

PWS Patient Voices

December 11, 2014

Theresa Strong, PhD
Chair, Scientific Advisory Board
Foundation for Prader-Willi Research

Beloranib

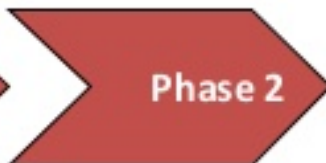
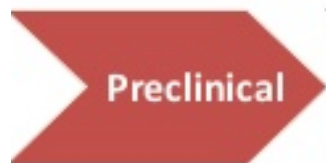
Oxytocin analog

Diazoxide

RM 493

AZP-531

oxytocin



CDER's Benefit-Risk Framework

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition		
Current Treatment Options		
Benefit		
Risk		
Risk Management		
Benefit-Risk Summary and Assessment		

FDA Safety & Innovation Act 2012-Prescription Drug User Fee



U.S. Food and Drug Administration
Protecting and Promoting Public Health

www.fda.gov

Patient-Focused Drug Development

- Patients are uniquely positioned to inform FDA understanding of the clinical context
- FDA could benefit from a more systematic method of obtaining patients' point of view on the severity of a condition, its impact on daily life, and their assessments of available treatment options
 - Current mechanisms for obtaining patient input are often limited to discussions related to specific applications under review, such as Advisory Committee meetings
- Patient-Focused Drug Development initiative offers a more systematic way of gathering patient perspective on their condition and treatment options
 - FDA will convene at least 20 meetings on specific disease areas over the next five years
 - Meetings can help advance a systematic approach to gathering input



Jim Kane
Janalee Heinemann
Rob Lutz
Theresa Strong



Prader-Willi Syndrome Patient Voices Survey

Purpose: Early step in making sure the voice of the PWS patient is known, accurately represented and understood at the FDA

Method: Anonymous online survey to gather information about patient (parent) views on PWS impact, severity, effectiveness of current treatment options, and attitudes towards clinical trials

Caveats: online survey (no method to confirm diagnosis), questions not extensively validated

October 21 – November 3, 2014

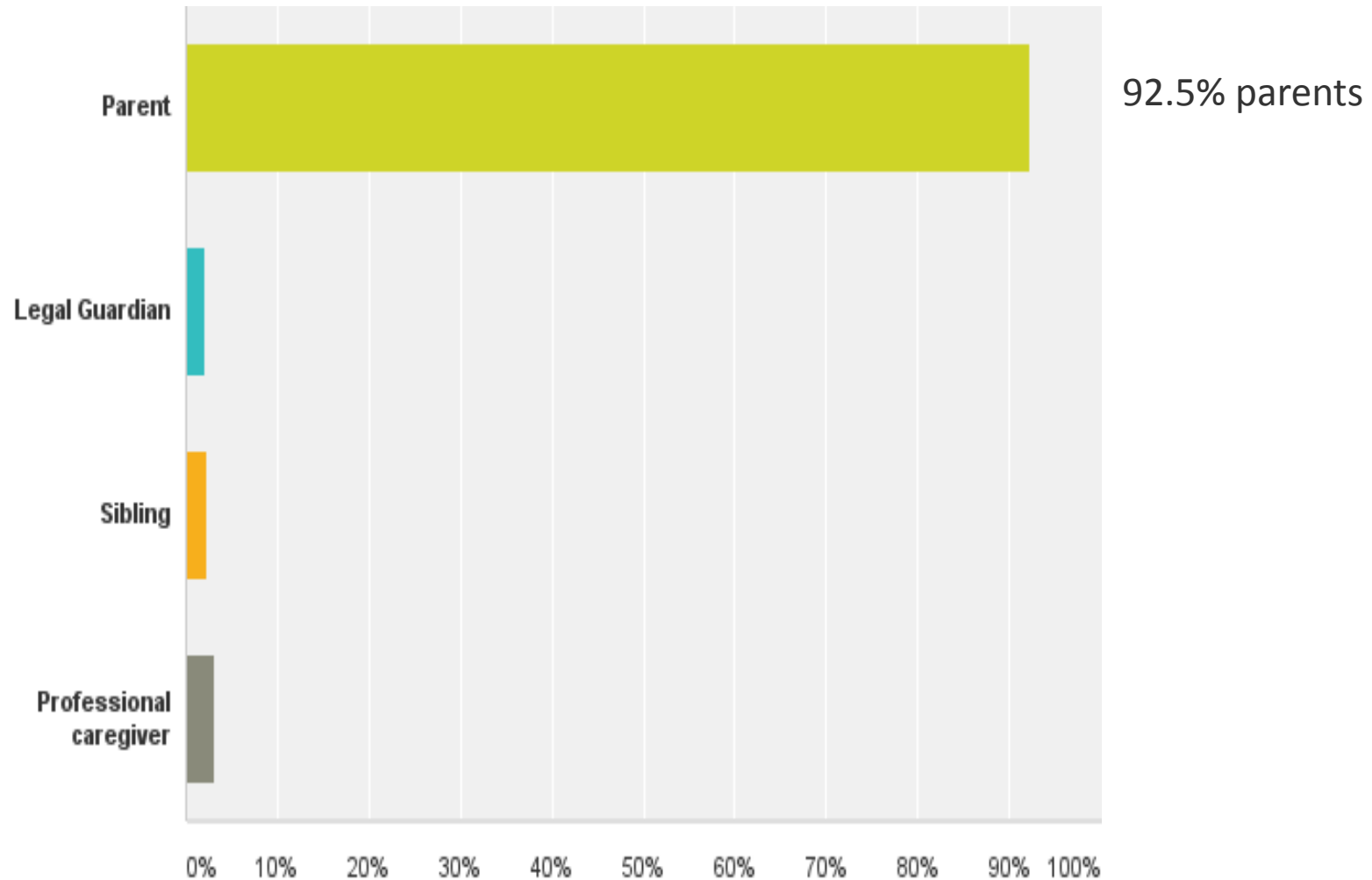
Distribution: email blasts, Facebook

779

Total Responses

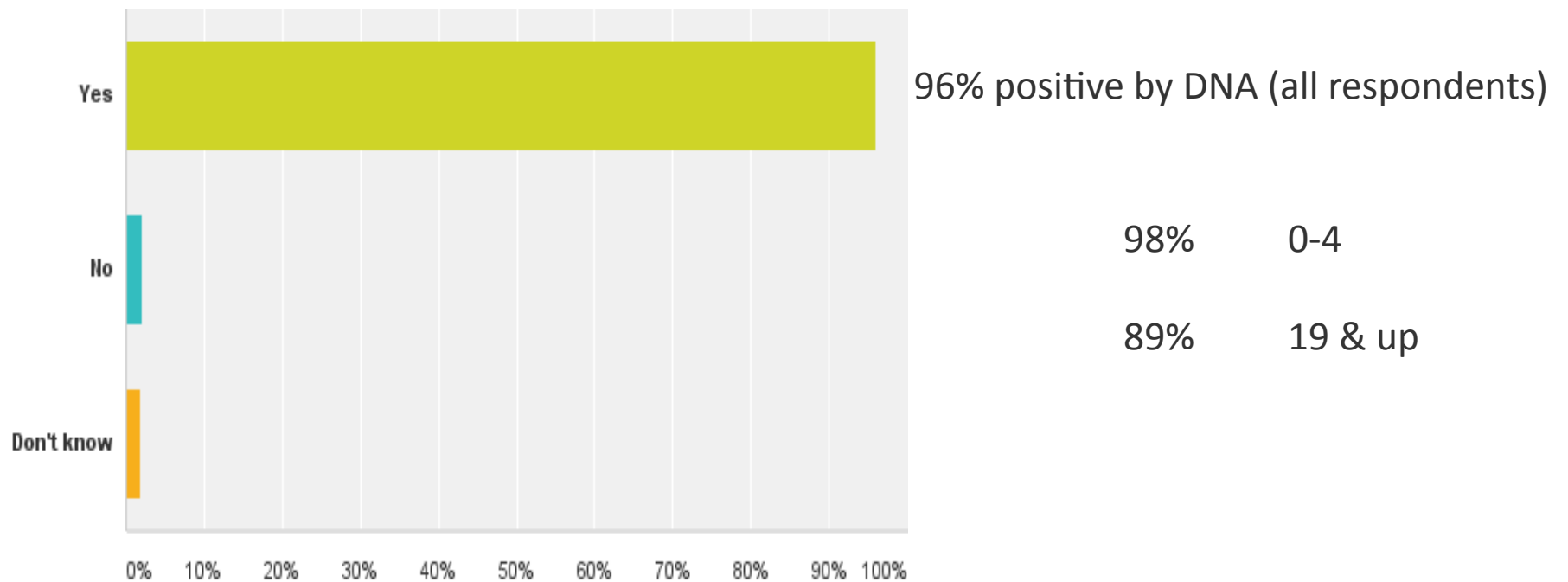
Q1: What is your relationship to the person with Prader-Willi syndrome (PWS)?

A: All



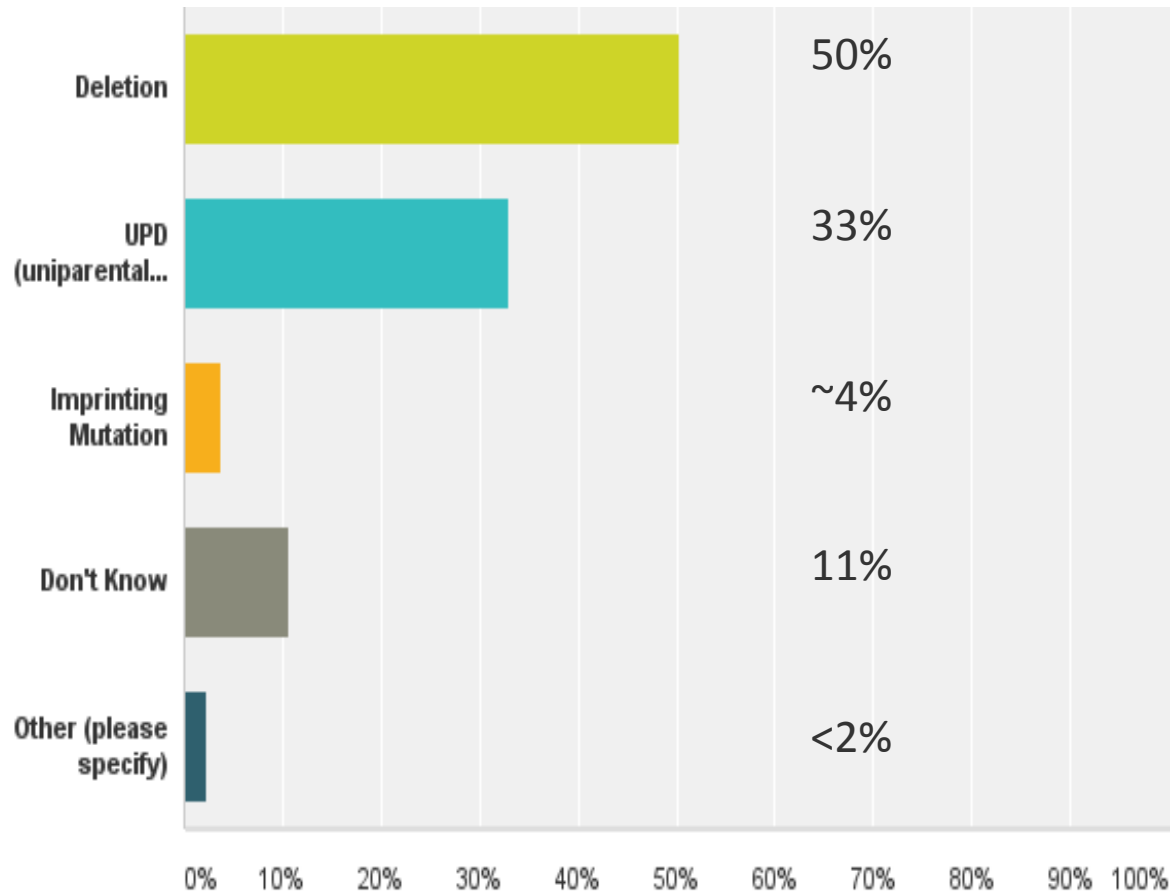
Q2: Has the person with PWS received a diagnosis based on a genetic (blood/DNA) test?

Answered: 779 Skipped: 0



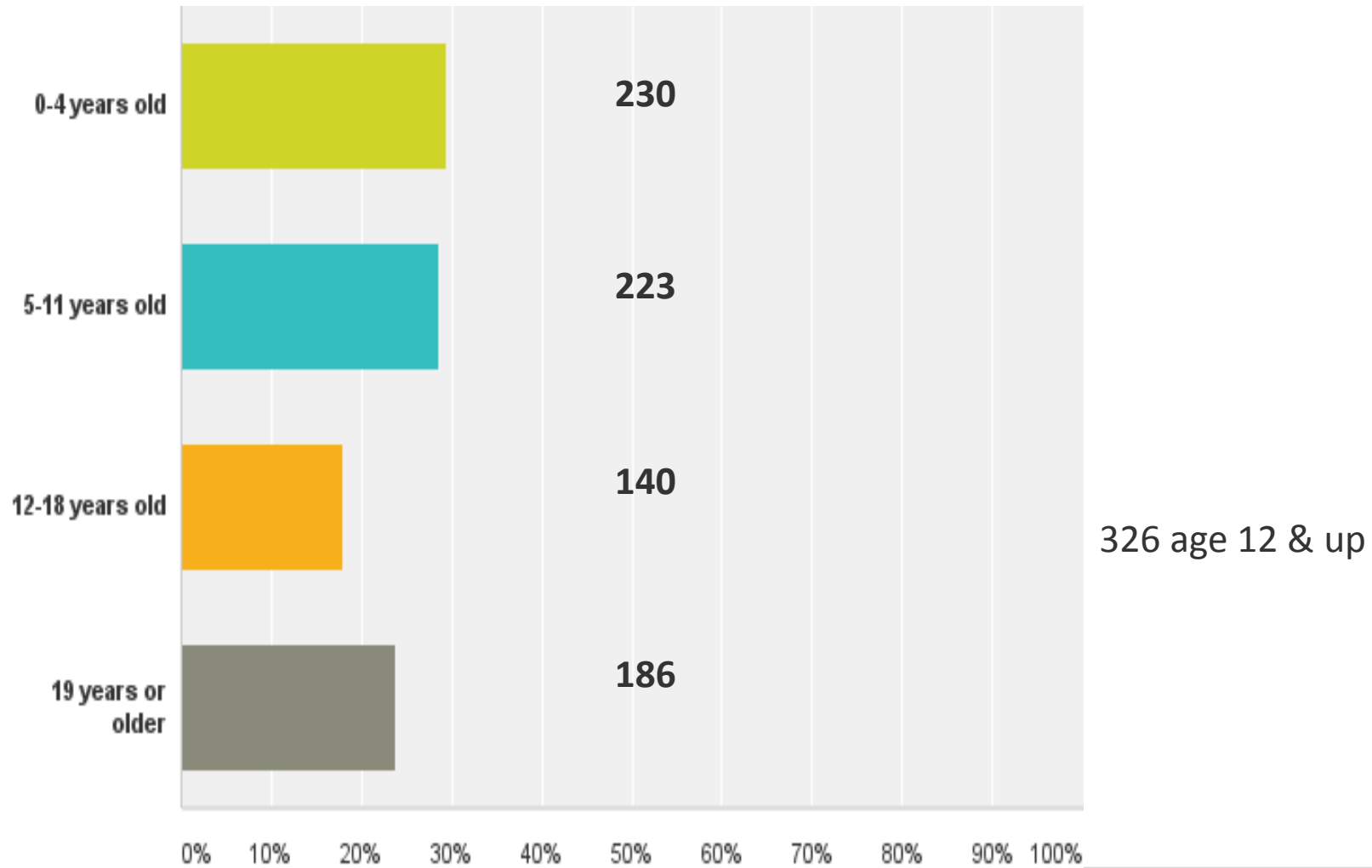
Q3: What genetic subtype does the person with PWS have?

Answered: 779 Skipped: 0



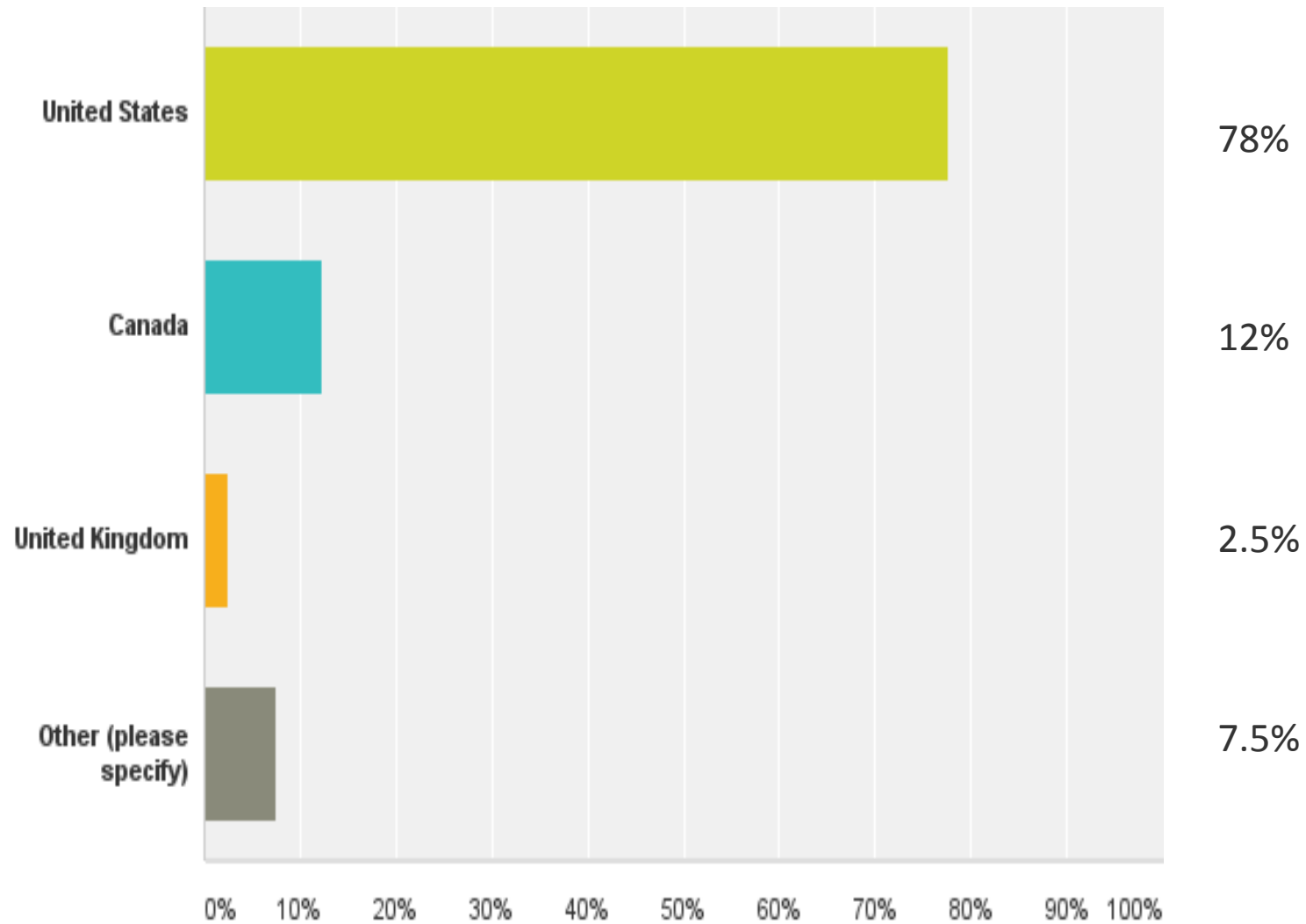
Q4: What is the age of the person with PWS?

Answered: 779 Skipped: 0



Q5: What is your country of residence?

Answered: 779 Skipped: 0



Symptoms and Impact

What symptoms of PWS has your loved one experienced to date; what symptoms have the biggest day to day impact? Longterm?

How significantly has PWS impacted the affected person, their primary caregiver and their family, and in what way?

Symptoms

What major symptoms has the person with PWS experienced so far? (*check all that apply*)

Hypotonia / weak muscles

Feeding problems (infants)

Developmental delay / Intellectual disability

Growth hormone deficiency

Hypogonadism / incomplete sexual development

Scoliosis

Gastrointestinal problems / chronic constipation

Sleep problems / Apnea / Daytime sleepiness

Hyperphagia (excessive hunger)

Overweight / Obesity

Skin picking

Difficulty interacting socially

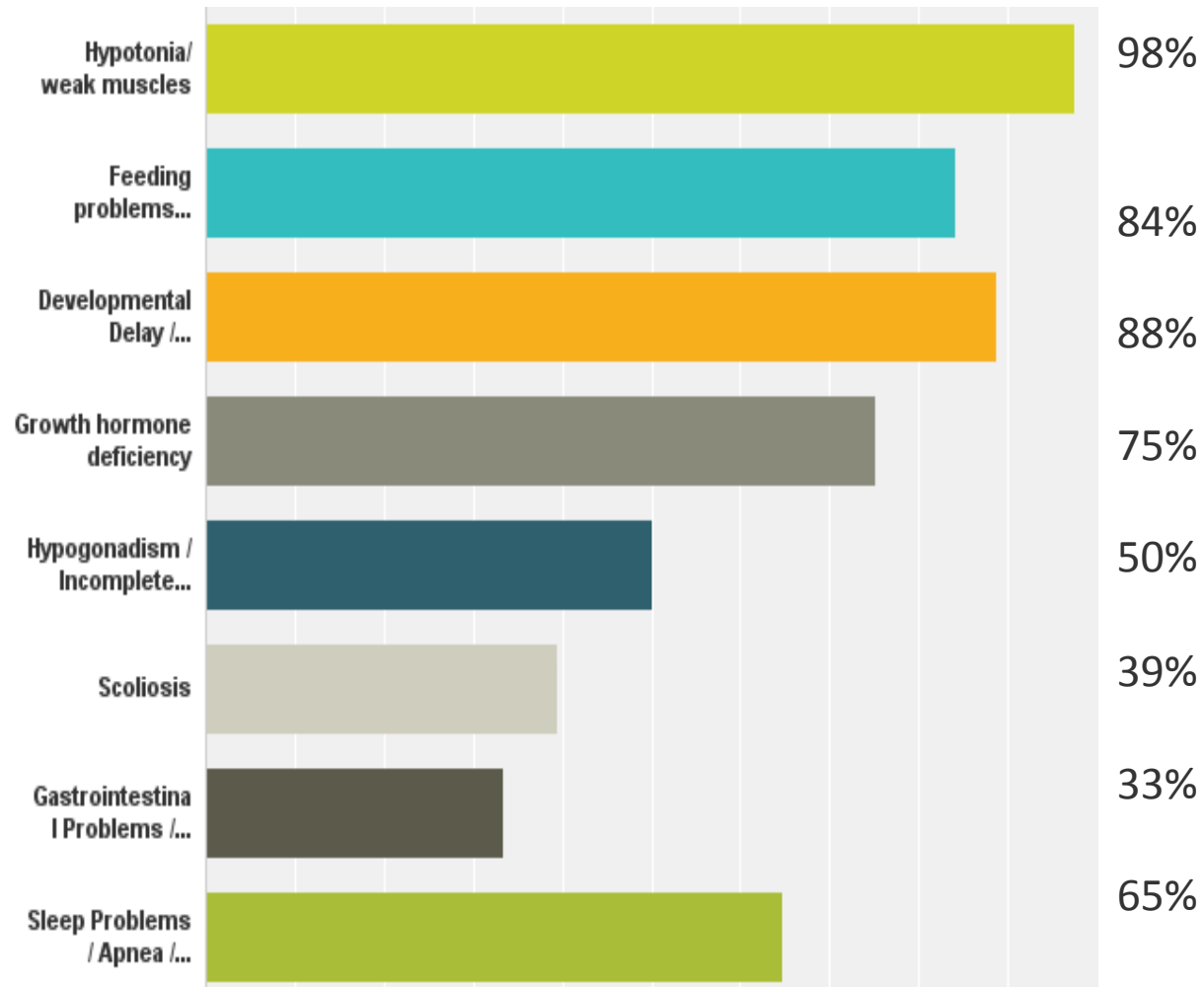
Difficult behavior around food

Difficult behavior, not related to food

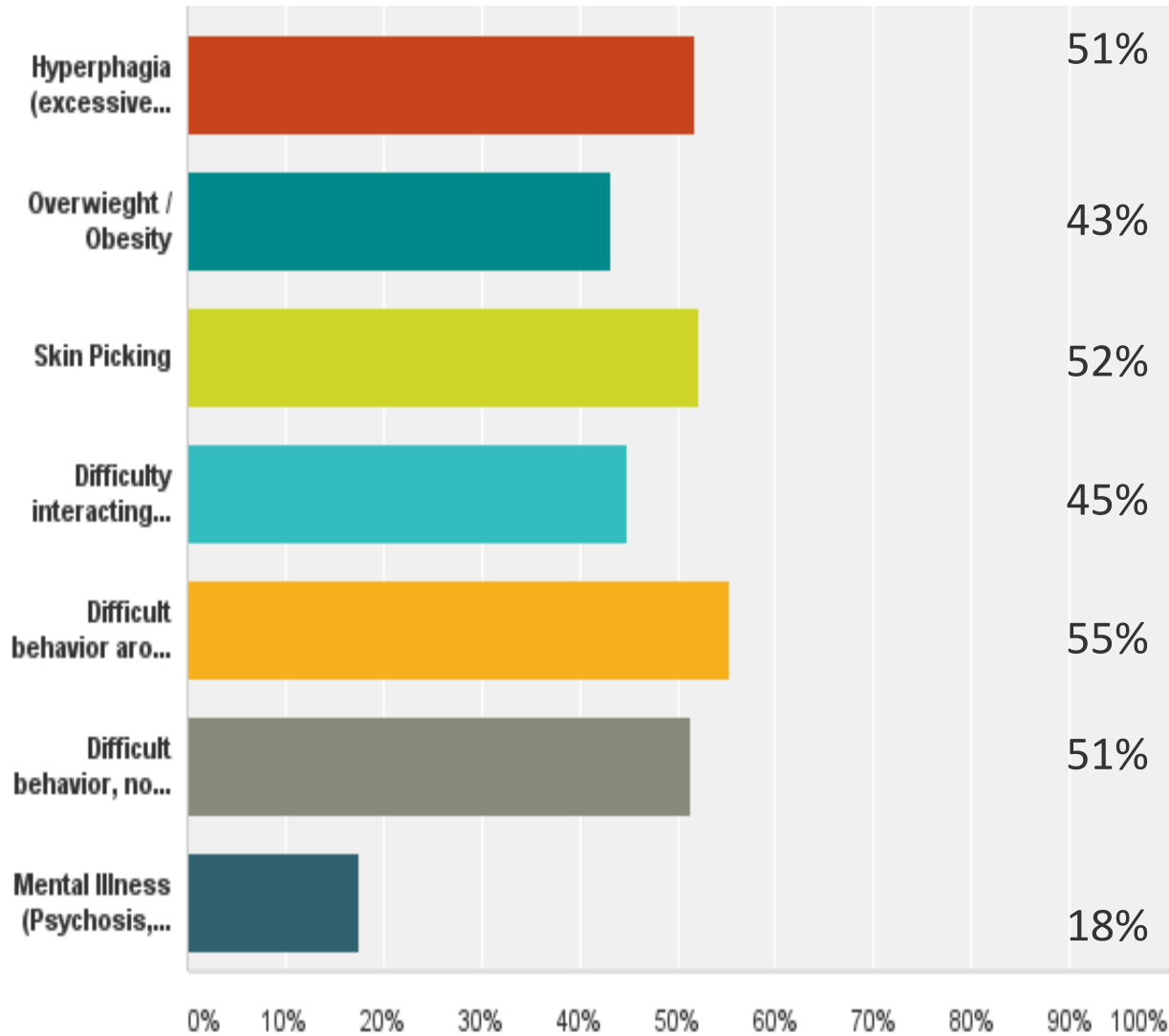
Mental Illness (psychosis, bipolar disorder, depression)

Q6: What major symptoms has the person with PWS experienced so far? (Check all that apply).

A: All

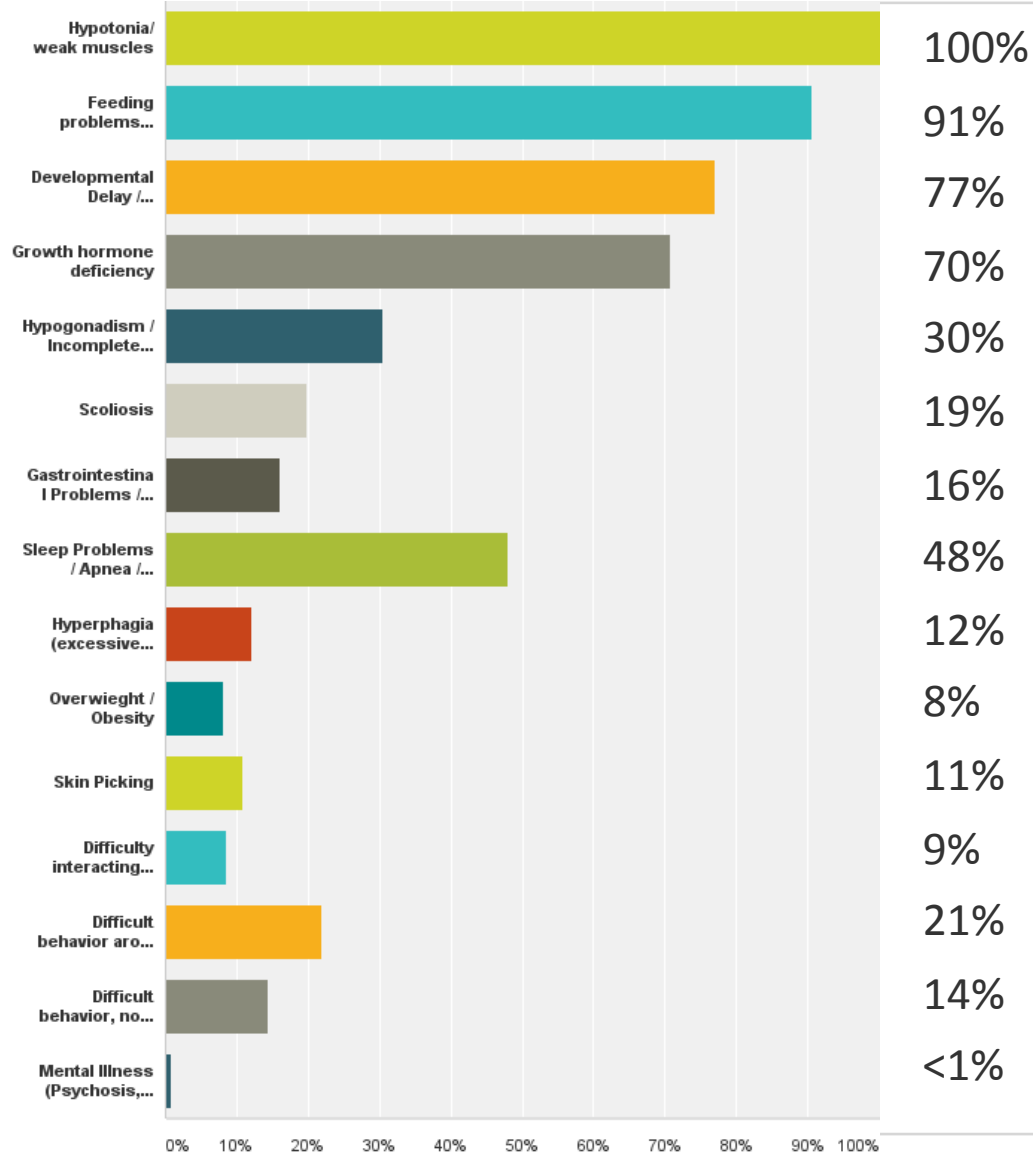


**Q6: What major symptoms has the person with PWS experienced so far?
(Check all that apply).**



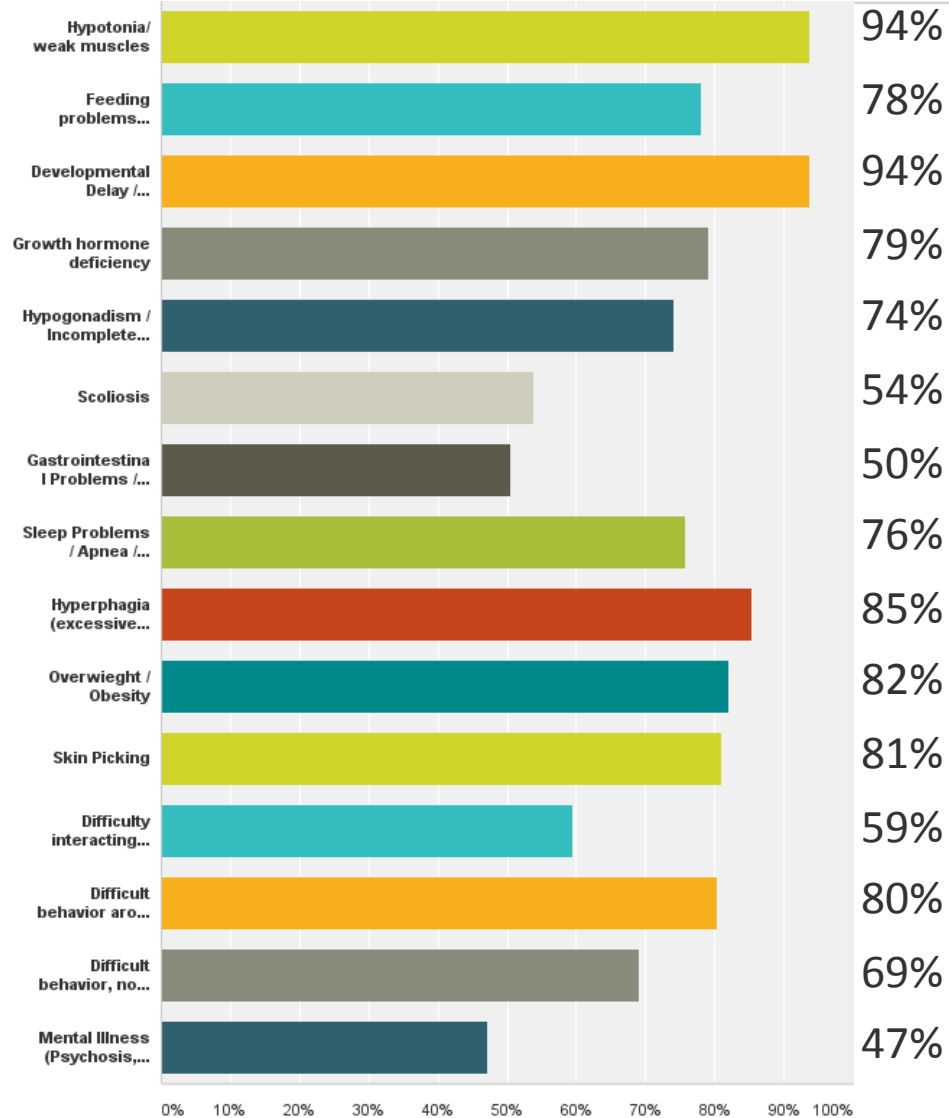
Q6: What major symptoms has the person with PWS experienced so far? (Check all that apply).

0-4 years old



Q6: What major symptoms has the person with PWS experienced so far? (Check all that apply).

19 & up

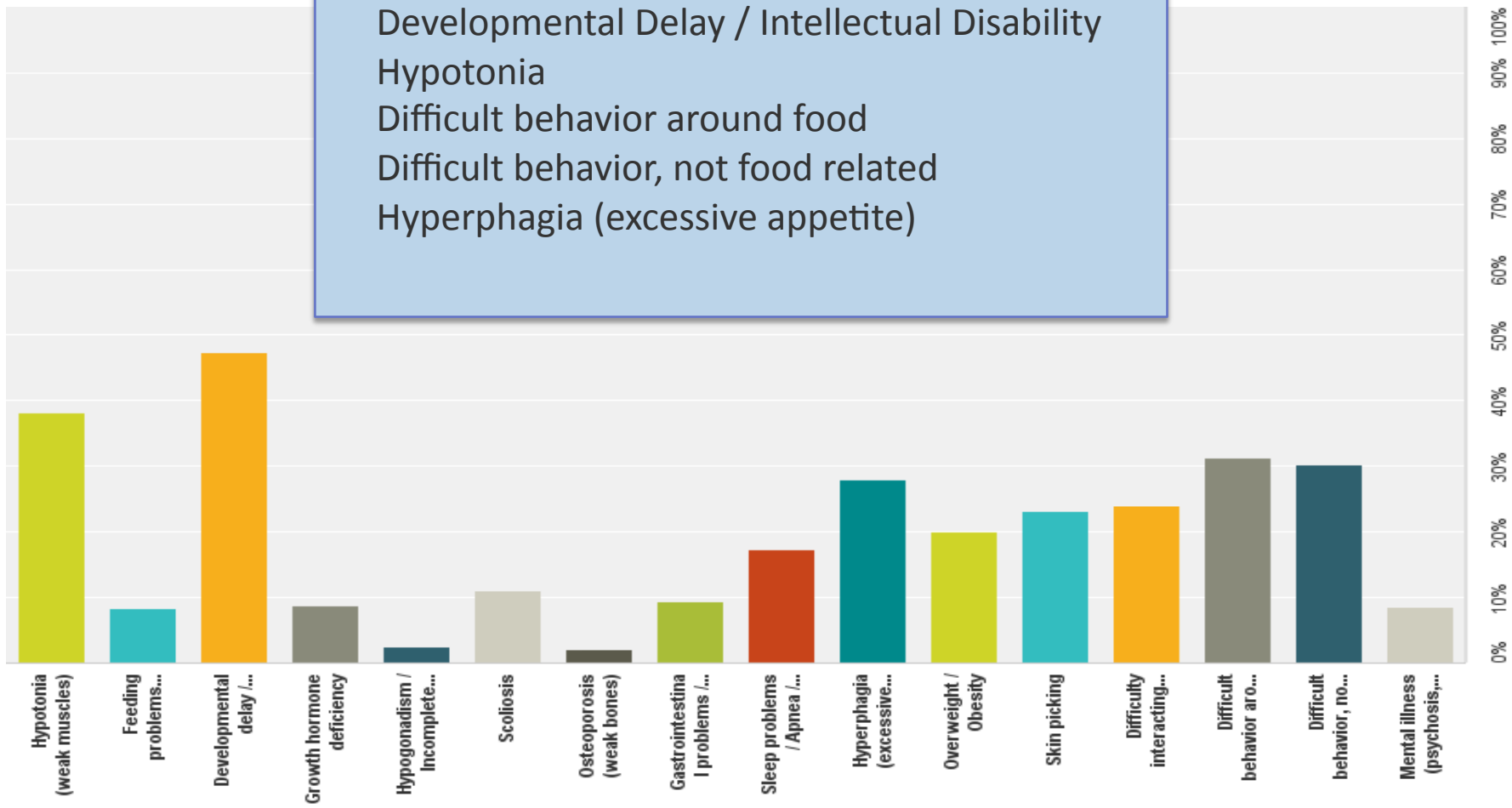


Q7: Which symptoms have the most impact on the person with PWS in their day-to-day living right now? (Choose up to 3).

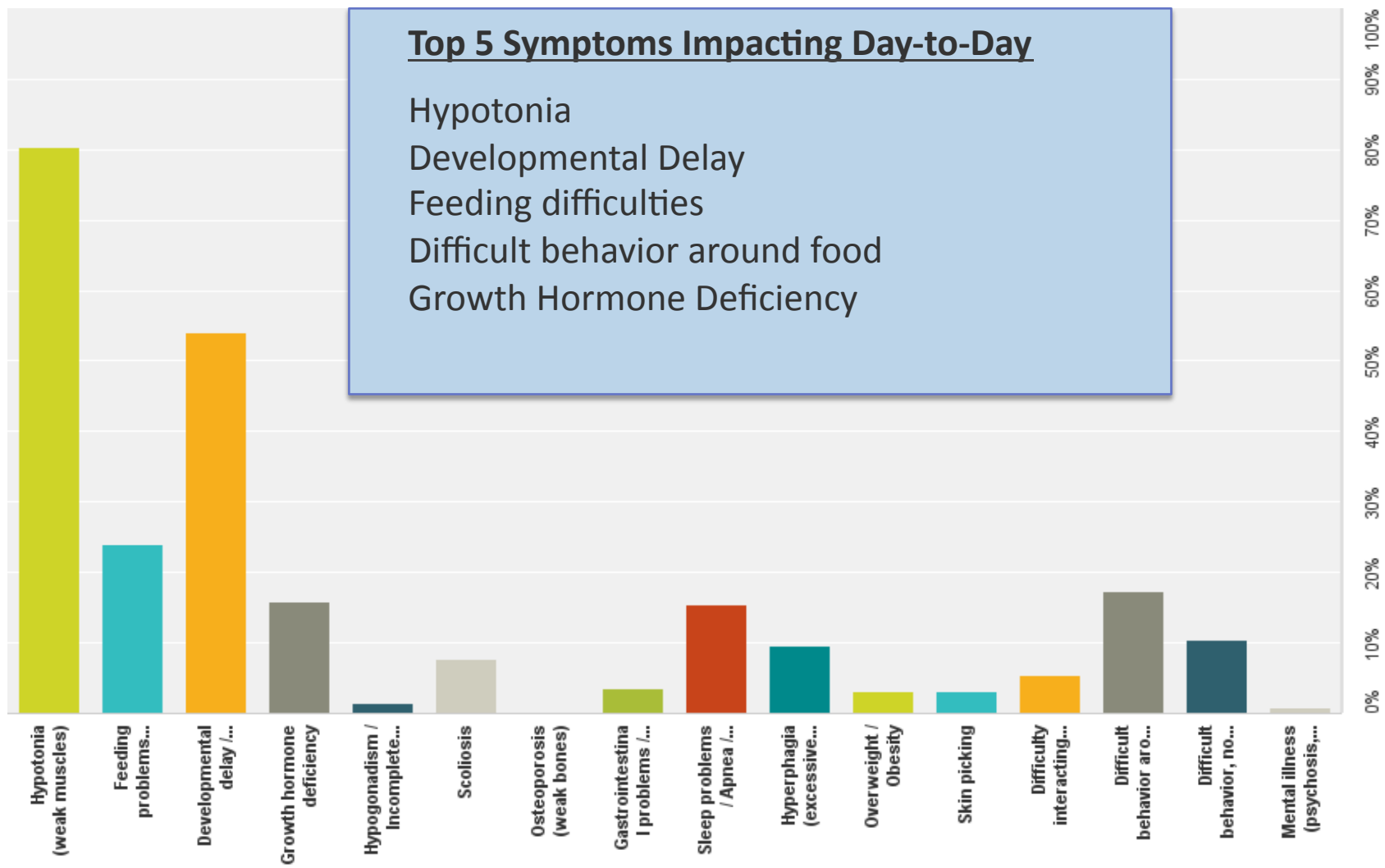
A:ALL

Top 5 Symptoms Impacting Day-to-Day

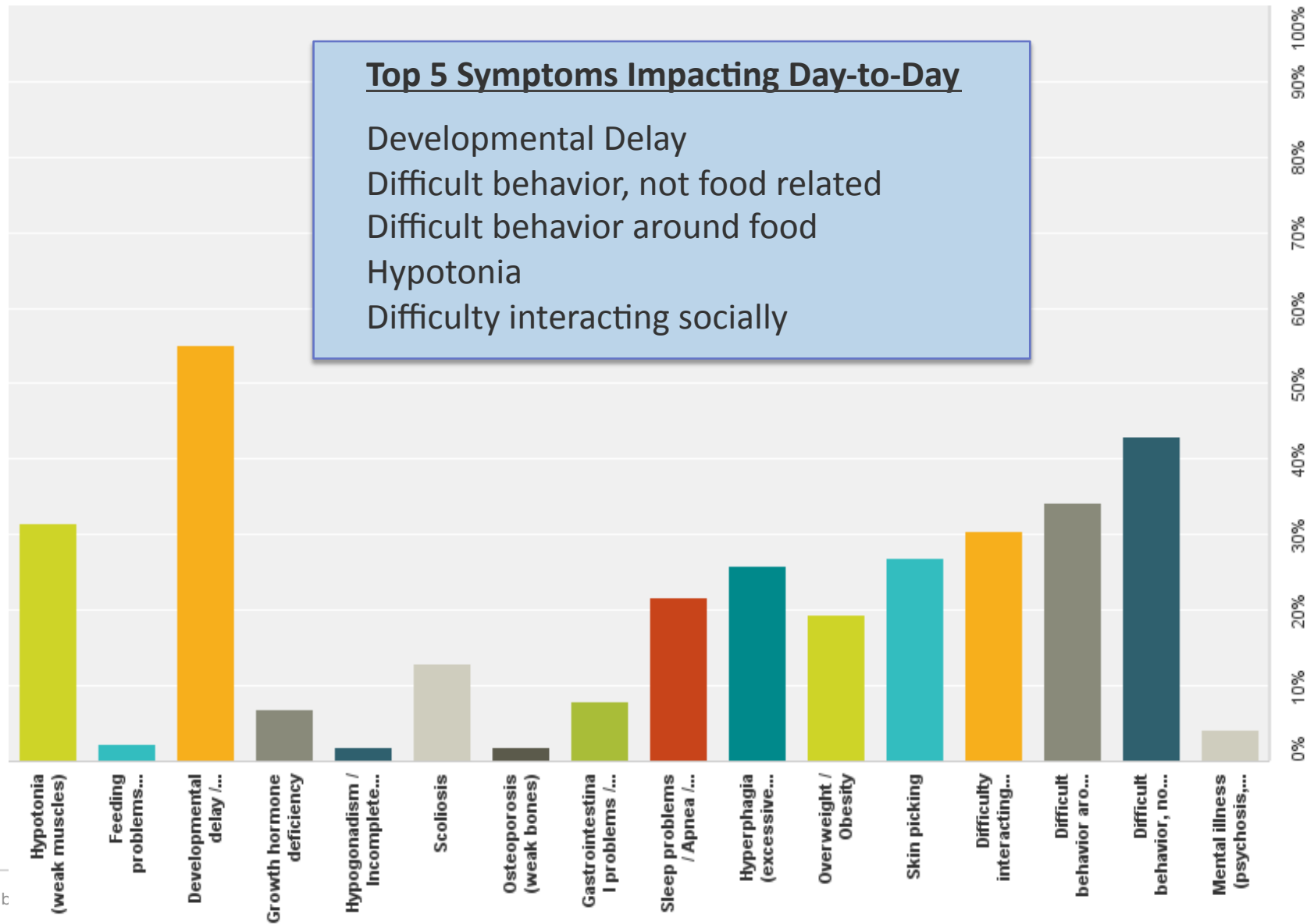
Developmental Delay / Intellectual Disability
Hypotonia
Difficult behavior around food
Difficult behavior, not food related
Hyperphagia (excessive appetite)



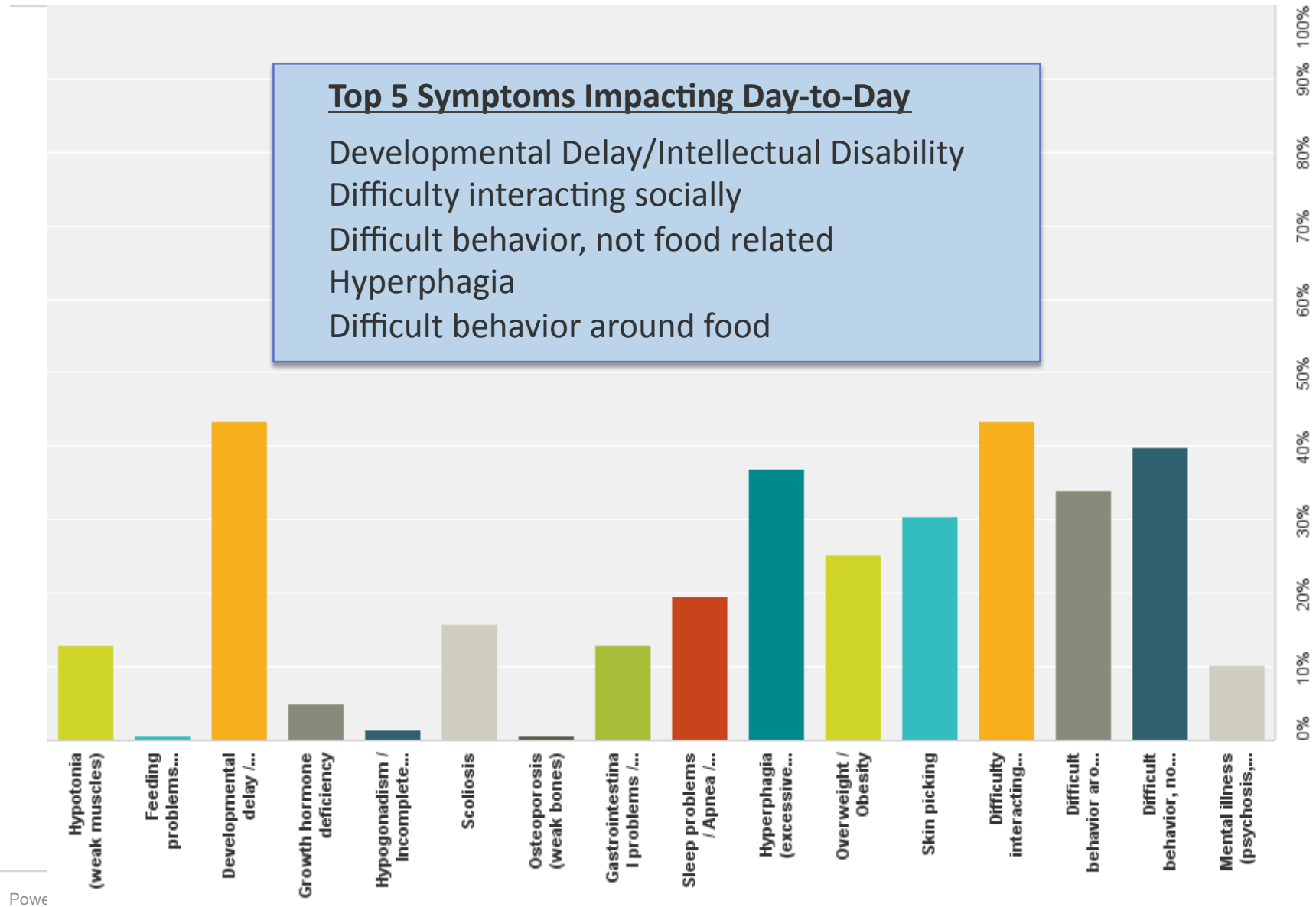
Q7: Which symptoms have the most impact on the person with PWS in their day-to-day living right now? (Choose up to 3). 0-4



Symptoms with biggest day to day impact – 5-11 yrs



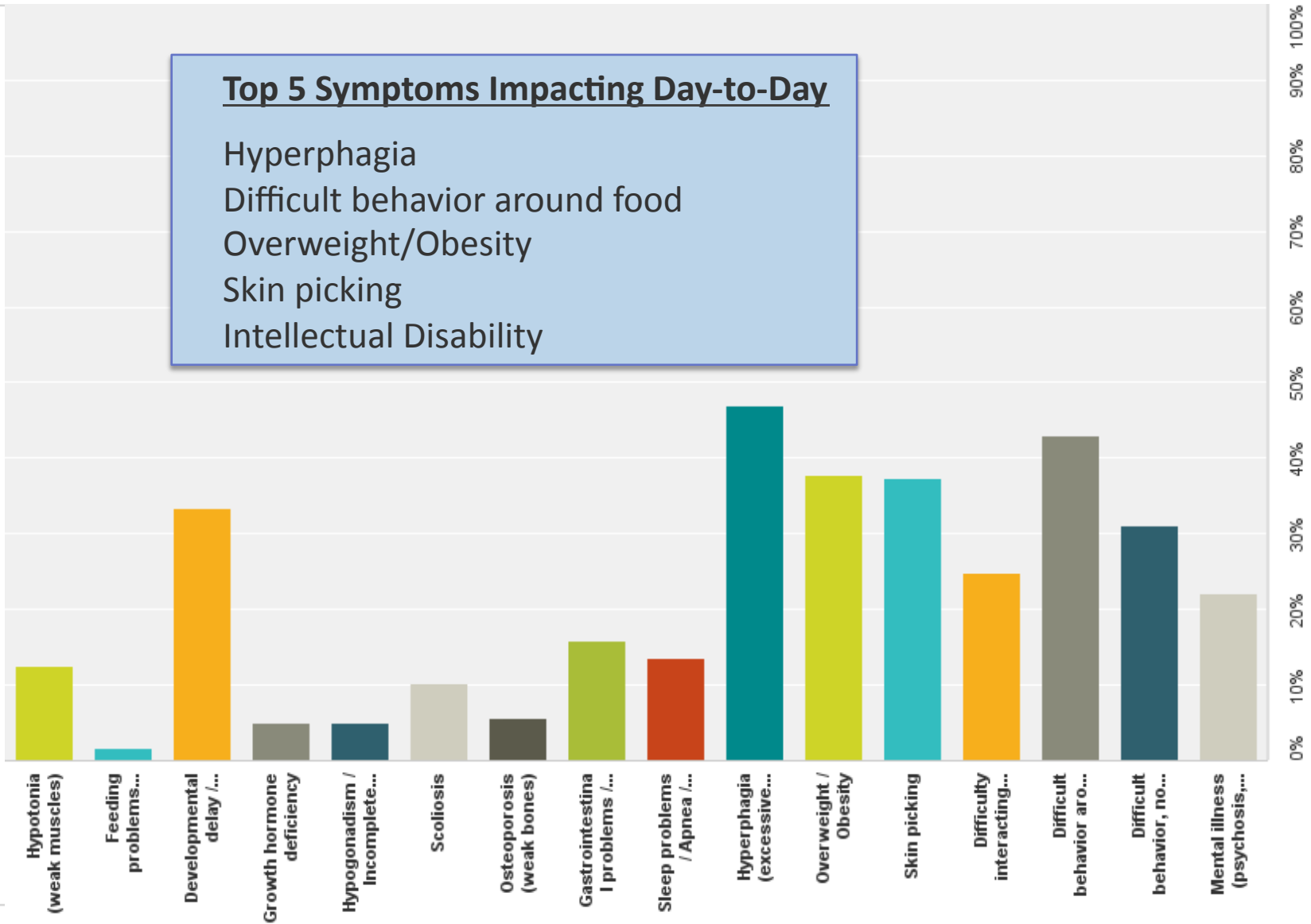
Symptoms with biggest day to day impact – 12-18 yrs



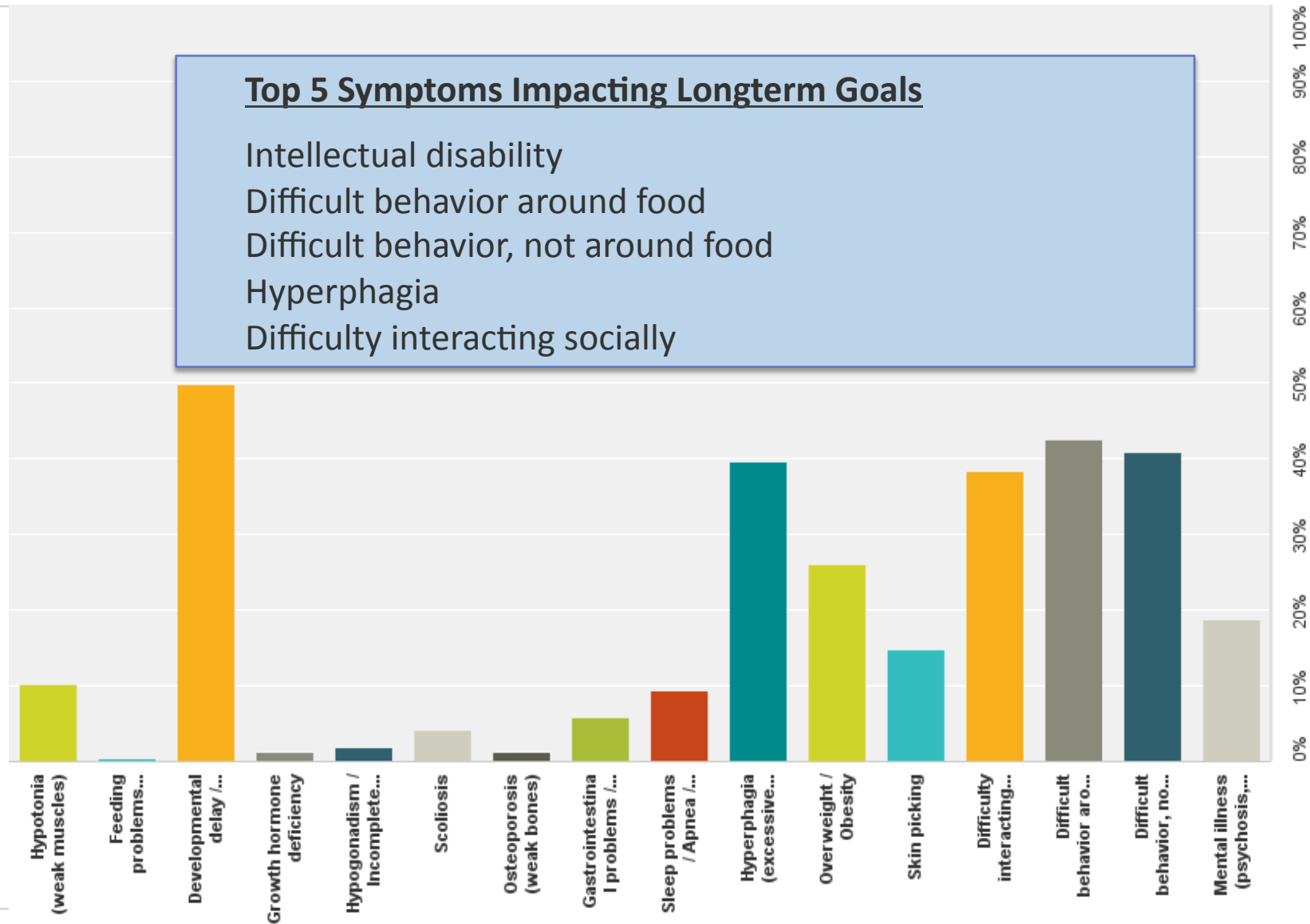
Q7: Which symptoms have the most impact on the person with PWS in their day-to-day living right now? (Choose up to 3). 19 & up

Top 5 Symptoms Impacting Day-to-Day

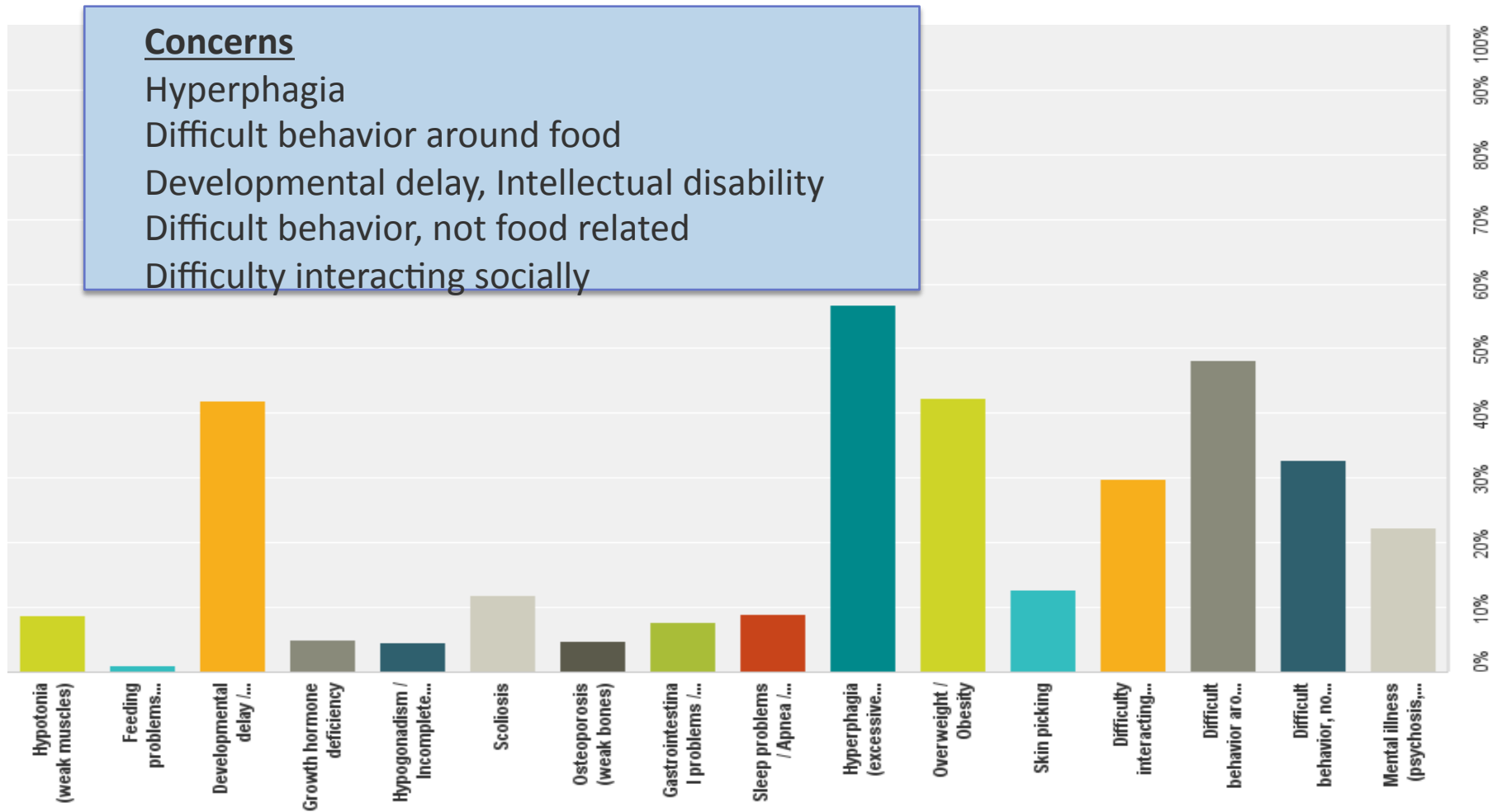
- Hyperphagia
- Difficult behavior around food
- Overweight/Obesity
- Skin picking
- Intellectual Disability



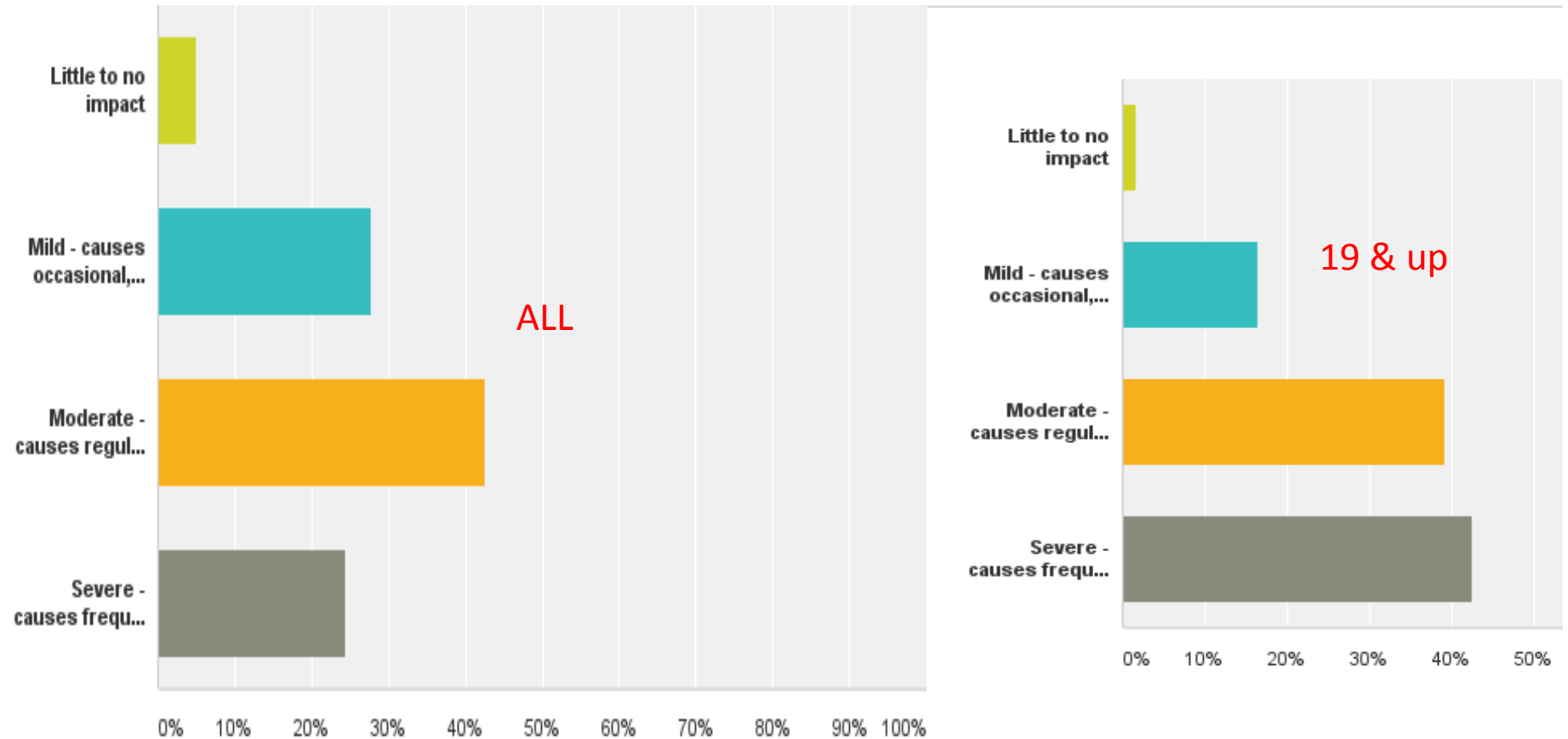
Q8: Which symptoms have had the most significant impact of the life of the person with PWS when considering their ability to achieve long term goals? (Choose up to 3). (12 & up)



Q9: Which symptoms do you worry about when thinking about the future of the person with PWS? (Choose up to 3). (ALL)

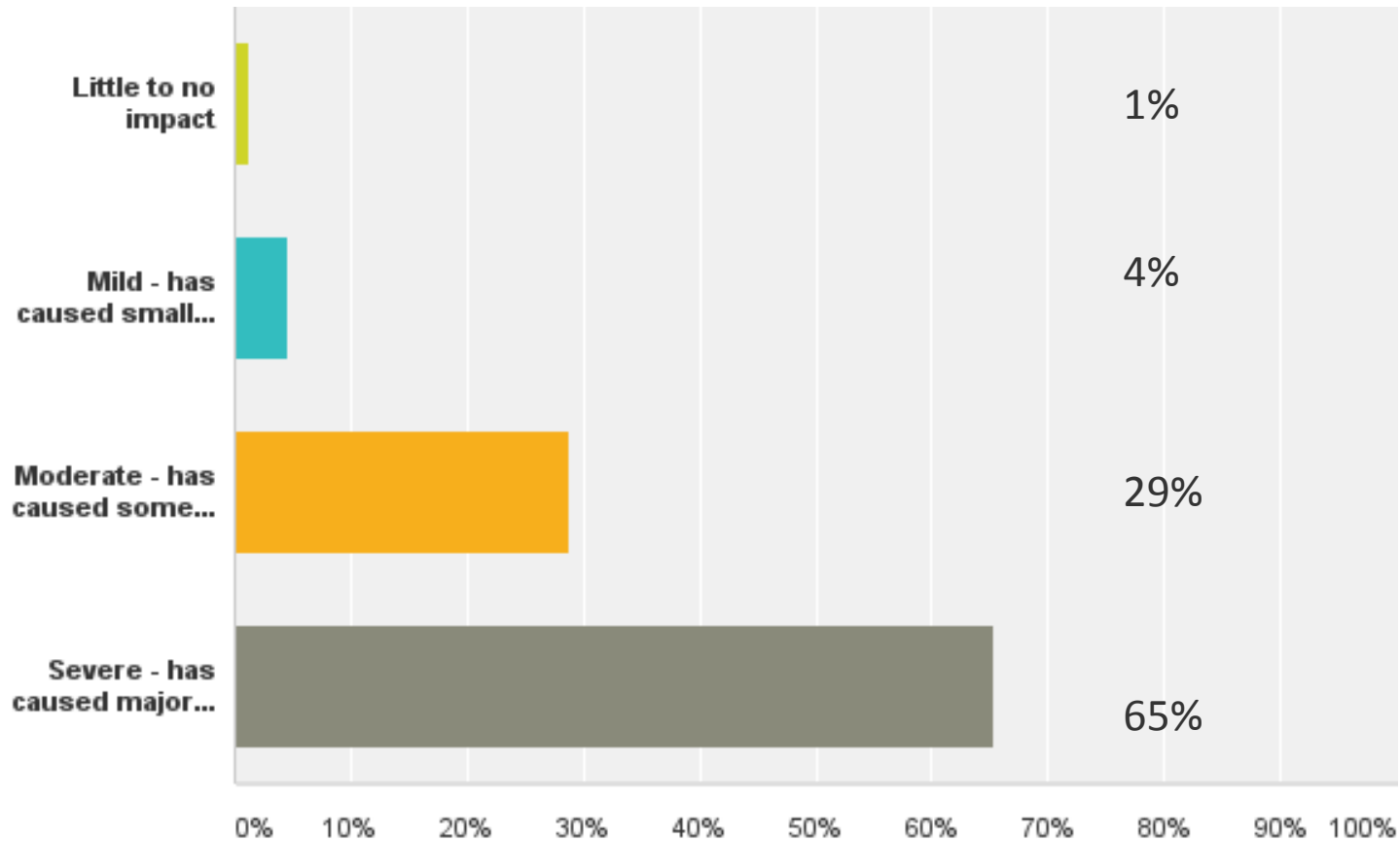


Q10: How would you rate the impact of PWS on the day-to-day life of the person with PWS so far?

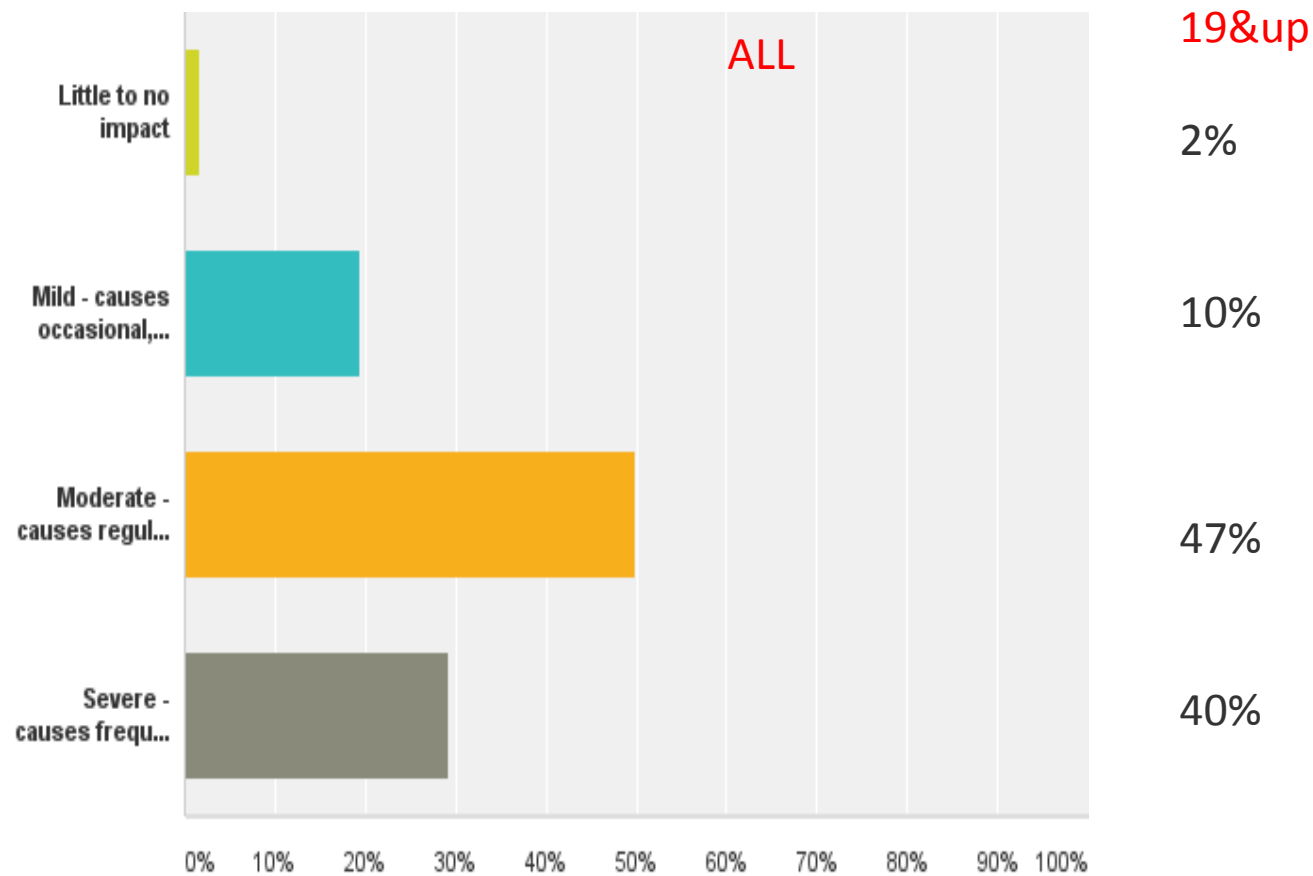


- Little or no impact
- Mild - causes occasional, manageable disruptions in normal daily living
- Moderate – causes regular, moderate disruptions in normal daily living
- Severe – causes frequent and severe disruptions in normal daily living

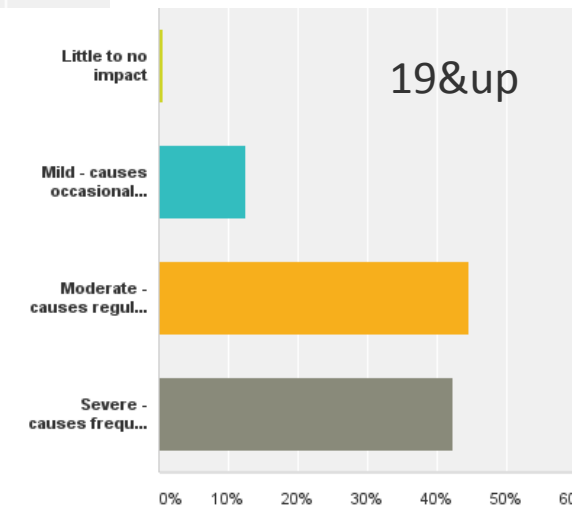
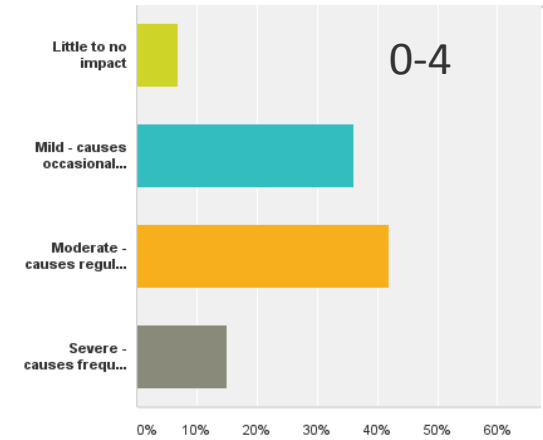
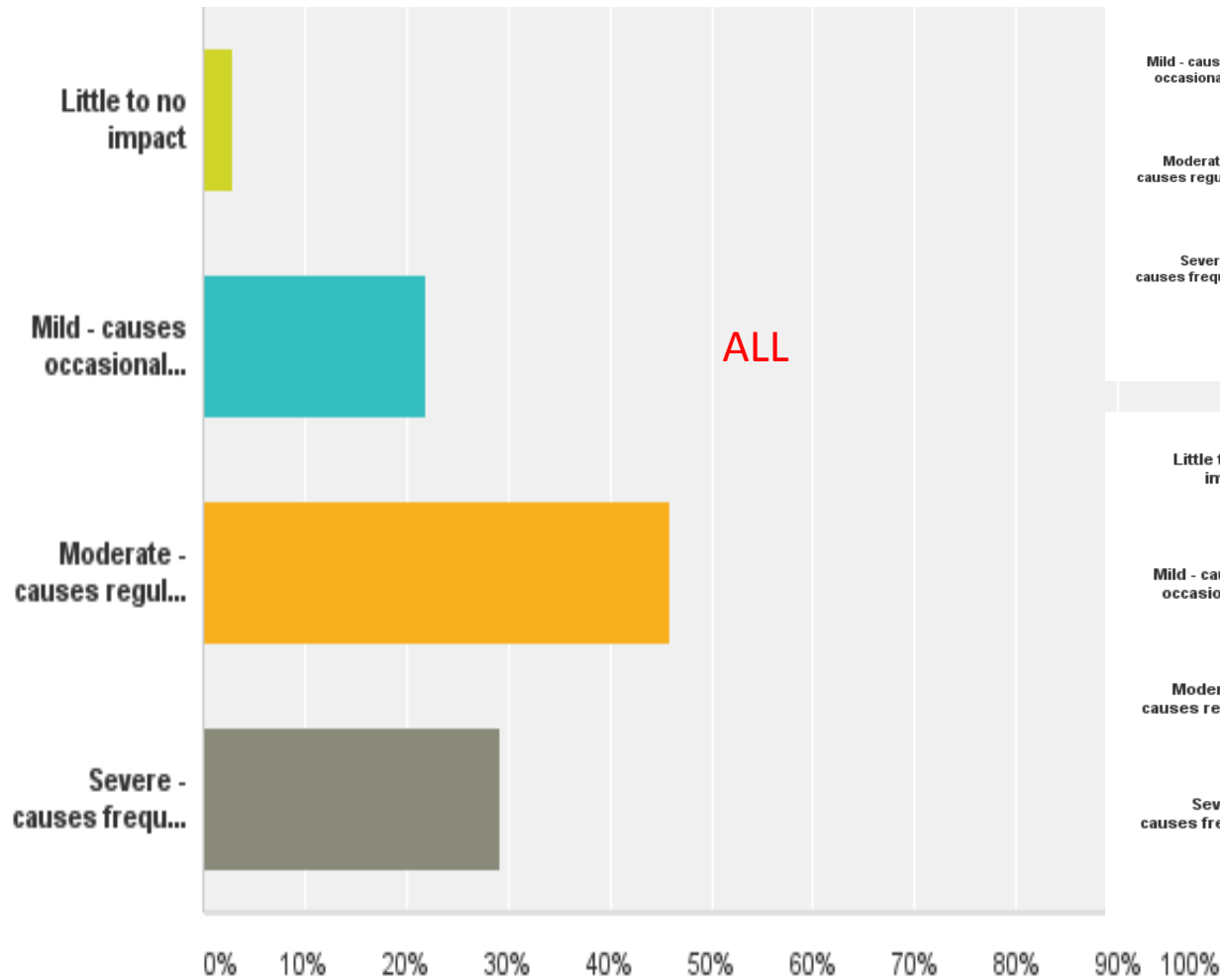
Q11: How would you rate the impact of PWS on the ability of the person with PWS to reach long term goals (education, job opportunities, independent living, healthy family relationships) ? 12 & up



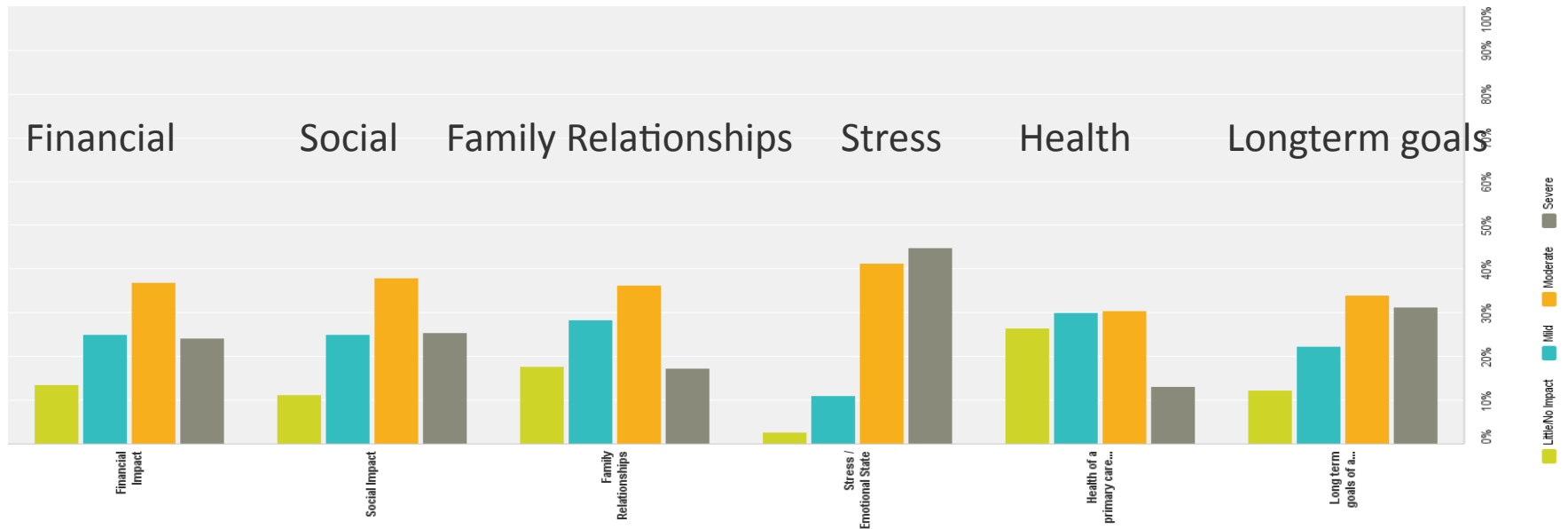
Q12: Overall how would you rate the impact of PWS on the primary caregiver of the person with PWS so far?



Q13: Overall how would you rate the impact of PWS on the family of the person with PWS so far?



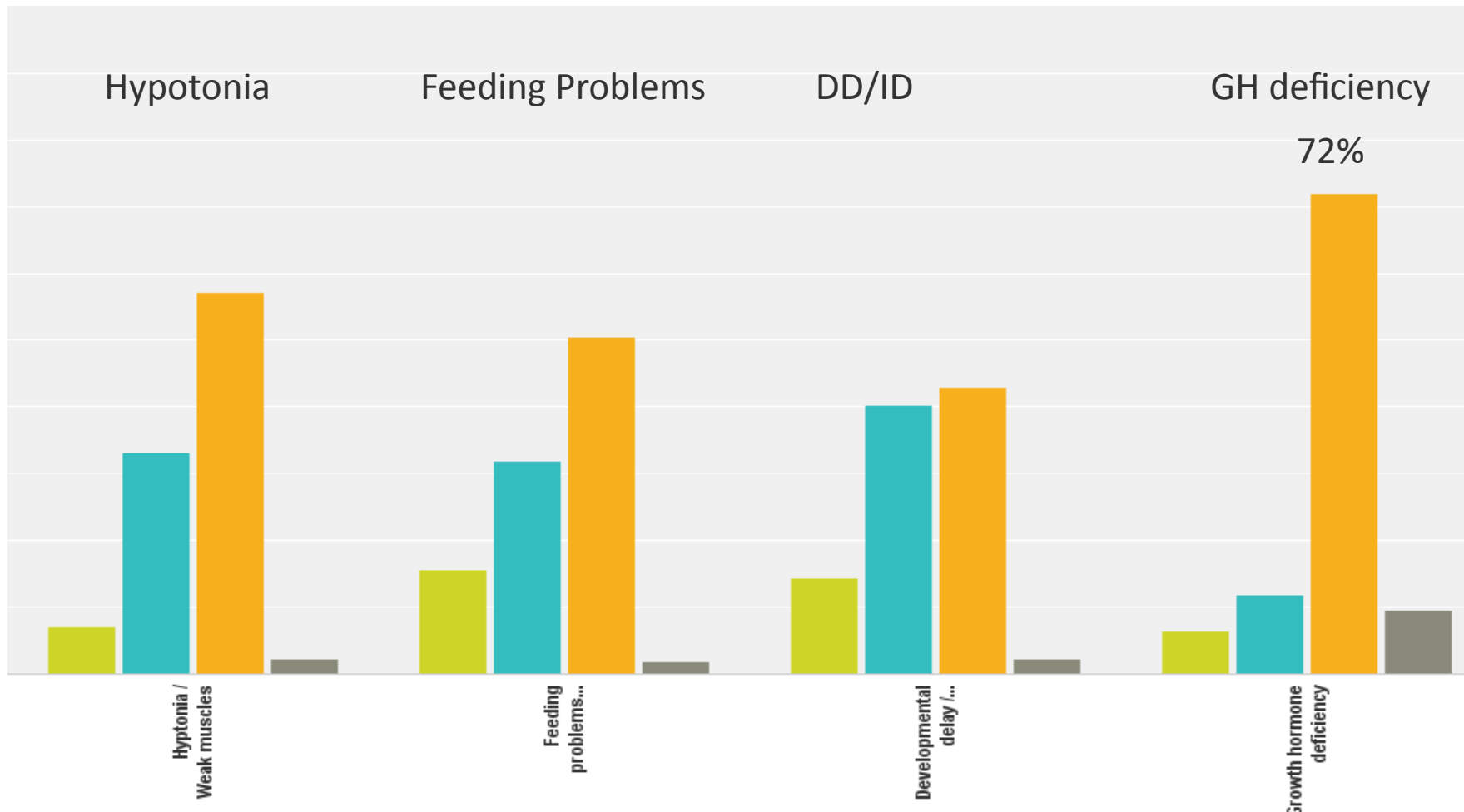
Q14: To what degree has PWS impacted the following aspects of the primary care giver's life? ALL



**Treatments – how do existing treatments work, what would
make a difference in the life of your loved one with PWS?**

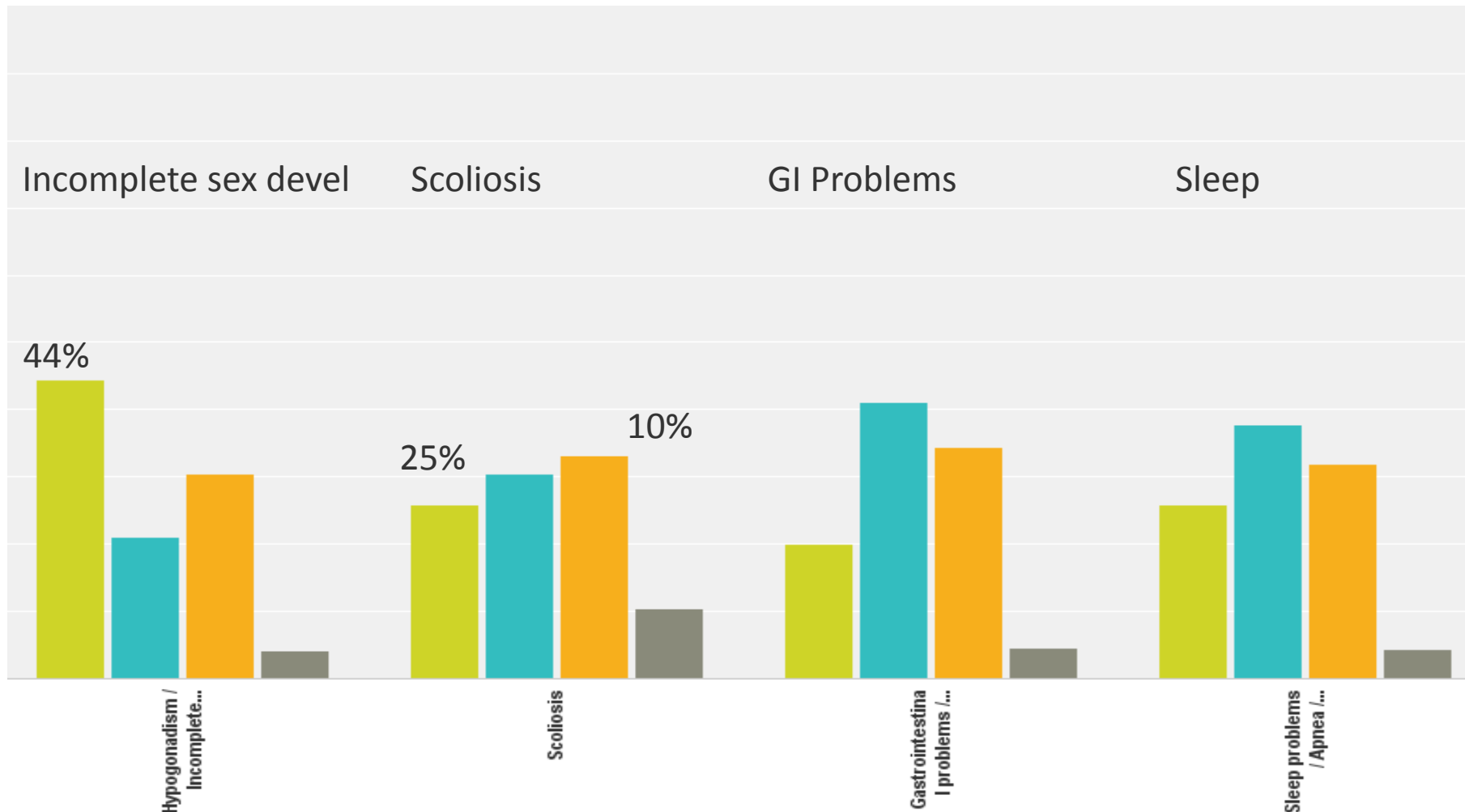
“unmet medical needs”

Q15: In thinking about all the treatments that you have used for the person with PWS (including physical and occupation therapy, supplements and drugs), how well do you feel these therapies are able to control the symptoms of PWS? Leave blank if not applicable.



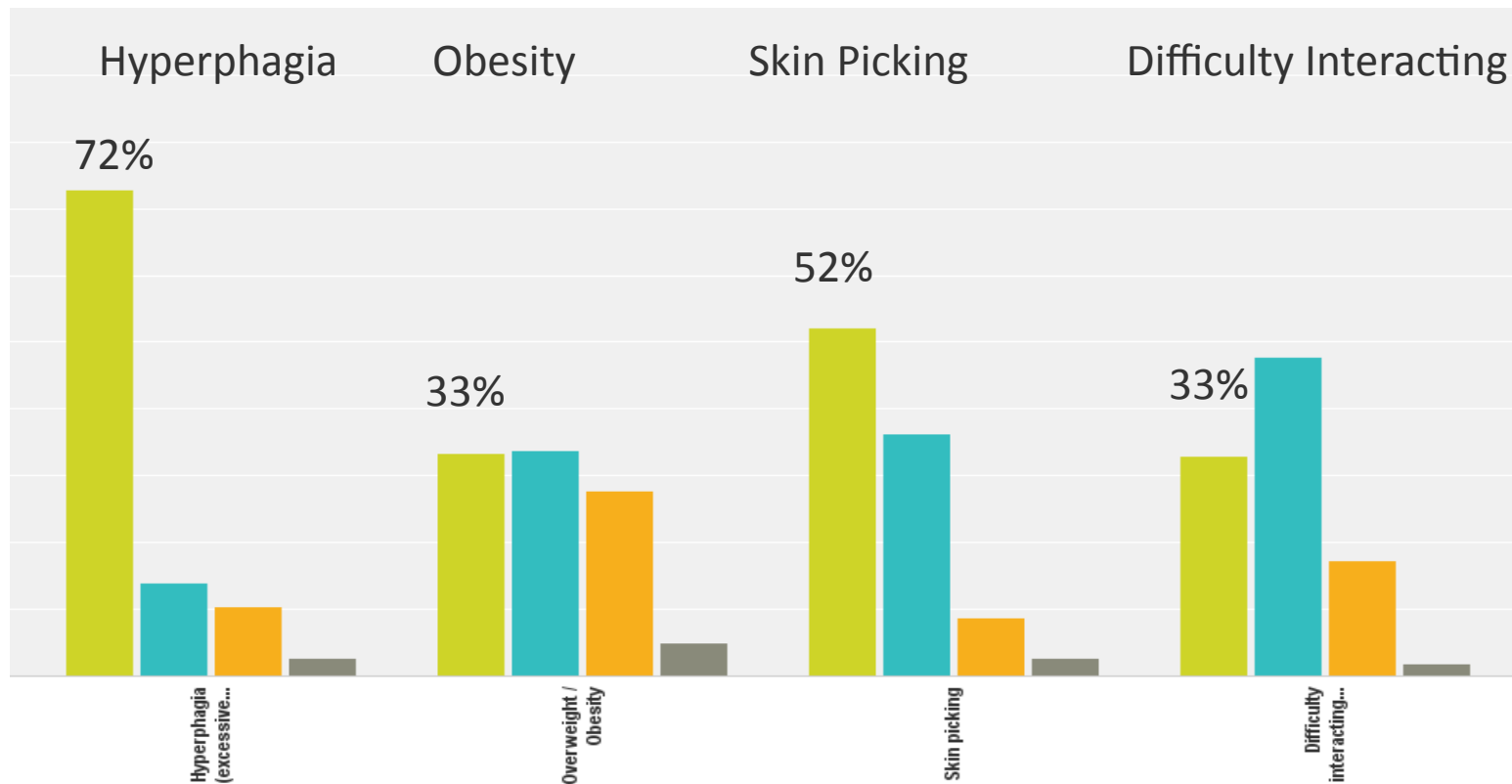
No effective therapy; Treatment helps somewhat; Treatment is very helpful; Treatment is helpful but side effects are significant

Q15: In thinking about all the treatments that you have used for the person with PWS (including physical and occupation therapy, supplements and drugs), how well do you feel these therapies are able to control the symptoms of PWS? Leave blank if not applicable.



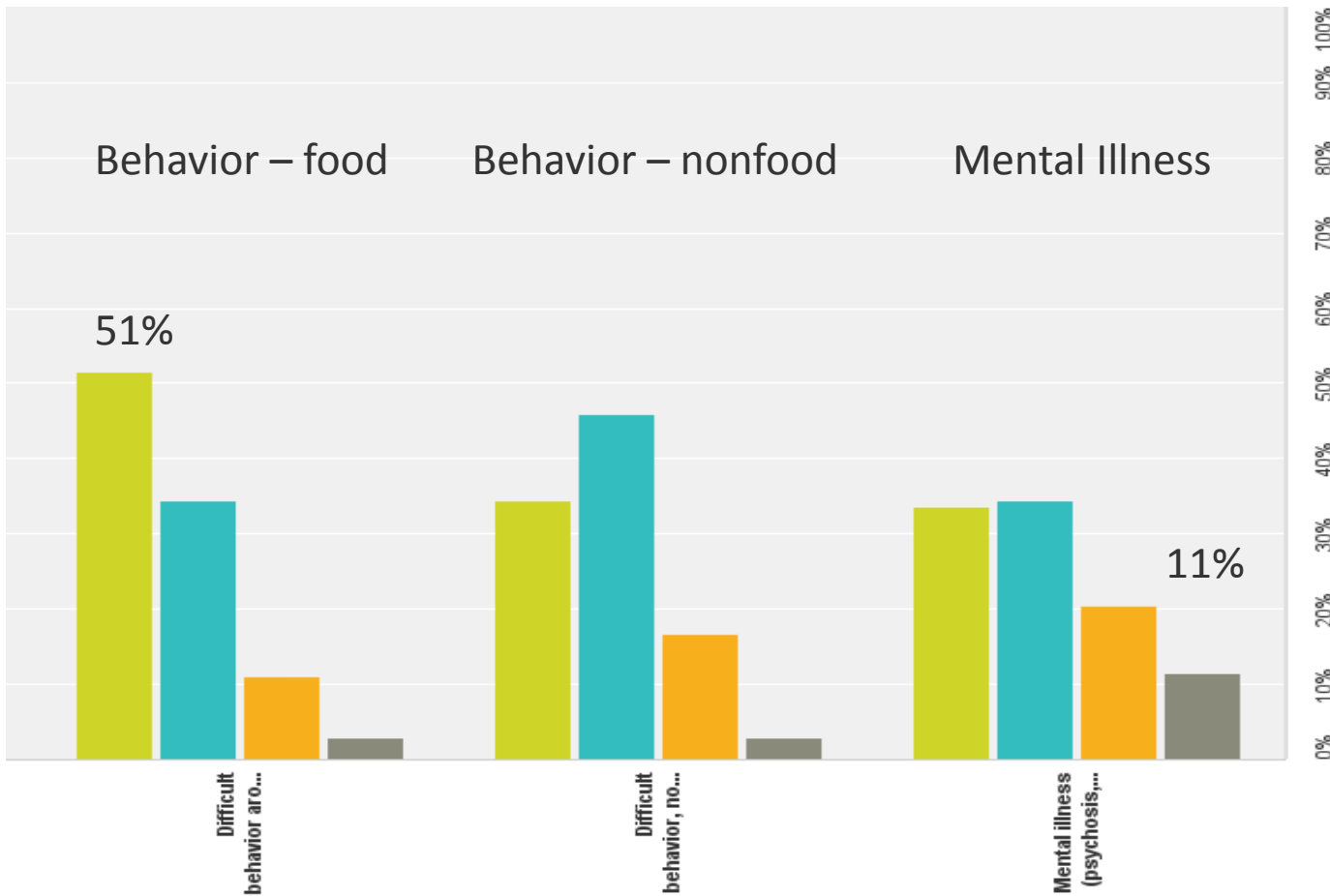
No effective therapy Treatment helps somewhat Treatment is very helpful
 Treatment is helpful but side effects are significant

Q15: In thinking about all the treatments that you have used for the person with PWS (including physical and occupation therapy, supplements and drugs), how well do you feel these therapies are able to control the symptoms of PWS? Leave blank if not applicable.



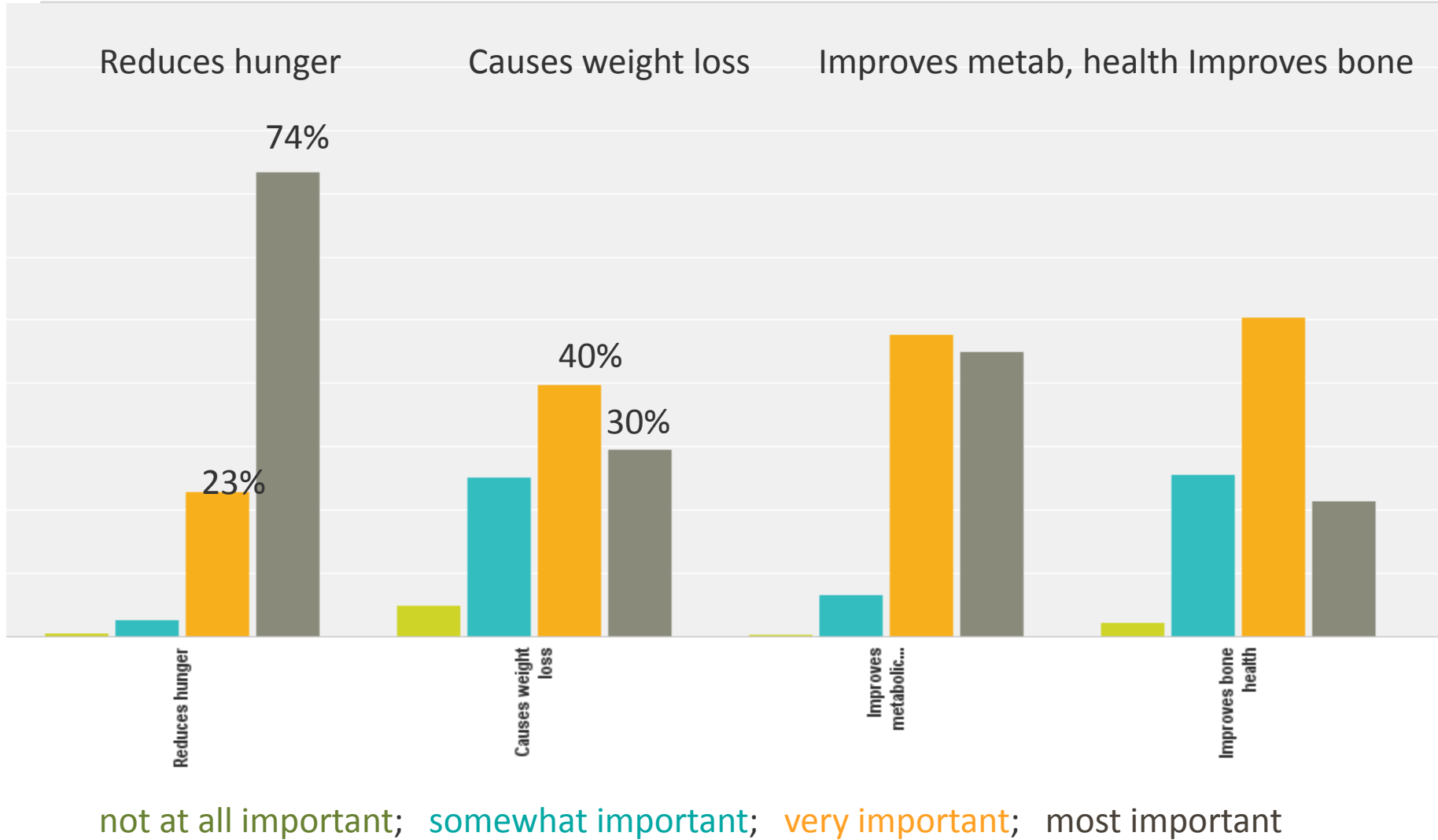
No effective therapy Treatment helps somewhat Treatment is very helpful
 Treatment is helpful but side effects are significant

Q15: In thinking about all the treatments that you have used for the person with PWS (including physical and occupation therapy, supplements and drugs), how well do you feel these therapies are able to control the symptoms of PWS? Leave blank if not applicable.

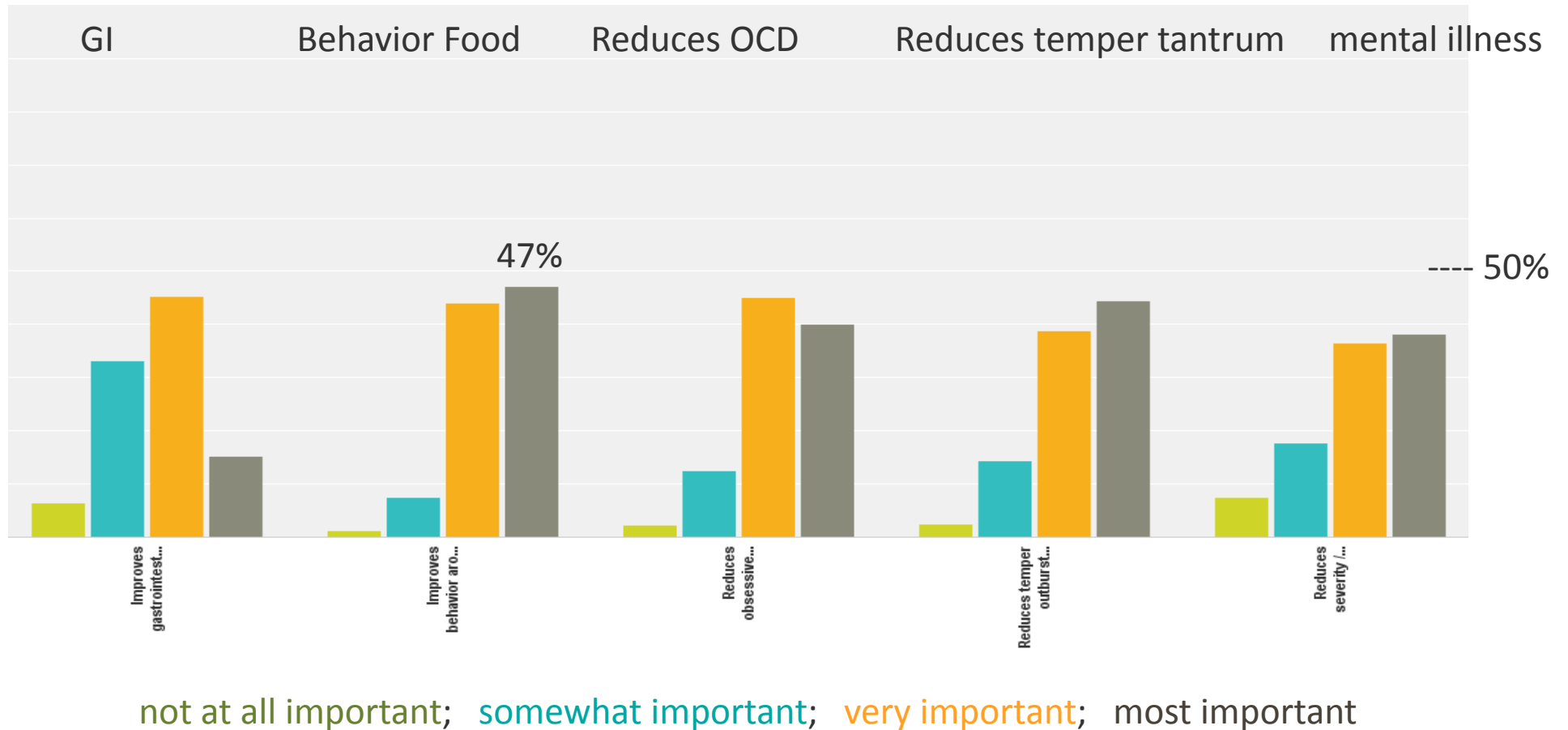


No effective therapy
 Treatment helps somewhat
 Treatment is very helpful
Treatment is helpful but side effects are significant

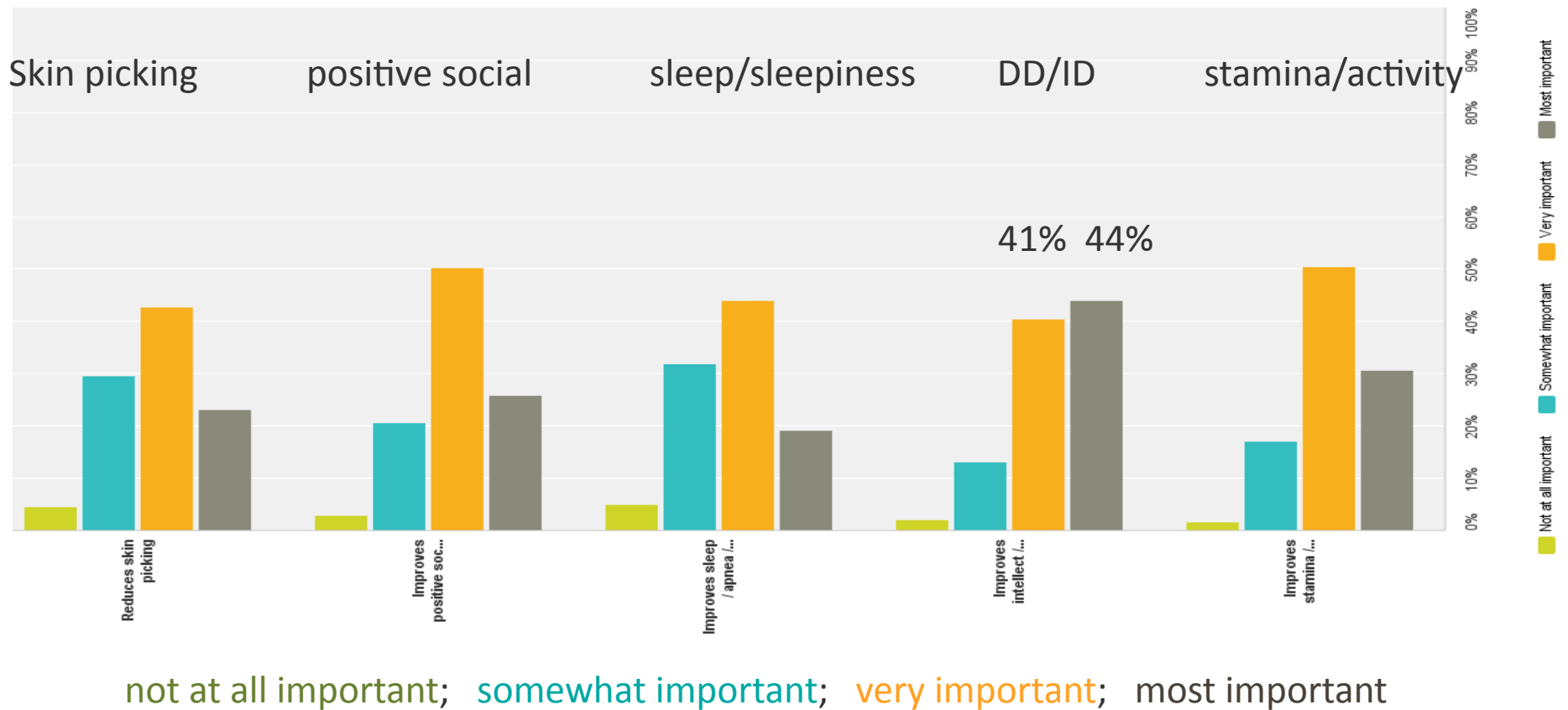
Q16: Assuming there is no complete cure for PWS, what specific things would you look for in an ideal treatment for PWS?



Q16: Assuming there is no complete cure for PWS, what specific things would you look for in an ideal treatment for PWS?

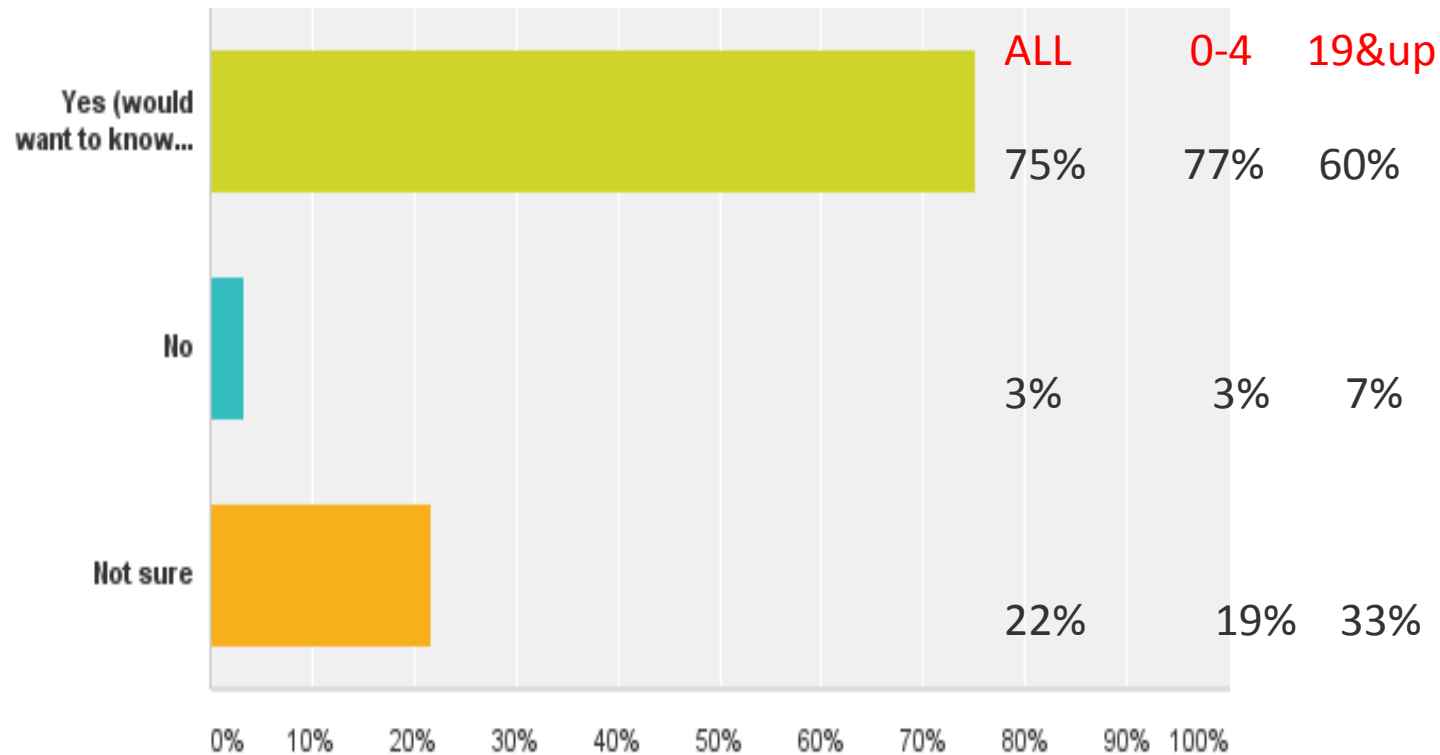


Q16: Assuming there is no complete cure for PWS, what specific things would you look for in an ideal treatment for PWS?



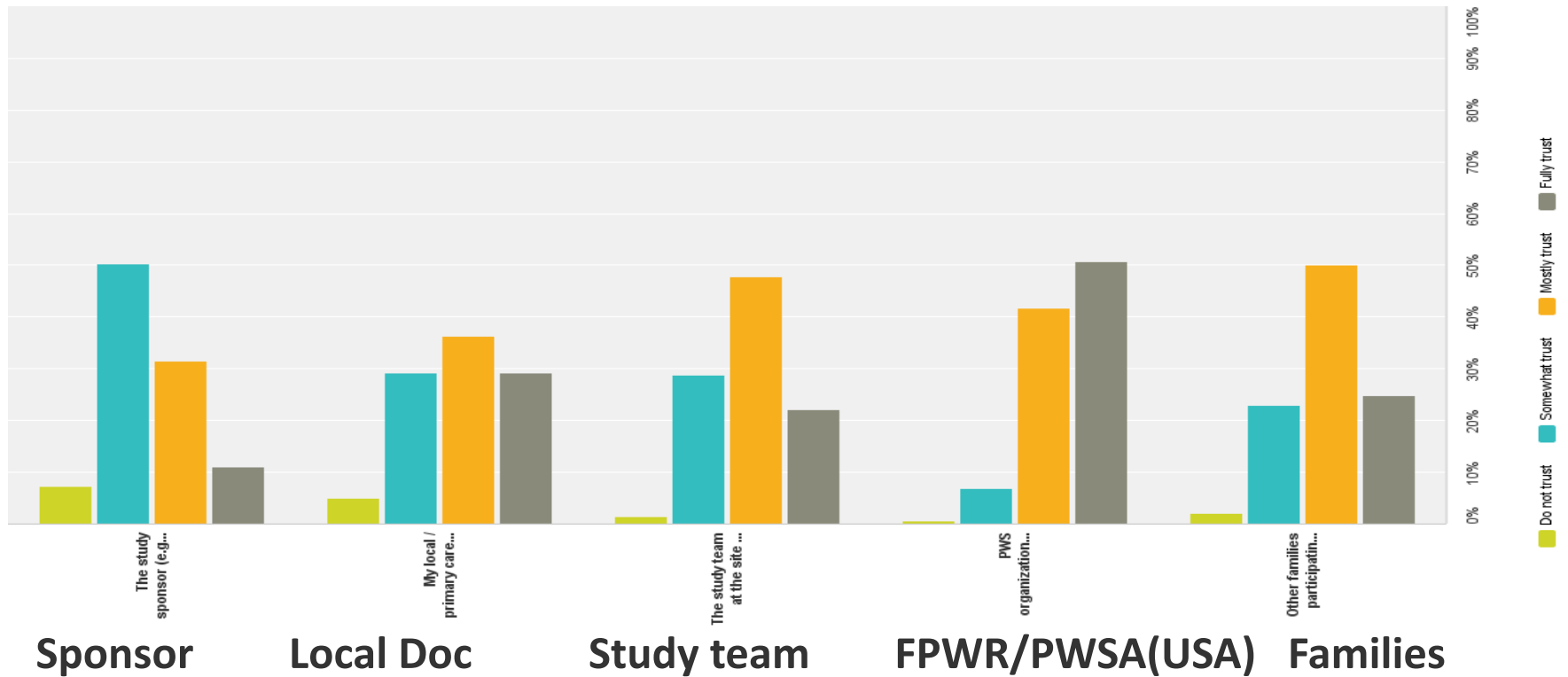
Attitudes towards clinical trials: hopes and concerns

Q17: Would you be willing to have your loved one with PWS participate in a clinical trial of an experimental / investigational treatment?



Q18: What is your level of trust when considering who would provide accurate information about a PWS clinical trial?

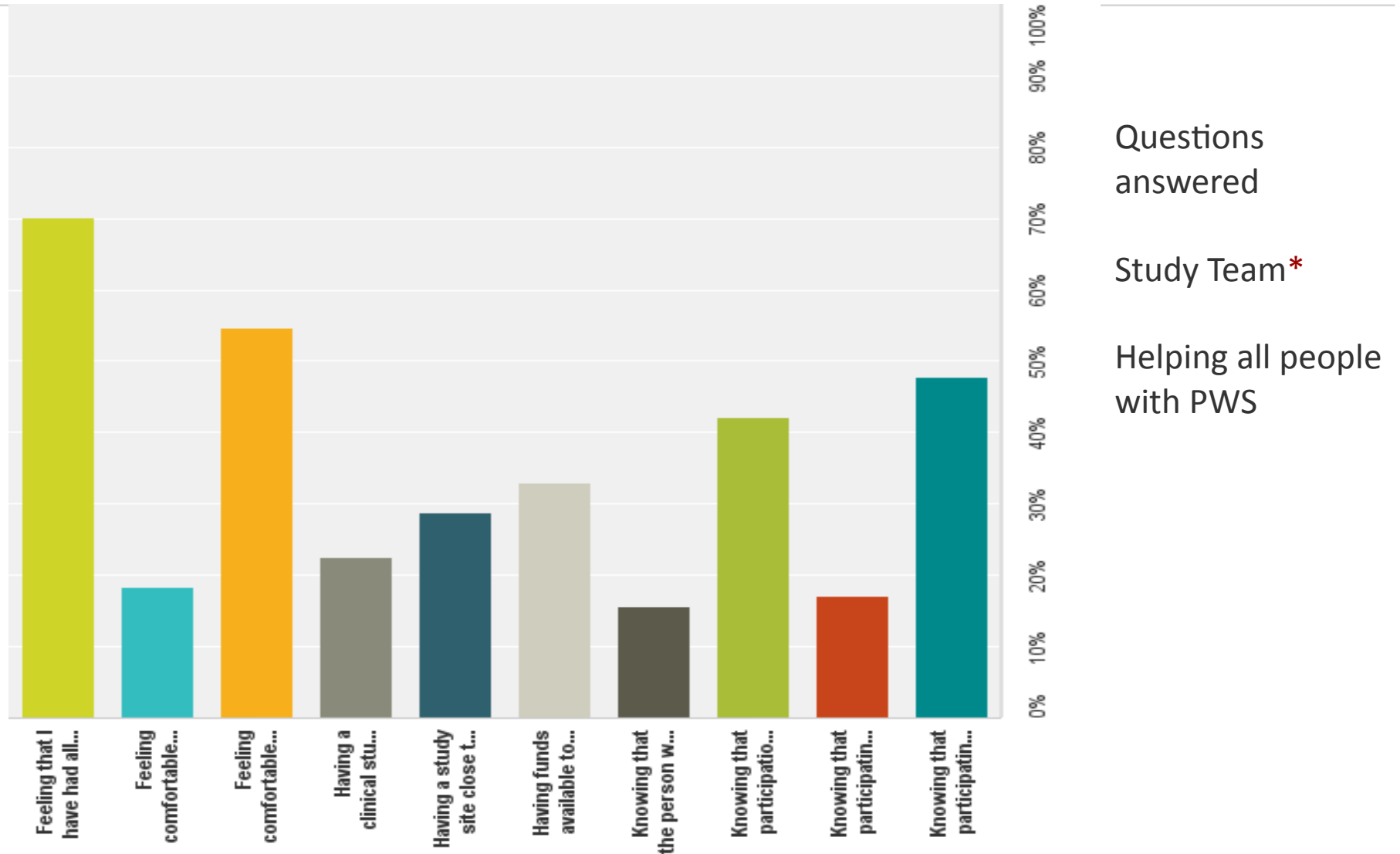
Do not trust Somewhat trust Mostly trust Fully trust



Q19: What factors would be most important in your decision about whether to enroll the person with PWS in a study or not? (Choose up to 3).

- Feeling that I have had all of my questions answered about the potential benefits and risks of the drug
- Feeling comfortable with the study sponsor (Pharmaceutical company or University)
- Feeling comfortable with the doctor and research team who will perform the study
- Having a clinical study schedule that works with my schedule and the schedule of the person with PWS
- Having a study site close to my home
- Having funds available to cover my travel expenses
- Knowing that the person with PWS is interested in participating
- Knowing that participation in a clinical trial may lead to a new treatment for the person with PWS
- Knowing that participating in a clinical trial might be the only way the person with PWS can try the drug and find out if it works for them
- Knowing that participating in a clinical trial may help all people with PWS have access to a potentially effective new therapy

Q19: What factors would be most important in your decision about whether to enroll the person with PWS in a study or not? (Choose up to 3).



Questions answered

Study Team*

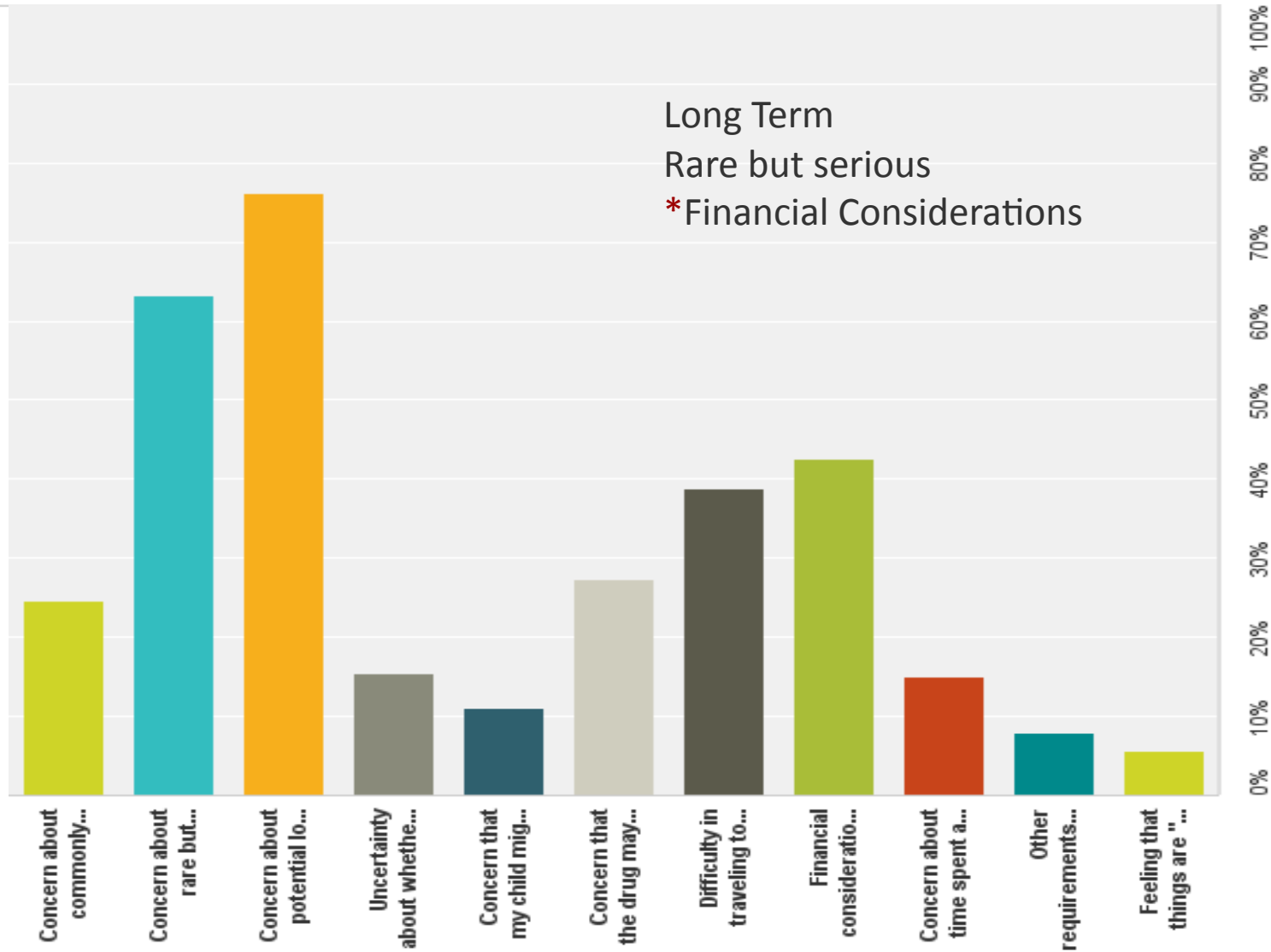
Helping all people with PWS

*Study team has all the information about the drug, know risks, possible benefits

Q20: What factors are most likely to keep you from enrolling with PWS in a clinical study? (Choose up to 3).

- Concern about commonly occurring, mild side effects, such as nausea, headache, etc
- Concern about rare but serious side effects
- Concern about a potential long term side effects
- Uncertainty about whether the treatment would improve health
- Concern that my child might not get the drug (placebo) or get enough of the drug
- Concern that the drug may work, but we will not be able to continue using it at the end of the study
- Difficulty in traveling to the study site multiple times – logistics
- Financial considerations – time off work and travel costs
- Concern about time spent away from work or other family members
- Other requirements of the trial (blood draws, surveys, lab tests)
- Feeling that things are “OK” as they are and there’s no need to try something new

Q20: What factors are most likely to keep you from enrolling with PWS in a clinical study? (Choose up to 3).



*Many studies pay travel and study related expenses

Open ended questions

Please provide any additional comments you may have on the impact of PWS on the person with PWS or the family.

- Constant stress
- Stress on family and person with PWS is overwhelming.
- There is a feeling of isolation with PWS, no one understands and few are willing to make the sacrifices necessary to keep the PWS patient safe (family/friends)
- Although having a child with PWS has changed our lives....it isn't all bad. It has given us a lot of experiences and qualities that we would not have had otherwise.
- Hyperphagia and behavior problems are the biggest barriers to my daughter's ability to choose a path in life for herself.
- His behaviors impact our whole family's mood and schedule.

Open ended questions

Please provide any additional comments you may have on whether current treatments are adequately addressing the symptoms of PWS.

- THEY ARE NOT. DUH.
- We have tried many treatments with no success. It is very disappointing for my child and frustrating to me. It is heartbreaking to see your child breakdown thinking that nothing will help this insatiable hunger. It brings her much shame.
- Nothing has been of any real help with the hyperphagia, obesity, and activity level.
- I think at each age and stage my son has gone through the challenges have been significant yet different. Low tone, mobility issues and feeding problems were hugely significant in his first 5 years, now that he is a teen we struggle with behaviour problems, skin picking and constant obsessing about food. These problems were almost non-existent for his first 10 years. So current treatments can never have a one-size-fits-all approach.

Open ended questions

Please provide any additional comments you may have about PWS clinical trials.

- Haven't had much experience with them. But very willing.
- More trials are needed in all aspects of PWS.
- I contacted a trial, asked to get info and was never sent anything. If there is a need for participants the researchers should follow through with willing people.
- I would be concerned about the impact on the PWS patient knowing they are in a trial....too much to obsess about.
- I do not think placebos should be given, those who do not participate provide the baseline.

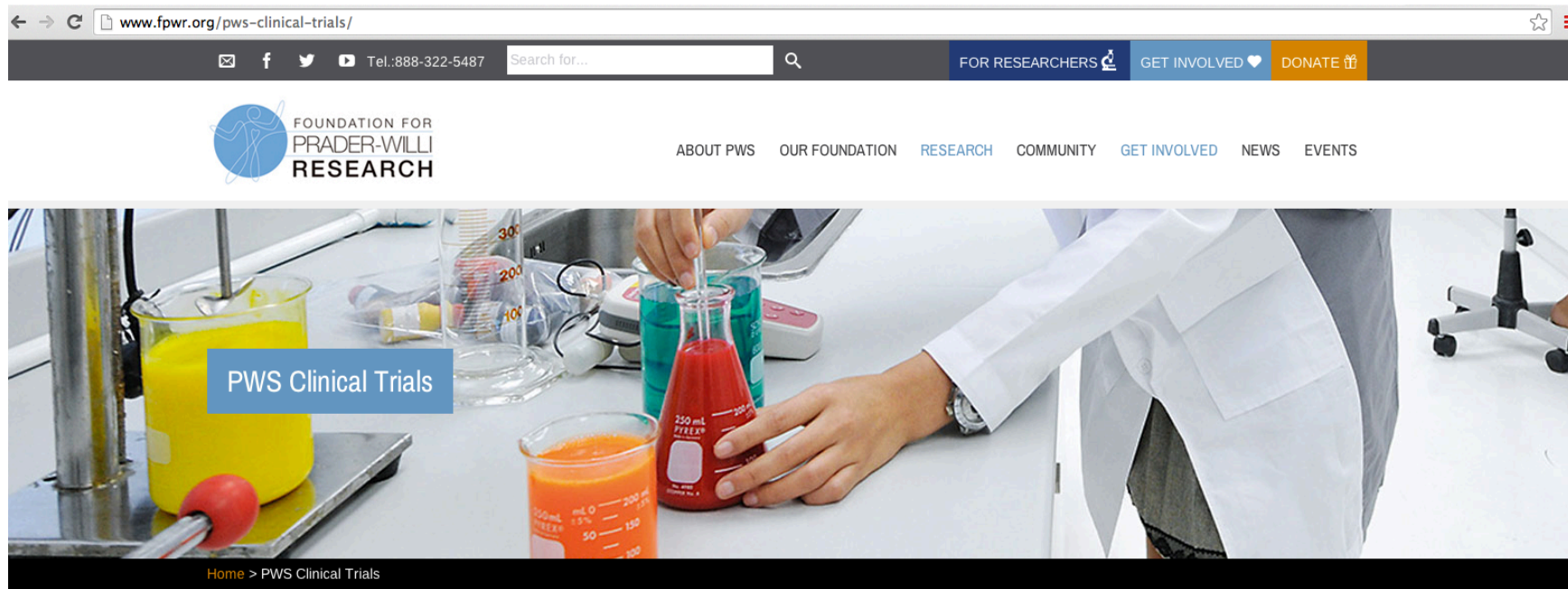


Future Steps

Develop Benefit Risk Assessment – patient perspective

Engage Stakeholders and continue discussions to ensure that treatments safely addressing unmet medical needs in the PWS population are advanced efficiently

Stay informed: <http://www.fpwr.org/pws-clinical-trials/>



Clinical studies aimed at better understanding Prader-Willi syndrome and investigating new treatments for PWS are taking place around the world. There are a number of ways you can stay informed:

- [Sign up](#) for our Clinical Trial Alerts Bulletin. We will announce new clinical trials as they open.
- Participate in the [Global PWS Registry](#). The registry will give you the option of allowing researchers to contact you about trial opportunities.
- Become a fan of the [FPWR Facebook page](#) and watch for our announcements!
- The clinicaltrials.gov website is a comprehensive list of clinical studies taking place around the world. Search for PWS trials using keywords "Prader-Willi" or "PWS".
- Learn more about clinical trials from our [webinar](#) and [FAQs](#).
- Check this page often for updates and additions to our list of [current clinical trials](#)!



PWS Clinical Trials Alert

Thank you!

Questions??