

## **5<sup>th</sup> International Symposium on ARSACS**

**Wednesday, Oct 31<sup>st</sup>**

*Jeanne Timmins Amphithéâtre, Montreal Neurological Institute and Hospital*

### **5<sup>th</sup> International Symposium on ARSACS meets PREPARE**

- 1.45 – 2.00 PM      Introduction  
**B. Brais**
- 2.00 – 2.30 PM      ARSACS in Turkey: novel patients, genotypes and phenotypes  
**N. Basak**
- 2.30 – 3.00 PM      Preparing treatment trials in ARSACS: trial- relevant clinical outcome measures, natural history and sample size calculations  
**C. Gagnon**
- 3.00 – 3.30 PM      Cell-based and fluid biomarkers in ARSACS: which might become trial-relevant?  
**F. Santorelli**
- 3.30 – 3.45 PM      Identifying MRI and fluid biomarkers in ARSACS: an embarking multicenter study  
**M. Synofzik**
- 3.45 – 4.30 PM      Searching for drugs and planning for preclinical trials  
**B. Brais**
- 4.30 – 5.30 PM      Videotaping of short talks for the Ataxia de Charlevoix-Saguenay Foundation, open to the general public (Board Room, NW 201)  
*Speakers will be given a specific time*
- 5.30 PM              **ARSACS Meeting – Cocktail and Dinner**  
ARSACS Dinner  
*Ferreira Restaurant, 1448 Peel Street – Private Entrance H3A 1S8*  
  
*For all ARSACS and PREPARE PIs*  
*(Other PREPARE members can register and participate at their own cost)*

## Thursday, Nov 1<sup>st</sup>

*De Grandpré Communications Centre, Montreal Neurological Institute and Hospital*

- 8.30 – 9.25 AM Videotaping of short talks for the Ataxia de Charlevoix-Saguenay Foundation (open to the general public)  
*(Jacques Bougie Board Room, NW 201)*  
*Speakers will be given a specific time*
- 9.30 – 12.30 AM What's new on Sacsin?  
Moderator: **Kalle Gerhing, McGill University**
- 9.30 – 10.00 AM Cytoskeletal dynamics and cell migration in Sacsin deficient cells  
**Paul Chapple, Queen Mary University of London**
- 10.00 – 10.30 AM Sacsin, mutated in the ataxia ARSACS, regulates intermediate filament assembly and dynamics  
**Benoit Gentil, McGill University**
- 10.30 – 11.45 AM *Coffee Break*
- 11.45 – 12.15 PM Sacsin function in cytoskeletal dynamics and organelle transport  
**Vincent Gerard, McGill University**
- 12.15 – 12.45 PM Unravelling co-translational quality control of mutant Sacsin in ARSACS pathogenesis  
**Fabiana Longo, Università Vita-Salute San Raffaele, Italy**
- 12.45 – 1.30 PM Lunch  
*Helen Penfield Atrium*
- 1.30 – 1.40 PM Group photo  
*Helen Penfield Atrium*
- 1.40 – 4.30 PM Finding compounds and targets to treat ARSACS  
Moderator: **Paul Chapple, Queen Mary University of London**
- 1.40 – 2.10 PM Sacsin function in health and disease  
**Roxanne Larivière, McGill University**

- 2.10 – 2.40 PM Altered synaptic and firing properties of cerebellar Purkinje cells in a mouse model of ARSACS  
**Alanna Watt and Anne McKinney, McGill University**
- 2.40 – 3.10 Structural and functional studies of Sacsin  
**Hamed Hojjat, McGill University**
- 3.10 – 3.30 PM *Coffee Break*
- 3.30 – 4.00 PM Ceftriaxone treatment improves motor performances and delays Purkinje cell degeneration in the ARSACS mouse model  
**Francesca Maltecca, Università Vita-Salute San Raffaele, Italy**
- 4.00 – 4.30 PM Genome-wide screening for SACS genetic interactions using CRISPR-Cas9 in human cell lines  
**Ramy Malty, University of Regina**
- 4.30 – 5.00 PM High Throughput Screen (HTS) using a Fibroblast cytoskeletal bundling assay  
**Nicolas Sgarioto, McGill University**
- 5.00 – 5.15 PM Wrap-up  
**Bernard Brais, McGill University**