**FPWR Virtual Research Symposium**

**September 30th - October 1st, 2020**

**Sept. 30th: 12-3 ET; Oct. 1st: 12-3 and 3:30-4:30 ET**

**Wednesday, September 30th**

**AFTERNOON SESSION I 12:00-1:30 PM**

12:00-12:10 pm

**Welcome and Introduction**

12:10- 12:30 pm

**A mouse model to assess PWS gene therapy,** James Resnick, University of Florida

12:30-12:50 pm

**Development of an epigenetically modified nonhuman primate model of Prader-Willi syndrome,** Yuta Takahashi, Salk Institute

12:50 – 1:10 pm

**Neonatal oxytocin administration impacts key developmental stages of hippocampus and restores adult social memory deficit in Magel2 deficient mice, a model for neurodevelopmental disorders,** Alessandra Bertoni, INMED, INSERM

1:10-1:30 pm

**Pathways to emotional outbursts and how we can use these to develop interventions,**

Siobhan Blackwell and Justin Chung, University of Birmingham

**BREAK 1:30-1:40 PM**

**AFTERNOON SESSION II 1:40-3:00 PM**

1:40-2:00 pm

**Impairment of neuronal architecture in the Snord116del mouse model of Prader-Willi syndrome,** Timothy Wells, Cardiff University

2:00-2:20 pm

**MAGEL2-regulated secretory granule and neuropeptide biogenesis are impaired in Prader-Willi syndrome,** Helen Chen, St. Jude Children’s Research Hospital

2:20-2:40 pm

**Preliminary results from the DESTINY PWS Phase III clinical study,** Jennifer Miller, University of Florida and the DESTINY PWS Investigators

2:40-3:00 pm

**Intranasal carbetocin improves symptoms of PWS in CARE-PWS Phase 3 study,** Davis C. Ryman, MD PhD on behalf of Levo Therapeutics and CARE-PWS study investigators

**Thursday, October 1st**

**AFTERNOON SESSION III 12:00 – 1:30 PM**

12:00-12:10 pm

**Welcome and Introduction**

12:10- 12:30 pm

**Digital gait-analysis based biomarkers for natural history studies and clinical trials with children who have Prader-Willi syndrome,** Claudine Kraan, Murdoch Children’s Research Institute.

12:30-12:50 pm

**Effects of Bifidobacterium animalis subsp. lactis (strain BPL1) supplementation in children and adolescents with Prader-Willi syndrome: a randomized crossover trial,** Marta Ramon-Krauel, Institut de Recerca Sant Joan de Deu

12:50-1:10 pm

**A newborn screening study using a new DNA methylation‑sensitive high-resolution melting assay on dried blood spots to detect Prader‑Willi syndrome,** Leticia Guida, Instituto Nacional de Saude da Mulher

1:10-1:30 pm

**Transcriptomic analysis of PWS model neurons - identification of new gene candidates and molecular mechanisms,** Gordon Carmichael, University of Connecticut

**BREAK 1:30-1:40 PM**

**CONCURRENT AFTERNOON SESSIONS 1:40-3:00 PM**

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| 1:40 -2:00 pm  **Microglial dysfunction in the pathogenesis of Prader-Willi syndrome,** Felipe da Silva, University of Amsterdam | 1:40 -2:00 pm  **Social cognition in Prader-Willi syndrome,** Elizabeth Roof, Vanderbilt University |
| 2:00-2:20 pm  **Single Nucleus RNA-Sequencing Reveals Conservation of Hypothalamic Cell Identities, but Specific Alterations in Gene Expression Profiles in *Magel2* null mice,** Hui Yu, University of Michigan | 2:00-2:20 pm  **Investigating the allocation of visual attention to salient stimuli in young children with Prader-Willi syndrome,** Suzannah Lester, University of Cambridge |
| 2:20-2:40 pm  **MAGEL2 modulates the ubiquitination and stability of the Bardet-Biedl syndrome protein BBS2,** Rachel Wevrick, University of Alberta | 2:20-2:40 pm  **Bone mineral density in adults with PWS and its relationship with gait parameters,** Daniela Rubin, California State University, Fullerton |
| 2:40-3:00 pm  **Molecular changes in Prader-Willi syndrome DPSC derived neuronal cultures reveals subtype-specific expression signatures and clues about Autism incidence,** Kaitlyn Victor, University of Tennessee Health Science Center | 2:40-3:00 pm  **Role of body cast application for scoliosis Associated with Prader-Willi syndrome,** Harold van Bosse, Shriner’s Hospital for Children |

**VIRTUAL POSTER SESSION Thursday, Oct. 1st: 3:30-4:30 PM ET**

1. **Correlation between nutritional phase and age, gender, genetic subtype in patients with PWS,** Kaveri Bhargava, Soleno Therapeutics
2. **Flexible scheduling to prevent the development of disabling resistance to change: acceptability and feasibility of a digital intervention co-produced with stakeholders,** Siobhan Blackwell, University of Birmingham
3. **HQ-CT Analysis by age, genetic subtype, and BMI – an update from PATH for PWS,** Jessica Bohonowych, Foundation for Prader-Willi Research
4. **Identifying RNA interacting partners for the SNORD116 family of small nucleolar RNAs,** Tomaz Bratkovic, University of Ljubljana
5. **Optimization of a viral approach for the embryonic expression of Necdin in Necdin-KO mice**

Julie Buron, INSERM

1. **Establishing potential contextual pathways of emotional outbursts,** Justin Chung, University of Birmingham
2. **Genetic subtype effects of intervention response to the Play-based Remote Enrichment To ENhance Development (PRETEND) program: Parent-training program for preschoolers with PWS,** Ellen Doernberg, Case Western Reserve University
3. **A novel triple monoamine reuptake inhibitor for the treatment of rare obesity disorders: pharmacology of CSTI-500 and Phase 1 clinical trial results,** Roman Dvorak, ConSynance
4. **Eye-Tracking as a potential biomarker of hyperphagia in children and adolescents with Prader-Willi syndrome,** Sarah-Marie Feighan, Trinity College Dublin
5. **Acceptance and Commitment Therapy to reduce stress in fathers of adolescents with PWS: a pilot project,** Janice Forster, PLEA
6. **The reduced satiation thought to occur in Snord116 knockout mice does not appear to be associated with reduced meal-activated signaling in the nucleus of the solitary tract,** Edward Fox, Purdue University
7. **CoRonavIruS Health Impact Survey (CRISIS) – adapted for Prader-Willi syndrome,** Louise Gallagher, Trinity College Dublin
8. **Snord116 post-transcriptionally increases Nhlh2 expression through a 3’ untranslated region motif,** Deborah Good, Virginia Tech
9. **Long-term control of BMI in adults with Prader-Willi syndrome living in residential hostels**

Harry Hirsch, Shaare Zedek Medical Center

1. **Investigating the epigenetic regulator SMCHD1 as a potential therapeutic target for the treatment of PWS,** Megan Iminitoff, Walter and Eliza Hall Institute of Medical Research
2. A point mutation in the SNRPN gene is associated with typical Prader-Willi syndrome phenotype, Virginia Kimonis, University of California, Irvine
3. **Atypical thermo-sensory reactivity in neonatal Magel2 deficient mice,** Valery Matarazzo, INSERM
4. **D-dimer Testing in asymptomatic individuals with Prader-Willi syndrome,** Lisa Matesevac, Foundation for Prader-Willi Research
5. **Content validity of the Epworth Sleepiness Scale for children and adolescents in Prader-Willi syndrome,** Erika McClure, Covance
6. **A Phase 2 Clinical Trial to study excessive daytime sleepiness and other symptoms in patients with Prader-Willi syndrome (PWS),** Albena Patroneva, Harmony Biosciences
7. **The factor structure of the Aberrant Behavior Checklist (ABC) in children with PWS,** Bonnie Taylor, Montefiore
8. **Investigating a new target for treatment in Prader-Willi syndrome,** Lauren Rice, The University of Sydney
9. **Cerebellar volumes associate with behavioral phenotypes in Prader-Willi syndrome,** Kenichi Yamada, Niigata University