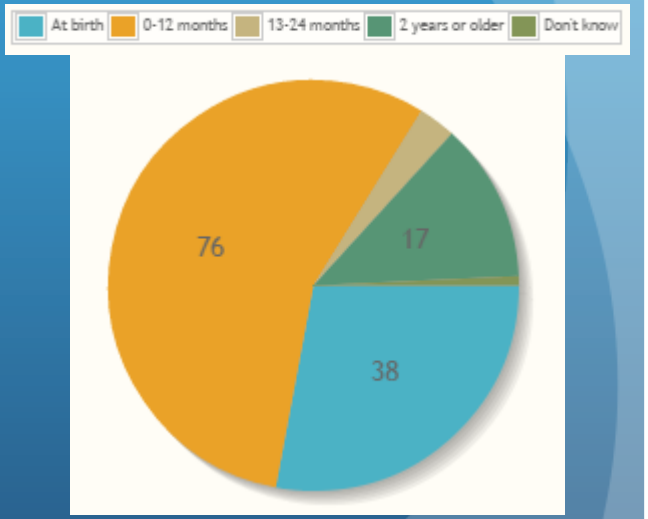
GLOBAL
PRADER-WILLI SYNDROME
REGISTRY

It starts with us.
Enroll today!

What is the PWS Registry?

- The purpose of the registry is to develop a comprehensive database of individuals with PWS
  - to better understand the full spectrum of PWS characteristics
  - to expedite the completion of clinical trials
  - to determine areas of needed research and treatments
  - to improve the lives of those affected by PWS.

What was the participant's age in years when diagnosed with PWS? (n=136)

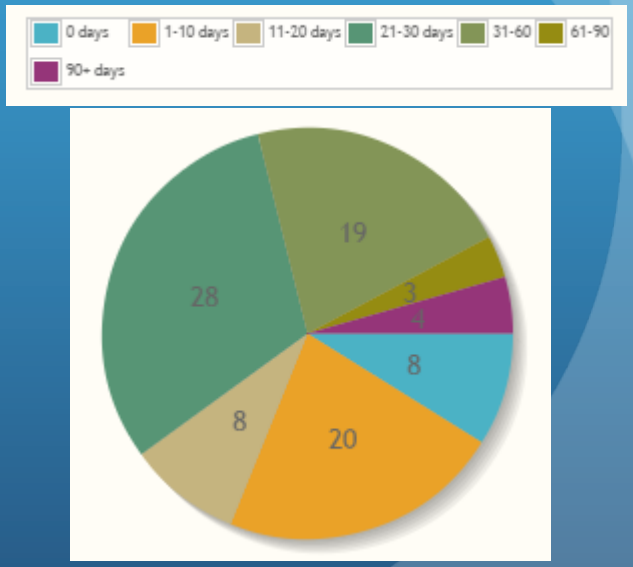


Participant = person with PWS

Is the data in the Registry secure?

- The Global PWS Registry is the ONLY IRB approved PWS data repository with the power to answer our many questions!
- The registry collects patient data through a series of “surveys”.
- Data is de-identified and privacy is secure.
- De-identified data can be ‘mined’ to find trends and answer questions.
- De-identified data may be used for research and publications.

After delivery, how many days did the participant stay in in a NICU? (n=90)



Participant = person with PWS

What is an IRB?

- The PWS Registry is an IRB (Institutional Review Board) approved research study
- IRB
  - In accordance with FDA regulations
  - An approved research ethics group that reviews/monitors biomedical research involving human subjects
  - Tasked with ensuring the protection of rights and welfare of research participants
- The registry has an IRB approved protocol and informed consent
  - Detailed documents laying out how data will be collected and used (these are available to everyone)

CHESAPEAKE IRB
Human Subject Protection Experts

What is informed consent? Assent?

- *Informed consent document (IRB reviewed/approved)*
  - *Explains the purpose, duration and description of the research*
  - *Describes any reasonably foreseeable benefits and risks*
  - *Describes how data will be collected and used*
  - *Details the privacy/protection of data*
  - *Provides contact information to ask any and all questions regarding the information provided*

- Upon a clear appreciation and understanding of the information presented, a participant provides "Informed Consent" by agreeing to the terms of the consent document

- For the PWS Registry, informed consent will usually be provided by a parent or legal guardian, on behalf of the person with PWS

Respondent = person answering the questions (e.g. parent/legal guardian)
Participant = person with PWS

What is Assent?

- "Assent" is a term used for those who are able to understand the terms, goals, benefits, risks, of the Registry in general, but are too young, or not legally able to provide informed consent. For example:
  - Adolescents age 14-17
  - Adults over 18, but still have a legal guardian
- This “informed assent” step encourages parents and legal guardians to discuss the Registry with a participant, and include them in the decision making process.
- They can also contribute useful information to help answer questions, particularly those pertaining to quality of life and well-being.

The Registry is composed of surveys which cover topics including:

- Birth history
- Developmental milestones
- Endocrinology
- Therapies
- Behavior/Mental health
- Supplements
- Medications
- Complete clinical/ medical history

Surveys can be completed individually

Opportunity to login/out, update information, complete new surveys, explore aggregate data

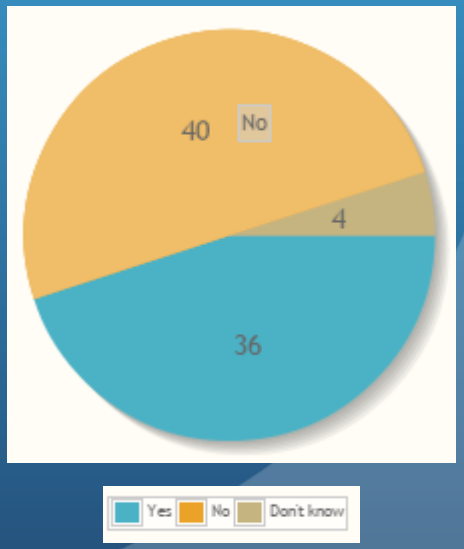
The PWS Registry is the **single most powerful tool** we have as a community to advance PWS research.

- **The Registry Will:**
  - Document the full range of PWS characteristics
  - Enable data trend analysis to generate new insights and identify areas for additional study
  - Facilitate partnerships with university researchers and pharmaceutical companies
  - Guide the development of standards of care
  - Expedite the completion of PWS Clinical trials
  - Allow participants to store their PWS medical data in one place
  - Accelerate solutions for PWS

The Registry will be able to answer many of the questions we have about PWS.

- What is the average age hyperphagia kicks in?
- What is the chance my child will develop scoliosis?
- What percentage of individuals with PWS receive Growth Hormone?
- And more!
- Bonus: As you respond to the surveys, you will see how your answers compare to the rest of the participating PWS community!

Has the participant ever been diagnosed w/ a back deformity (sciolosis/lordosis/kyphosis)? (n=80)



Benefits of participation?

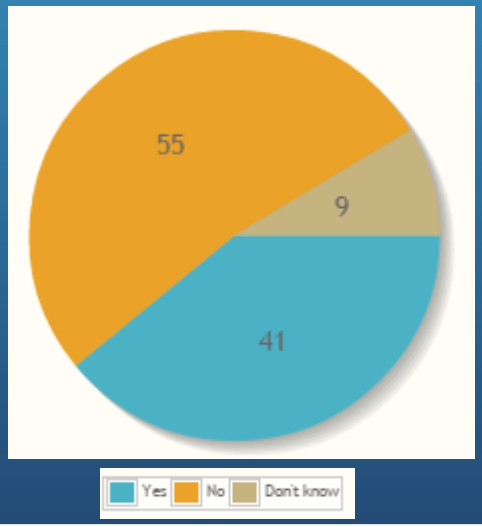
- You will be able to compare your responses against those of the participating PWS community.
- De-identified data will be used to search for trends and answer questions such as: is the UPD subtype more susceptible to austism? Or How many 18 year olds with PWS go to college?
- Researchers will be able perform statistical analysis on de-identified data to answer their research questions
- Patients interested in participating in clinical studies may elect to be contacted if they match eligibility for an upcoming study.

The Power of the PWS Registry relies on community participation

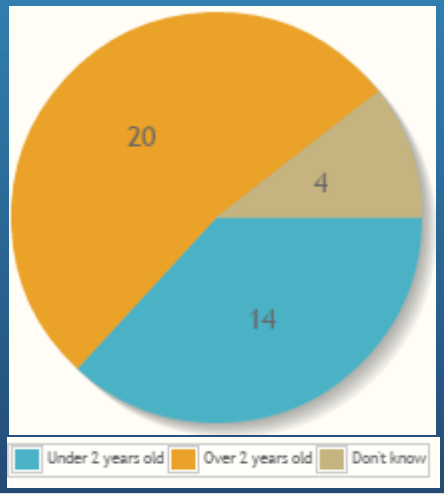
- The faster we can collect a statistically significant amount of data, the faster we can begin making the registry work for us!
- Enroll today!! www.pwsregistry.org

Apraxia in PWS

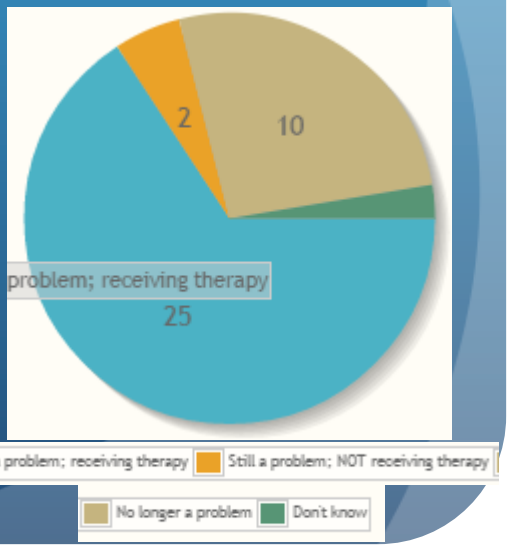
Ever diagnosed with apraxia?



Age at diagnosis?

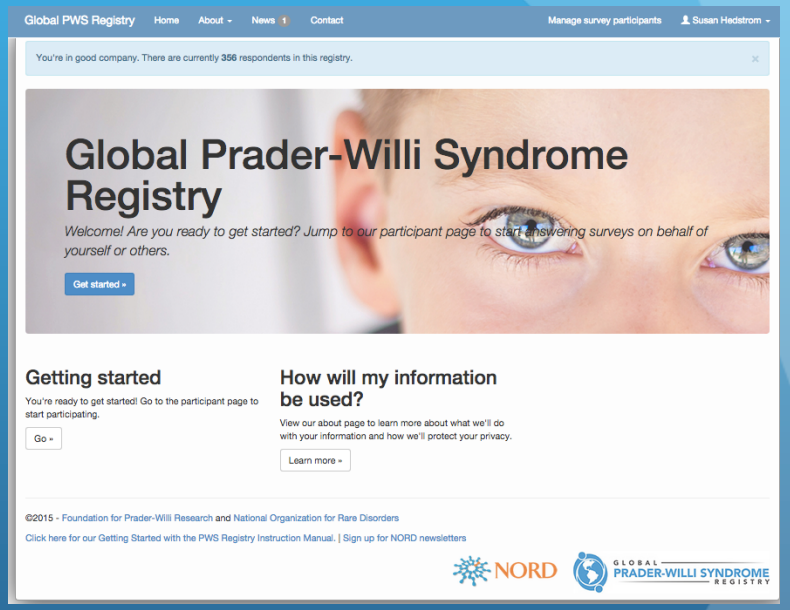


Current status



Resources to Help You Get Started

- Global PWS Registry Group on Facebook
- Getting Started Document: available in footer of registry
- One-on-One assistance: email info@pwsregistry.org



Tips for a smooth registry experience

- Respondent = person answering questions (e.g. parent/legal guardian)
- Participant = person with PWS
- Consent and assent must be provided before proceeding to any surveys. (You can revoke consent at anytime)
- Responses can be “saved as draft”. Log back in to the account to complete and submit surveys as final responses. Once final answers are submitted, they can not be changed

The Save and Submit buttons are hard to find for new users. Look to the bottom right hand corner of your web browser.