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Characterization of Dietary Protein Intake in PKU Patients

Mesaki K. Ndugga-Kabuye[1], Sharon Ernst[1], Nicole McWhorter[1], Casey Woodbury[1], Kristina Humphreys[1], Aoife Brennan[1], Caroline Kurtz[1] and Marja Puurunen[1]
[1]Synlogic Inc

Introduction

- Standard treatment for patients with PKU is a protein-restricted diet, supplemented with medical formula. Most patients with classic PKU can only tolerate < 500 mg Phe per day (10 g natural protein) to maintain adequate Phe control. Compliance with a life-long Phe-restricted diet is challenging and dietary adherence decreases with age. Phe levels above the recommended range can lead to neurocognitive and behavioral challenges.
- The patients' perspective on a desired change in natural protein intake can help define clinically meaningful endpoints for clinical studies.

Methods

- We conducted an online survey of self-identified PKU patients and parents/caregivers together with the National PKU Alliance (NPKUA) and the Canadian PKU and Allied Disorders (CanPKU) with a focus on Phe levels, dietary management, and the patient and caregiver perspective on the need for additional natural protein in their diet.
- The survey consisted of 27 questions and was offered through the SurveyMonkey® platform. It was available in both English and French.

Results

Baseline Characteristics

- 64 PKU patient surveys (55% USA), and 77 parent/caregiver surveys (51% USA) were completed. Mean age of the adults with PKU was 36.0 years old (range 18.3 to 56.7), and 8.7 years old (range 0.2 to 17.4) for the children with PKU. Male patients accounted for 38.6% of the adults and 40.6% of the children.

PKU clinical status and current therapy

- 45.7% of adults and 50.7% of children were on Sapropterin. (Figure 1)

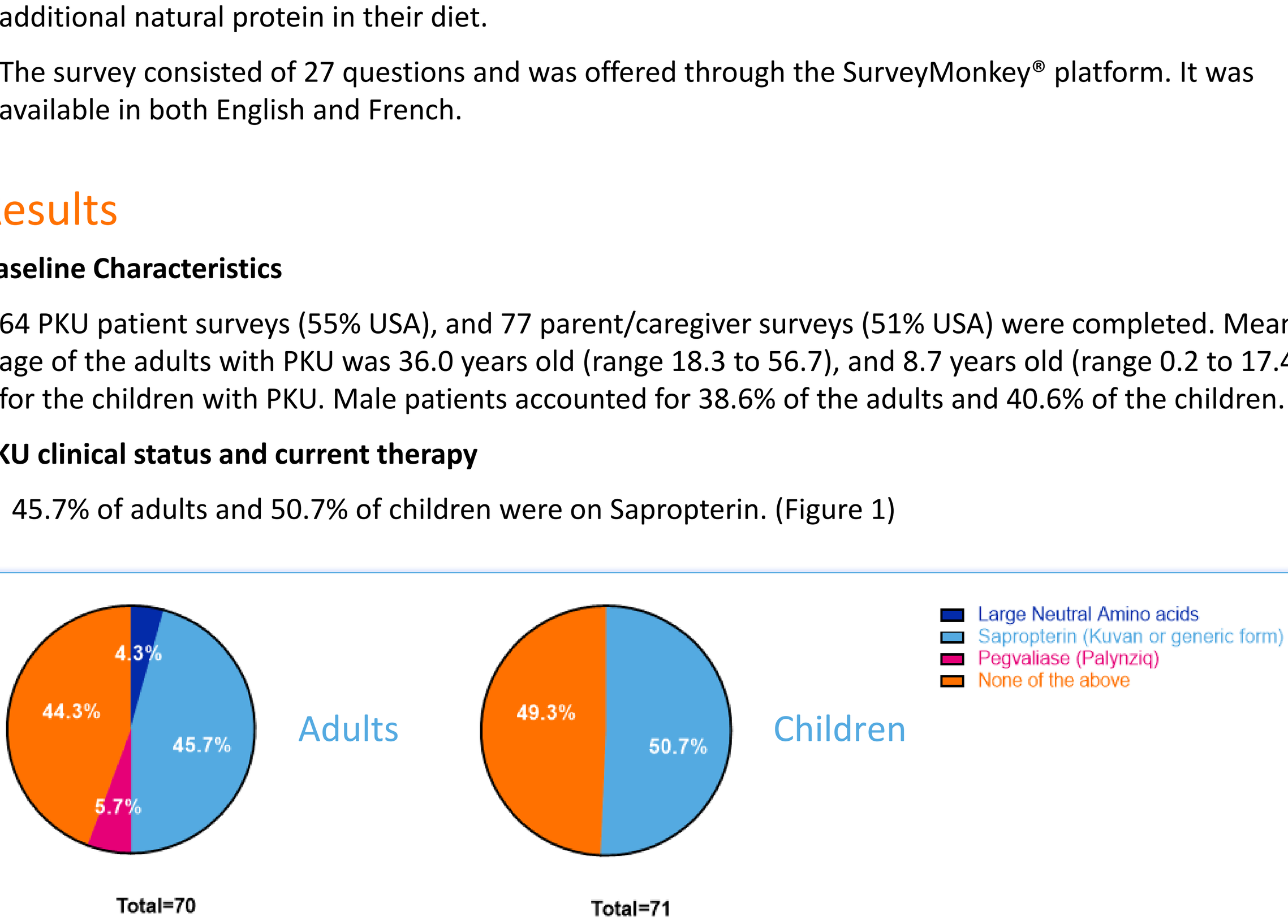

Figure 1. Current therapy, all participants.

PKU clinical status and current therapy

- Overall, target Phe of < 360 umol/L was not reached by 67.7% of all adults and 36.7% of all children at their most recent blood Phe assessment. Of those on Sapropterin, 48.3% of adults and 44.4% of children did not reach the target Phe of < 360 umol/L. (Figures 2 and 3).
- Mean daily natural protein intake was 27.1 g in adults (range 4.7-90) and 16.0 g in children (range 3-50). Mean daily Phe intake was 1353.8 mg in adults (range 235-4500) and 798.6 mg in children (range 150-2500). The mean intake was higher in those on sapropterin (Figure 4).

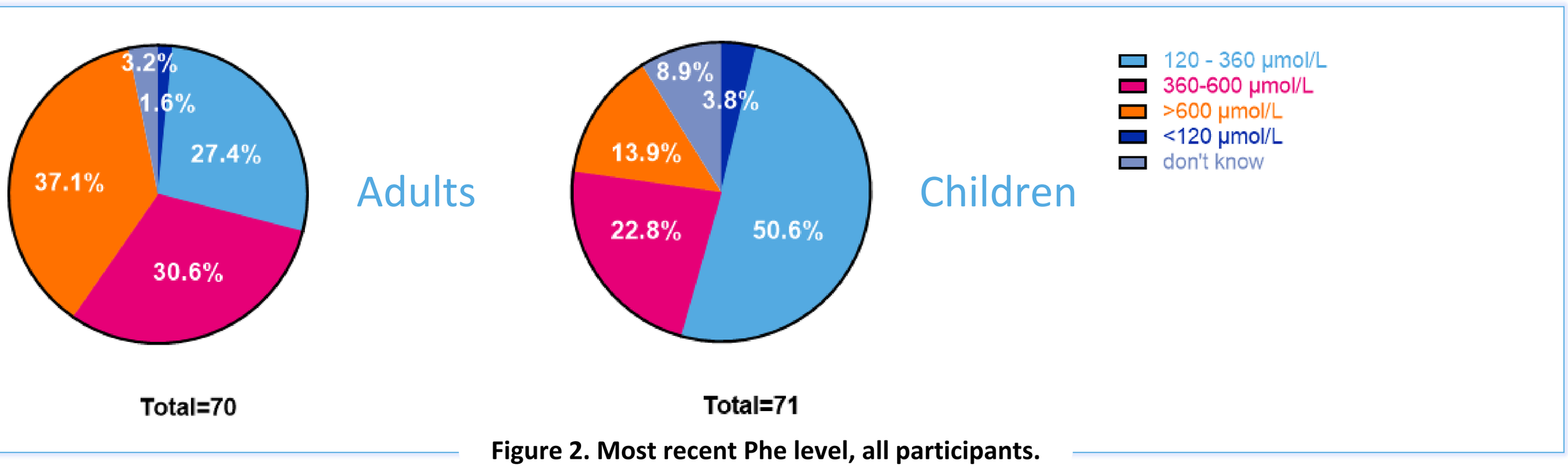

Figure 2. Most recent Phe level, all participants.

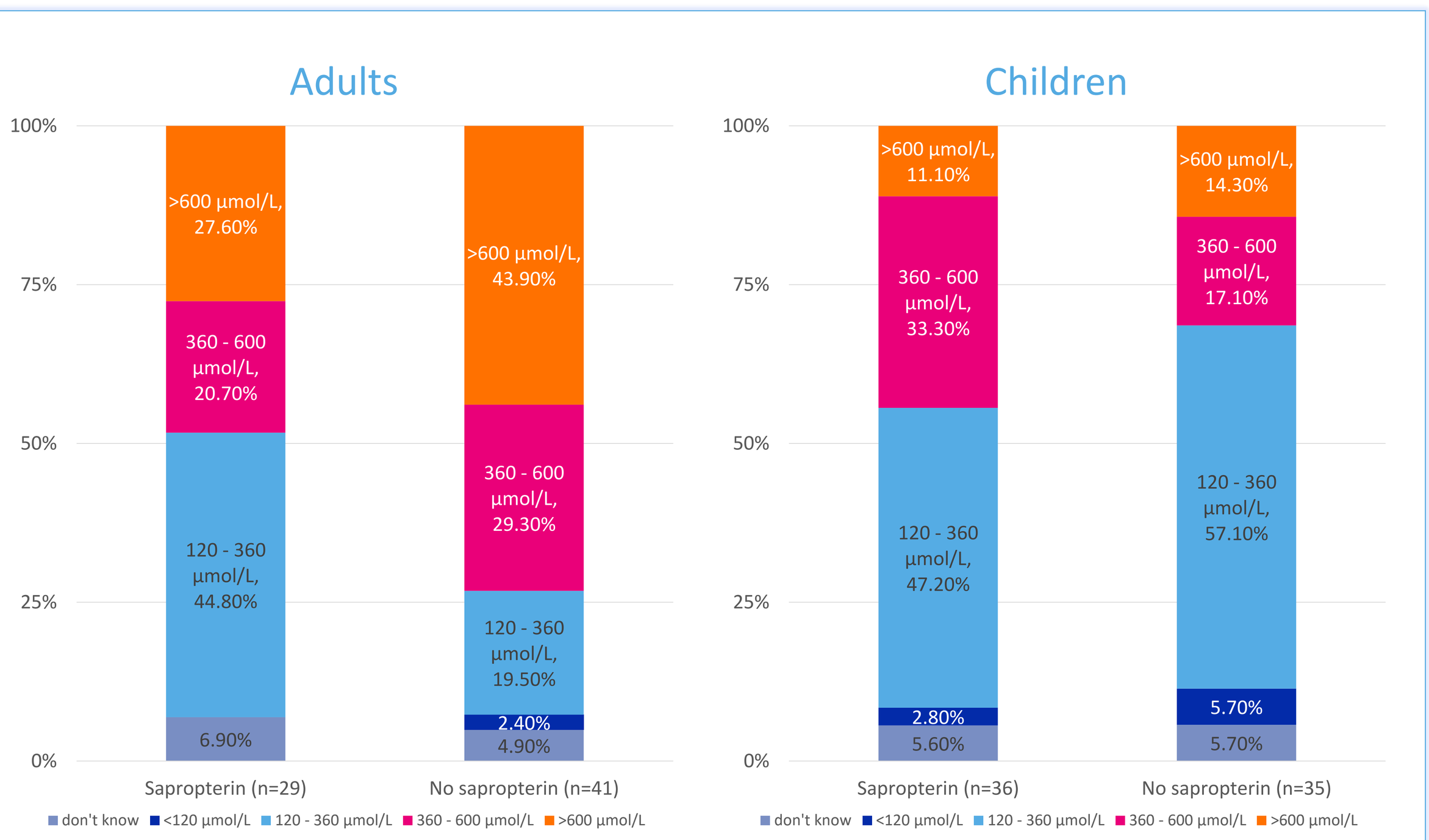

Figure 3. A significant proportion of adult and pediatric patients, including those on Sapropterin, have Phe levels above the recommended target level of < 360 umol/L. Most recent Phe level; Sapropterin vs No Sapropterin.

Adults

	Sapropterin		No Sapropterin	
	Phe (mg)	**protein (g)**	**Phe (mg)**	**protein (g)**
Mean (range)	860 (500 - 1500)	32.1 (6 – 90)	458.9 (150 – 1100)	22.5 (4.65 – 70)

Children

	Sapropterin		No Sapropterin	
	Phe (mg)	**protein (g)**	**Phe (mg)**	**protein (g)**
Mean (range)	524.4 (100 - 1200)	22.0 (6 – 50)	291.4 (100 – 575)	6.9 (3 – 13.5)

Figure 4. Question: On average, how much phe (in mg) or natural protein (in g) do you usually consume in a day? Please enter only one unit of measure in your answer, phe or protein.

Unmet need for additional natural protein

- Almost all patients in both groups reported that they would like to increase the amount of natural protein in their diet. (Figure 5)
- 2-3 g of additional natural protein would be considered meaningful to 54.4% of all adult patients, and 80.9% of all pediatric patients. 59.9% of all patients on Sapropterin would consider 2-3 g of additional natural protein meaningful (e.g., 1 slice white bread, 1/2 cup broccoli, or 1 medium potato). (Figure 6)

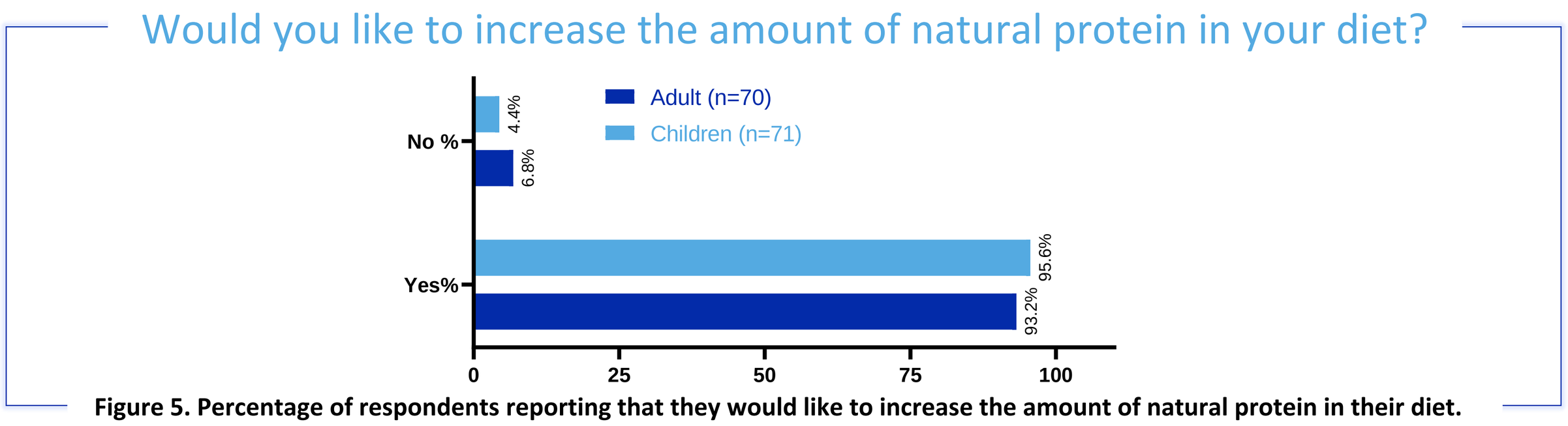

Figure 5. Percentage of respondents reporting that they would like to increase the amount of natural protein in their diet.

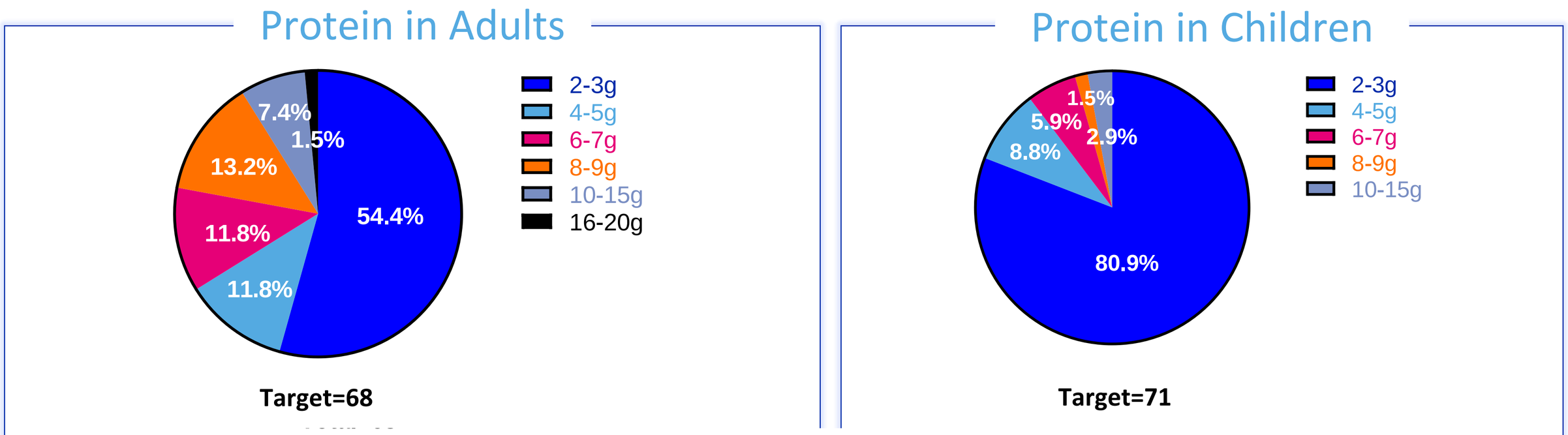

Figure 6. What amount of natural protein increase per day, would be considered meaningful.

Conclusions

- Even with the use of currently available therapies, the unmet need for PKU patients remains high.
- A significant proportion of both adult and pediatric PKU patients do not reach recommended Phe level (<360 µmol/L) on currently available therapies.
- The PKU diet is difficult to adhere to and virtually all PKU patients would like to be able to add more natural protein to their diet
    - Even 2-3 g of additional natural protein per day would be considered meaningful by the majority of PKU patients (or their caregivers).
    - Examples of foods with 2-3g of protein include 1 slice white bread, 1/2 cup broccoli, 1 medium potato

Limitations

- Survey participants were self-reported PKU patients or parents/caregivers of PKU patients. For purposes of analysis, respondents that did not provide the age of the PKU patient were assigned to the adult category if the PKU patient survey was completed, or the pediatric category, if the parent/caregiver survey had been completed.

References

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