinal data from the SPORTAX registry (<i>Thomas Klockgether, DZNE Bonn, Germany</i>)</li> <li>▪ C-Path (<i>Kael White, Ataxias Consortium at Critical Path Institute, USA</i>)</li> </ul> |                                  |
| <b>15:00</b> | <b>Coffee Break</b>   | Garden Court III                 |
| <b>15:50</b> | <b>Session 2: Patient-centred outcomes</b><br><i>Chair: Lauren Moore, NAF, USA</i>  | Laliq Ballroom                   |
| 15:50        | KEYNOTE LECTURE: Activities of Daily Living<br><i>Christian Rummey, Clinical Data Science, Switzerland</i>  |                                  |
| 16:10        | Patient-focused outcomes in genetic ataxias: cross-sectional validity and longitudinal sensitivity to change of the FARS-ADL<br><i>Andreas Traschütz, University of Tübingen, Germany</i>   |                                  |
| 16:25        | Determining clinical meaningful change of clinical and non-clinical outcomes: how can it be achieved?<br><i>Sophie Tezenas, Sorbonne University, France</i>   |                                  |
| 16:40        | Capturing clinical progression in multisystemic genetic ataxias: lessons from 1637 prospective assessments in patients with autosomal recessive or early-onset ataxia<br><i>Andreas Traschütz, University of Tübingen, Germany</i>  |                                  |
| 16:50        | Temporal dynamics of the Scale for the Assessment and Rating of Ataxia (SARA).<br><i>Paul Moulairie, Sorbonne University, France</i>  |                                  |
| 17:00        | Novel AGI activities <ul style="list-style-type: none"> <li>▪ Work program for SARA modification<br/><i>Thomas Klockgether, DZNE Bonn, Germany</i></li> <li>▪ AGI Data Sharing Policy<br/><i>Matthis Synofzik, University of Tübingen, Germany</i></li> </ul>   |                                  |
| <b>17:40</b> | <b>Group photo</b>  |                                  |
| <b>18:00</b> | <b>Poster session (incl. small reception)</b><br>– on-site only –   | Garden Court III                 |
| <b>19:30</b> | <b>End of meeting day 1</b>   |                                  |
| <b>20:00</b> | <b>AGI Steering Committee meeting dinner</b> – invitation only –<br>SC members will meet Ira & Holm at 20:00 at the hotel reception   |                                  |

**Saturday, 5 November 2022**

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|--------------|---|------------------|
| <b>8:30</b>  | <b>Session 3: Biomarkers</b><br><i>Chair: Matthis Synofzik, Tübingen, Germany</i>   | Lalique Ballroom |
| 8:30         | KEYNOTE LECTURE: Remote disease specific movement monitoring<br><i>Lisa Alcock, Newcastle University, UK</i>  |                  |
| 8:50         | Body worn sensors – remote monitoring in Ataxias: Results from the US & Europe <ul style="list-style-type: none"> <li>A Score of Integrated Balance for Ataxia (SIBA)<br/><i>James McNames, Portland State University, USA</i></li> <li>Towards ecologically valid biomarkers: real-life gait assessment capture subtle longitudinal and preataxic changes in cerebellar ataxia<br/><i>Winfried Ilg, University of Tübingen, Germany</i></li> </ul> |                  |
| 9:20         | Challenges and possibilities of automated speech monitoring in ataxia<br><i>Adam Vogel, University of Melbourne, Australia</i>  |                  |
| 9:35         | Blood levels of neurofilament light as trial biomarkers in degenerative ataxias: the examples of spinocerebellar ataxia type 1 (SCA1) and multiple system atrophy of cerebellar type (MSA-C)<br><i>Carlo Wilke, University of Tübingen, Germany, <a href="#">online</a></i>   |                  |
| 9:50         | Imaging biomarker recessive ataxias<br><i>Marcondes C Franca Junior, University of Campinas, Brazil</i>   |                  |
| 10:05        | Advanced multimodal MRI detects preataxic and early-stage alterations in SCA1 and SCA3 with high sensitivity.<br><i>Gülin Öz, University of Minnesota, USA</i>  |                  |
| 10:15        | Subtle abnormalities of gait and posture in the prodromal stage of spinocerebellar ataxia type 2.<br><i>Yasmamy González-Garcés, Centre for the Research &amp; Rehabilitation of Hereditary Ataxias Holguín, Cuba, <a href="#">online</a></i>   |                  |
| 10:30        | Panel discussion: regulatory acceptance – presymptomatic stage<br><i>Stephanie Fradette, Biogen, USA, <a href="#">online</a>   Michael Benatar, University of Miami, USA, <a href="#">online</a>   Matthis Synofzik, University of Tübingen, Germany</i>  |                  |
| <b>11:00</b> | <b>Coffee Break</b>   | Garden Court III |
| <b>12:00</b> | <b>Session 4: Therapy</b><br><i>Chair: Tetsuo Ashizawa, Houston, USA</i>  | Lalique Ballroom |
| 12:00        | KEYNOTE LECTURE: Developing mutation-specific splice-switching ASOs for ultra-rare and private ataxias<br><i>Willeke van Roon-Mom, Leiden University Medical Center, the Netherlands</i>  |                  |
| 12:20        | KEYNOTE LECTURE: Genetic therapy approaches for neurodegenerative brain diseases and tackling the delivery challenge: the example of genetic FTD<br><i>Chris Shaw, King's College London, UK, <a href="#">online</a></i>  |                  |
| 12:40        | Increasing power of clinical trials in SCA1, 2, 3 and 6 with efficient designs and SARA scales.<br><i>Emilien Petit, Sorbonne University, France</i>  |                  |
| 12:50        | Cerebellar transcranial direct current stimulation in spinocerebellar ataxia type 3: a randomized, double-blind, sham-controlled trial.<br><i>Roderick Maas, Radboud University Medical Center, the Netherlands</i>   |                  |
| <b>13:00</b> | <b>Industry session</b><br><i>Chair: Holm Graessner, Tübingen, Germany</i>  | Lalique Ballroom |
| 13:00        | Development of VO659, a CAG repeat targeting ASO in development for PolyQ diseases<br><i>Scott Schobel, Vico Therapeutics</i>   |                  |
| 13:10        | AAV gene therapy for cardiac and central nervous system involvement in Friedreich's Ataxia<br><i>Darin Falk (CSO), Lacerta Therapeutics, Inc.</i>   |                  |
| 13:20        | Improving clinical trial design using digital wearable technology in Friedreich ataxia (FA) and spinocerebellar ataxias (SCAs) 1, 2, 3, and 6<br><i>Koene R.A. Van Dijk, Pfizer Inc.</i>  |                  |
| <b>13:30</b> | <b>End of meeting</b>   |                  |