Challenge: Improving clinical trial participation

Diane Webster (PhD), Research Director

Skyla, 2
Tom, 7
Mitchell, 9
Olivia, 11
Developing PWS therapies

- Basic Science/ Clinical Science
- Target Identification
- Target Validation and optimisation
- Pre-clinical
- Clinical Trials (phase 1 – 3)
- Regulatory & commercialisation

Infrastructure (eg, Patient and Clinical Registries, Biobanks)
PWS Clinical Trials

Number of participants:
- Phase 1: 5 – 15
- Phase 2: 30 – 40
- Phase 3: 120 - 150

Regulatory approval & reimbursement
The Challenge:
How can we accelerate the development of new drugs for PWS by improving enrolment in clinical trials?

Discussion Starters:

• Reaching more of our community
  o getting in touch with the families who are not on our mailing lists?

• What’s not working – and how might we address these barriers?
  o including language barrier (HC-QT often needs to be completed in English)

• What is working (facilitators)?

• Matchmaking – how do we find the right trials for interested participants?
Prader Willi Research Foundation Australia

Our vision is to improve clinical outcomes and deliver better treatments for people living with PWS