Purpose: The purpose of the PWS Research Strategy Workshop is to develop a comprehensive research strategy to advance the science of PWS. Participants will prioritize research questions and identify opportunities and needs. Recommendations will be made with respect to resource development, collaborative opportunities and training mechanisms to facilitate attainment of research goals.

Sunday, November 15th

1:00 PM - 6:00 PM  Registration:  Prader-Willi Syndrome Research Strategy Workshop  
Location: EMC Foyer, DoubleTree Hotel

3:00 PM -  Hotel Check-In: DoubleTree Hotel and Executive Meeting Center  
8120 Wisconsin Avenue  
Bethesda, MD 20814

6:00 PM - 8:00 PM  Welcome Reception: Cocktails and heavy hors d’oeuvres
6:00  Reception Begins  
Location: Foyer, Level II, Doubletree Hotel
6:30-6:40  Welcome  
Location: Grand Ballroom A
6:40-7:00  PWS: A Parent’s Perspective  
Keegan Johnson  
President, Foundation for Prader-Willi Research Canada
7:00-8:00  Keynote Address  
Francis Collins, MD, PhD  
Director, National Institutes of Health
8:00  Welcome Reception Ends
TBD  Dinner on Own

Monday, November 16th

7:30 AM  Continental Breakfast  
Location: Continuous break area, Level II

8:00 AM - 12:15 PM  General Session  
Location: Grand Ballroom D

Goal: In this session, each moderator will present an overview of their area of expertise, providing a ‘state of the field’ assessment, with an emphasis on areas of particular opportunity and need with respect to Prader-Willi syndrome. A final presentation will introduce the NIH Chemical Genomics Center and the new Therapeutics for Rare and Neglected Disease program.

8:00 – 8:15  Welcome and Workshop Goals  
Theresa Strong, PhD
8:15 – 8:45  Overview: Clinical Issues  
Dan Driscoll, MD, PhD
8:15 - 9:15  Overview: Obesity, energy balance and hypothalamic function
Steve O’Rahilly, MD, FRCP, FRS
9:15 -9:45 Overview: Psychopathology and mental illness
Tony Holland, MRCP, MRCPsych
9:45-10:15 Break
10:15-10:45 Overview: Molecular Genetics of PWS (genotype to phenotype)
Uta Francke, MD
10:45-11:15 Overview: Animal models of PWS and related phenotypes
Rachel Wevrick, PhD
11:15-12:15 The NIH Chemical Genomics Center and the Therapeutics for Rare and Neglected Diseases Program
Chris Austin, MD
Director, NIH Chemical Genomics Center
Senior Advisor for Translational Research, NIH

12:15- 1:30 PM Lunch (on site)

1:30- 5:30 PM Breakout Sessions
Location: Jasmine (Clinical), Juniper (Obesity), Lavender (Animal Models), Lilac (Genetics) and Orchid (Mental Health) Rooms (third level)
Goal: Workshop participants will break out into small groups [Genetics, Animal Models, Obesity, Clinical Issues and Mental Health] to discuss specific questions on how best to move the field forward. Moderators will guide the discussion.

1:30 – 3:30 Break out via five designated themes – Definition of research questions
3:30 – 4:00 Break and re-shuffle
4:00 – 5:30 Small group discussions with intermingling of themes to address specific cross-disciplinary questions

7:00 PM – 9:00 PM Dinner at Trattoria Sorrento (0.45 mi from hotel)
4930 Cordell Ave, Bethesda

Tuesday, November 17th

7:30 AM Continental Breakfast
Location: Continuous break area, level II

8:30 – 11:15 AM General Session (all participants)
Location: Grand Ballroom D
Moderators’ reports: Moderators will review the plan developed by their group: 15 min presentation + 15 min discussion for each topic area
Clinical Issues 8:30 - 9:00
Obesity 9:00 – 9:30
Mental Health 9:30 - 10:00
Break 10:00- 10:15
Genetics 10:15 -10:45
Animal Models 10:45 -11:15

The remainder of the meeting will be spent addressing unanswered or incompletely answered questions as identified by participants. Issues of resource development, training, and fostering cross-disciplinary studies will be addressed in the context of research needs and opportunities.
11:30 AM - 12:30 PM  Working Lunch  
Location: Grand Ballroom D

12:30-2:00 PM  Finalize Research Strategy

2:00 PM  Prader-Willi Research Strategy Workshop concludes

**Goal:** At the end of the Workshop, participants will have developed a comprehensive research strategy for PWS that will accelerate efforts to understand the disorder at the molecular, cellular and systems level, and ultimately lead to more effective therapeutic interventions. A draft report will:

- Identify gaps in knowledge
- Prioritize basic and clinical research questions
- Ascertain existing resources and technologies that can be applied to the research questions
- Identify needed resources and technologies
- Develop approaches to foster cross-disciplinary collaborations and training opportunities / initiatives

**Planning Committee:**
Theresa Strong, PhD    University Alabama at Birmingham (Chair)
Dan Driscoll MD, PhD    University of Florida
David Eckstein PhD    Office of Rare Disease Research, NIH
Uta Francke MD    Stanford University
Tony Holland MRCP    University of Cambridge
Stephen O’Rahilly MD    University of Cambridge
Rachel Wevrick PhD    University of Alberta

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QUESTIONS TO BE CONSIDERED IN THE MONDAY BREAKOUT SESSION  1:30 – 5:30 PM

For each area [Clinical Issues, Obesity, Mental Health, Genetics, Animal Models]:

What are the current areas of strength?

What are the most pressing research questions that need to be addressed to move the field forward?

What are areas of weakness in addressing those questions?

What resources are currently available that might be applied to these problems?  (including infrastructure, IT, financial, and biological)

What resources are currently unavailable, but would significantly impact the field?

What training opportunities exist?  Are needed?

What are the opportunities for collaborations/multidisciplinary approaches, what are potential areas of cross-fertilization?  What additional areas of expertise are needed to efficiently address research questions?

How can the link between basic and clinical research be strengthened?

What approaches can be taken to foster research in target areas?