2018 PWS Research Symposium Agenda
October 4th, 2018

REGISTRATION	**POMPEIAN BALLROOM FOYER**	**7:00-8:30 AM**
BREAKFAST		**7:30-8:30 AM**

MORNING SESSION 1 – *POMPEIAN BALLROOM* **8:30-10:00 AM**

8:30-8:40 am
Welcome

8:40-9:00 am
Predictors of psychosis in PWS
Carrie Bearden, PhD, University of California, Los Angeles

9:00-9:20 am
Families of individuals with Prader-Willi syndrome: A transactional model
Elisabeth Dykens, PhD, Vanderbilt University

9:20-9:40 am
Does the mechanism of action of intranasal oxytocin in the neonate start in the periphery?
Elizabeth Hammock, PhD, Florida State University

9:40-10:00 am
Characterizing endosomal recycling pathways in primary neurons derived from dental pulp stem cells in individuals with PWS
Helen Chen, PhD, St. Jude Children's Research Hospital

BREAK **10:00-10:30 AM**

MORNING SESSION II – *POMPEIAN BALLROOM* **10:30 -11:50 AM**

10:30-10:50 am
Decreased mortality associated with growth hormone use and lower BMI in PWS
Virginia Kimonis, MD, University of California, Irvine

10:50-11:10 am
Preclinical pharmacology and safety of a novel MetAP2 inhibitor for Prader-Willi syndrome
Micaella Fagan, PhD, Zafgen

11:10 -11:30 am
Early findings from neurobehavioral and neurophysiological studies of a novel *Magel2* knockout rat model
Derek Reznick, Baylor College of Medicine

11:30 -11:50 am
Schaaf MAGEL2 knockdown and SHFYNG patient cell lines exhibit alterations in mTOR and autophagy pathways
Emeline Crutcher, Baylor College of Medicine

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LUNCH – **12:00-1:30 PM**

AFTERNOON SESSION I **1:30-3:10 PM**

BREAKOUT 1: POMPEIAN BALLROOM

1:30-1:50 pm
Histamine-3 inverse agonist Pitolisant: May it constitute a new therapeutic approach for Prader-Willi syndrome?
Marta Pace, PhD, Istituto Italiano di Tecnologia

1:50-2:10 pm
Polymorphisms in the oxytocin receptor (OXTR) modulate response to intranasal oxytocin therapy in individuals with Prader-Willi syndrome
Frederick Kweh, PhD, University of Florida

2:10-2:30 pm
Development of intranasal carbetocin for the treatment of Prader-Willi syndrome
Davis Ryman, MD, Levo Therapeutics

2:30-2:50 pm
DCCR-mediated agonization of the ATP-sensitive potassium channel: A proposed mechanism of action to treat hyperphagia in PWS patients
Neil Cowen, PhD, Soleno Therapeutics

2:50-3:10 pm
The efficacy and safety of tesofensine/metoprolol co-administration in adult patients with Prader-Willi syndrome: an exploratory phase 2a study
Roman Dvorac, PhD, Saniona

BREAKOUT 2: CAPRI

1:30-1:50 pm
SNORD116 missing in Prader-Willi syndrome regulates mRNA stability of immediate early genes
Stefan Stamm, PhD, University of Kentucky

1:50-2:10 pm
Consequences of targeted *SNORD116* deletion in human and mouse neurons
Giles Yeo, PhD, University of Cambridge

2:10-2:30 pm
Significant differences for gene expression distinguishes PWS subtypes and reveals transcripts associated with ASD risk in UPD cases
Lawrence Reiter, PhD, University of Tennessee Health Science Center

2:30-2:50 pm
Physiological excitation/inhibition imbalance in *Magel2*-deficient mice and oxytocin system
Francoise Musçatelli, PhD, Institut de Neurobiologie de la Méditerranée (INMED)

2:50-3:10 pm
Evidence of neuroinflammation in the *Magel2*-null hypothalamus
Deborah Kurrasch, PhD, University of Calgary

BREAK **3:10-3:30 PM**

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AFTERNOON SESSION II 3:30-4:50 PM

BREAKOUT 3: POMPEIAN BALLROOM

3:30-3:50 pm

Exploring impulsive behavior in a mouse model for PWS

Anthony Isles, PhD, Cardiff University

3:50-4:10 pm

Social cognitive ability in preschoolers with PWS and preliminary response to remote parent-training using the PRETEND program

Anastasia Dimitropoulos, PhD, Case Western Reserve University

4:10-4:30 pm

Collaborating with stakeholders in PWS on the development of a "flexible scheduling" early intervention approach designed to prevent the development of disabling resistance to change

Siobhan Blackwell, MPsychSc, University of Birmingham

4:30-4:50 pm

Vagus nerve stimulation for the treatment of temper outbursts in people with Prader-Willi syndrome

Jessica Beresford-Webb, MS, University of Cambridge

BREAKOUT 4: CAPRI

3:30-3:50 pm

Reproductive function in PWS: Evaluation of the HPG axis using GnRH stimulation testing

Diane Stafford, MD, Boston Children's Hospital

3:50-4:10 pm

Cellular and molecular basis of insulin-secretion deficiency in Prader-Willi syndrome

Robert Nicholls, PhD, UPMC Children's Hospital of Pittsburgh

4:10-4:30 pm

MAGEL2, a gene implicated in Prader-Willi syndrome, modulates key circadian rhythm proteins at the cellular level

Vanessa Carias, University of Alberta, Edmonton

4:30-4:50 pm

CRISPR engineering and molecular profiling of PWS cellular models

Derek Tai, PhD and Xander Nuttle, PhD, Harvard University

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POSTER SESSION – *POMPEIAN BALLROOM* 5:00-7:00 PM

1. **Caregiver priorities for endpoints to evaluate treatments for Prader-Willi syndrome: A best-worst scaling**
   Jui-Hua Tsai, MD, Johns Hopkins

2. **Treating Prader-Willi syndrome: analysis of medications, treatments, and supplements taken by PWS patients**
   Leah Pachkowski, Soleno Therapeutics

3. **A caregiver "Prader-Willi syndrome medication input" questionnaire**
   Nikita Srivastava, Soleno Therapeutics

4. **Design of the PATH for PWS study: A non-interventional, observational, natural history study of serious medical events in Prader-Willi syndrome**
   Jaret Malloy, PhD, Zafgen

5. **The novel MetAP2 inhibitor, ZGN-1258, reduces body weight and food intake in mouse models of obesity**
   Micaella Fagan, PhD, Zafgen

6. **The novel MetAP2 inhibitor, ZGN-1258, increases locomotor activity and reduces anxiety-like behavior in mouse models of obesity and anxiety disorders**
   Micaella Fagan, PhD, Zafgen

7. **ZGN-1258: A novel potent MetAP2 inhibitor with reduced risk of coagulopathy**
   Micaella Fagan, PhD, Zafgen

8. **Growth hormone unmasked laryngomalacia and worsened obstructive sleep apnea in infants with Prader-Willi syndrome**
   Parisa Salehi, MD, Seattle Children's Hospital

9. **Pediatric weight management in patients with Prader-Willi syndrome: Pilot initiative of intensive weight management clinic intervention coupled with behavioral program**
   Alaina P. Vidmar, MD, Children's Hospital Los Angeles

10. **Dysmorphology features in Prader-Willi syndrome is influenced by molecular class and growth hormone**
    Virginia Kimonis, MD, University of California, Irvine

11. **Cognitive improvements in children with Prader-Willi syndrome following pitolisant treatment**
    Lara Pullen, PhD, The Chion Foundation

12. **Effect of macronutrient composition on diet-induced thermogenesis in Prader-Willi syndrome (PWS): preliminary findings**
    Maha Alsaif, University of Alberta

13. **Profiling the gut microbiome composition and function in North-American children with and without Prader-Willi syndrome**
    Shima Afhami, University of Alberta

14. **Prader-Willi syndrome mental health research strategy workshop: Update on the top 10 recommendations**
    Lauren Schwartz, PhD, Foundation for Prader-Willi Research

15. **Guanfacine extended release for the reduction of aggression and self injurious behavior in Prader-Willi syndrome - A case series**
    Deepan Singh, MD, NYU Winthrop Hospital

16. **Titration to target dose improves safety profile of diazoxide choline controlled-release tablet (DCCR)**
    Jennifer Abuzzahab, MD, Soleno Therapeutics

17. **A neutralizing monoclonal antibody to gastric inhibitory polypeptide (GIP) prevents and treats obesity in normal and *ob/ob* mice**
    Michael Wolfe, MD, Case Western Reserve University

18. **A study on maternal attachment, sleep and lipid metabolism in a mouse model of Prader-Willi syndrome**
    Hanako Tsushima, PhD, Istituto Italiano di Tecnologia

19. **Study of melanin concentrating hormone and orexin/hypocretin neurons in Prader-Willi syndrome**
    Marta Pace, PhD, Istituto Italiano di Tecnologia

20. **Reactivation of Prader-Willi syndrome genes by epigenetic editing**
    Yuna Kim, PhD, Duke University

21. **Elucidating the function of MAGEL2 through its protein-protein interaction network defined by proximity labeling (BioID) and mass spectrometry**
    Matthea Sanderson, University of Alberta