

synlogic



Designed for Life

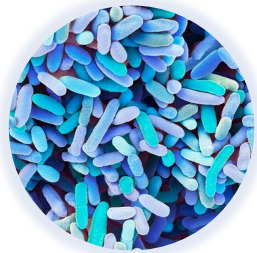
Synthetic Biotic Medicines for the Treatment of PKU

Marja Puurunen, MD, PhD, Head of Metabolic Programs

Global PKU Patient Conference
September 2021

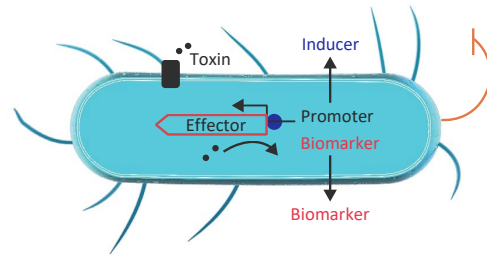
A new class of medicines

Synthetic Biotic platform



Non-pathogenic bacterial chassis

+



Programmable, engineered

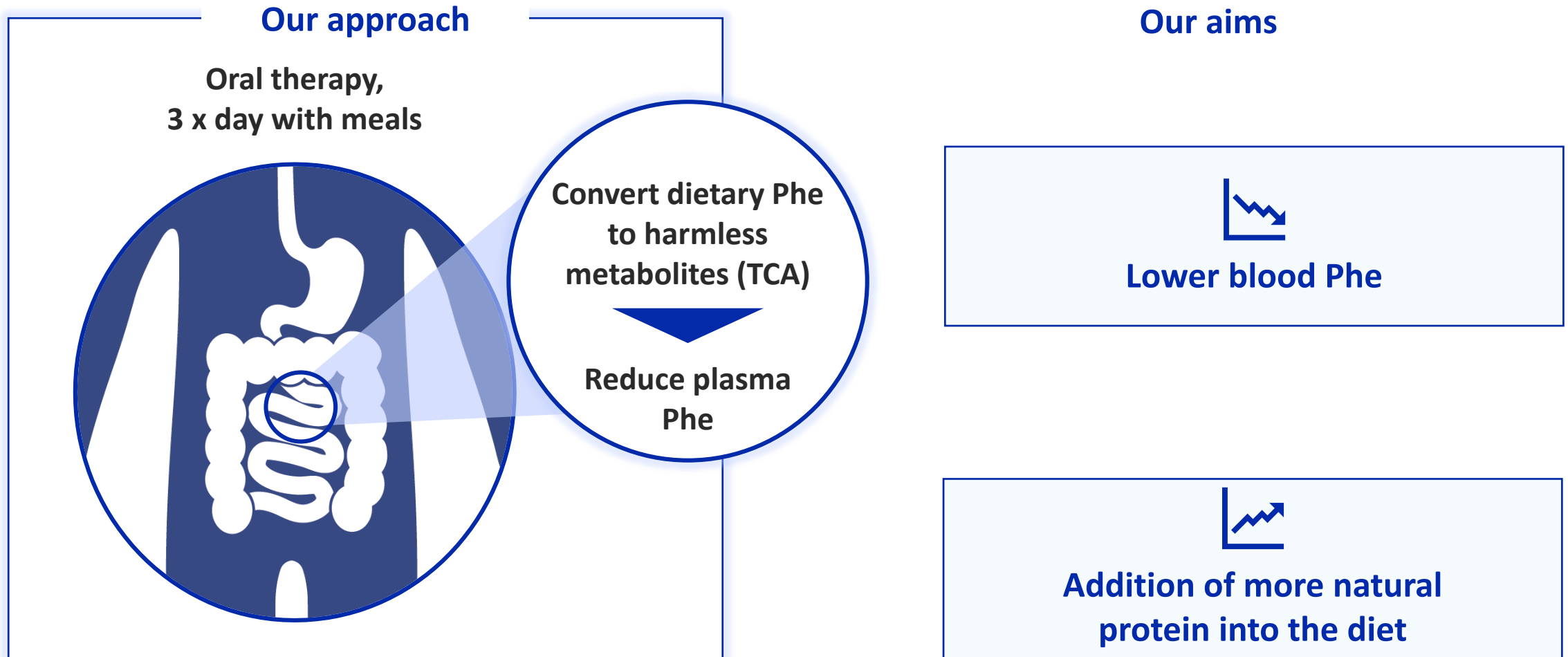


Potential Treatment for PKU

- Consumes toxic metabolites from the GI tract
- Potential treatment for **PKU** and other allied disorders

Our goal is to develop targeted therapies Synthetic Biotic medicines for the treatment of rare and metabolic disease

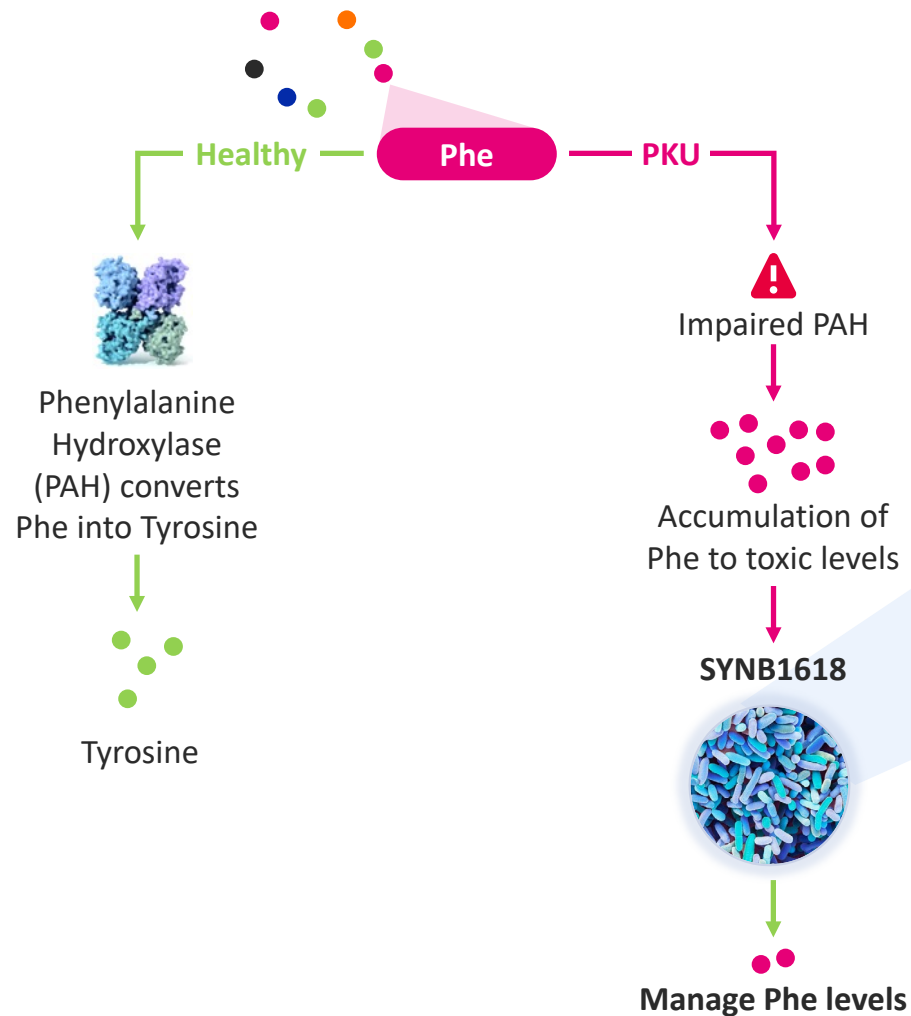
A potential new approach to managing phe



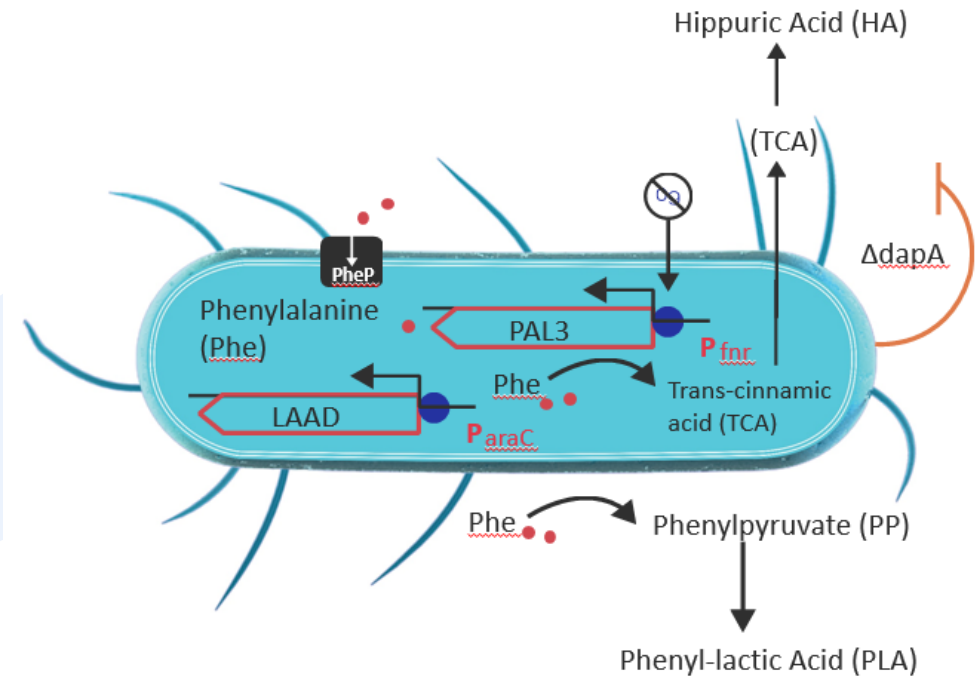
Synlogic has initiated a Ph2 Study in PKU patients (SynPheny)

How do Synthetic Biotic medicines for PKU work?

Amino acids from dietary proteins
(absorption and recirculation)

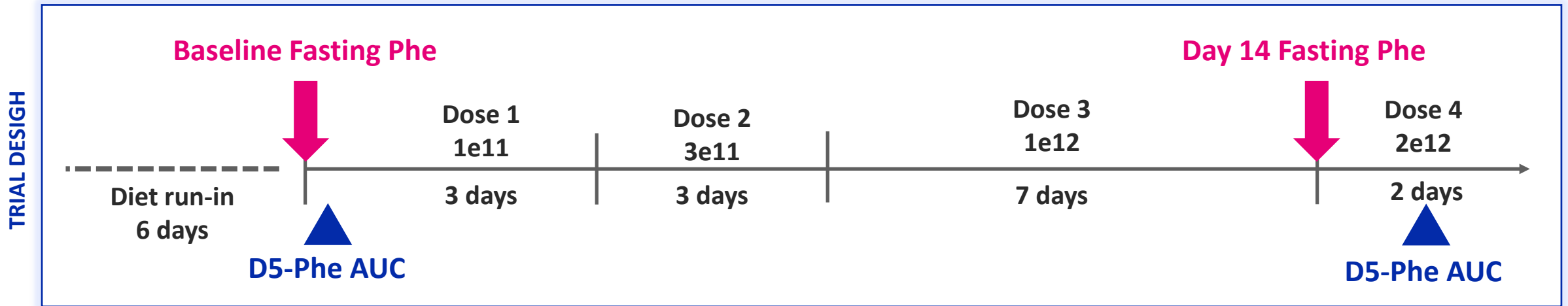


Engineered Probiotic Bacteria: *E. coli* Nissle
Components of Synthetic Genetic Circuit



SynPheny-1: Phase 2 Proof of Concept Study Ongoing

Currently Recruiting in the United States



ClinicalTrials.gov NCT04534842

Study website <https://pkuresearchstudy.com/>

PKU Patient Survey



Patient Survey: Understanding the Needs of PKU

Goal

Understanding the daily challenges PKU patients face with a focus on Phe levels, dietary management, and need for additional natural protein in their diet

Method

Online survey of PKU patients and parents/caregivers together with NPKUA and CanPKU

Study Participants

Participants

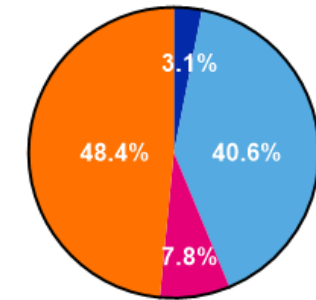
- 64 PKU patients (55% US)
- 77 parents/caregivers (51% US)

- 40.6% of adults and 53.2% of children on Kuvan

- Mean daily natural protein intake was
 - 25.3 g in adults (range 5-78)
 - 19.1 g in children (range 3-90)

- Mean daily Phe intake was
 - 1267 mg in adults (range 250-3900)
 - 953 mg in children (range 150-4500)

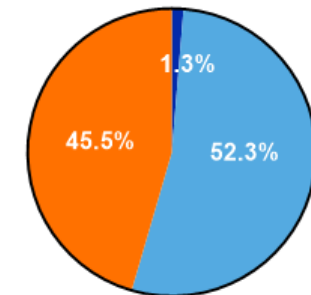
Adults



Total=64

- Large Neutral Amino acids
- Sapropterin (Kuvan or generic form)
- Pegvaliase (Palynziq)
- None of the above

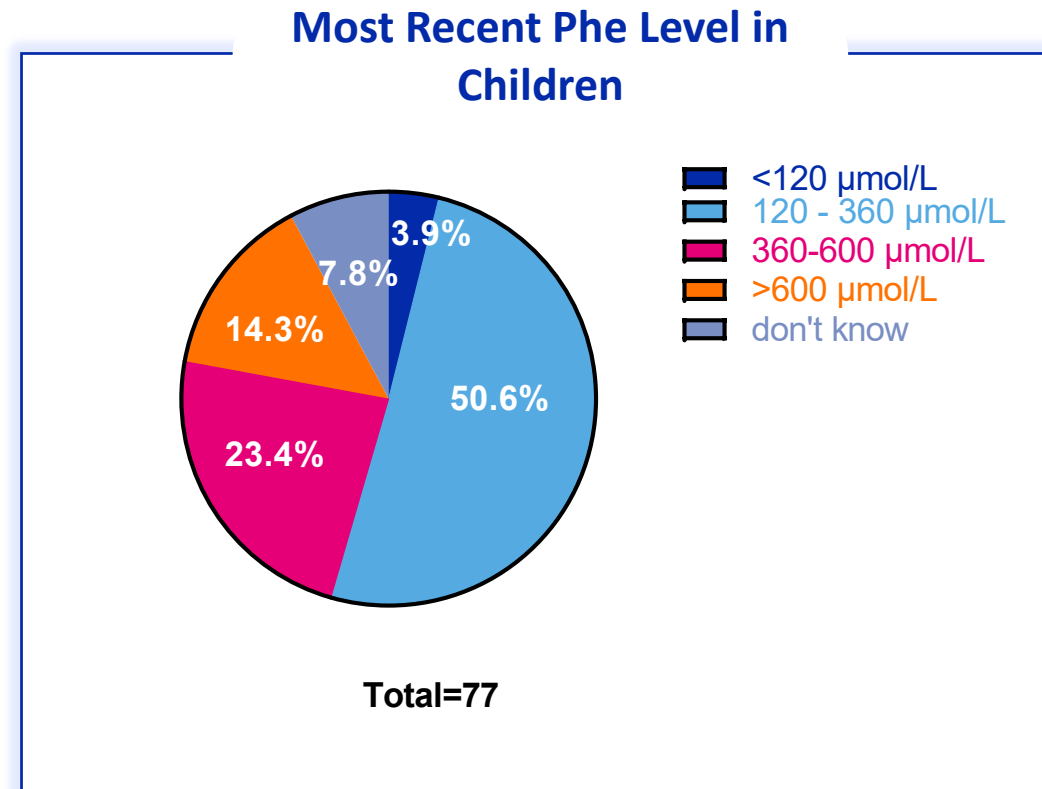
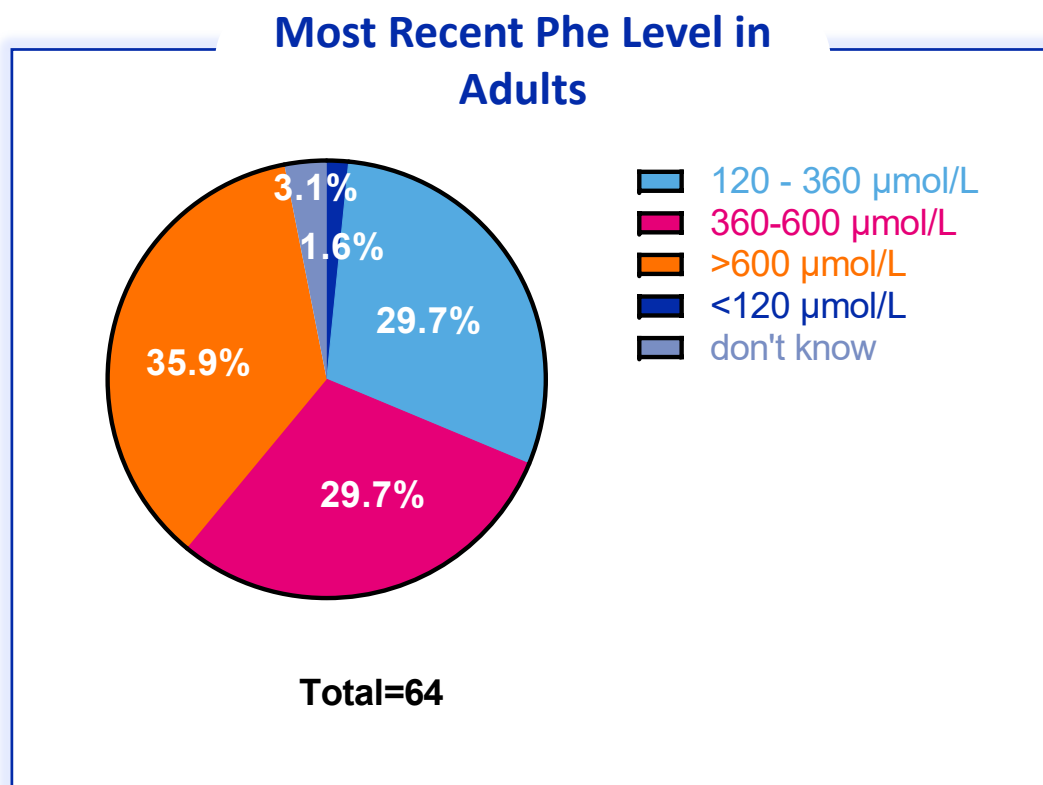
Children



Total=77

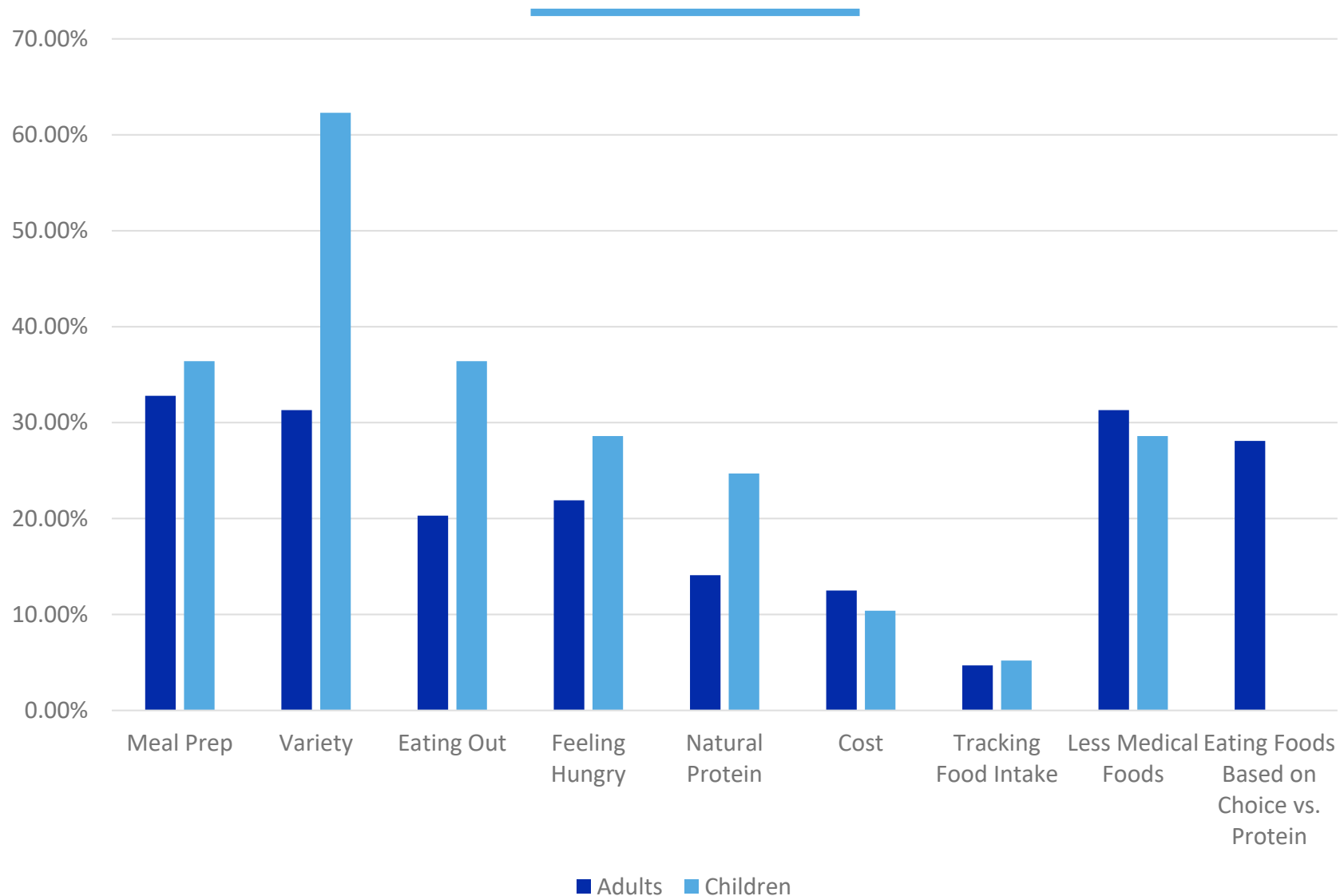
Large proportion of PKU patients not at target

2 in 3 adults and 1 in 3 children did not reach target of < 360 $\mu\text{mol/L}$



Clear unmet need for novel therapies despite existing treatment options

What are the biggest challenges of the PKU diet?

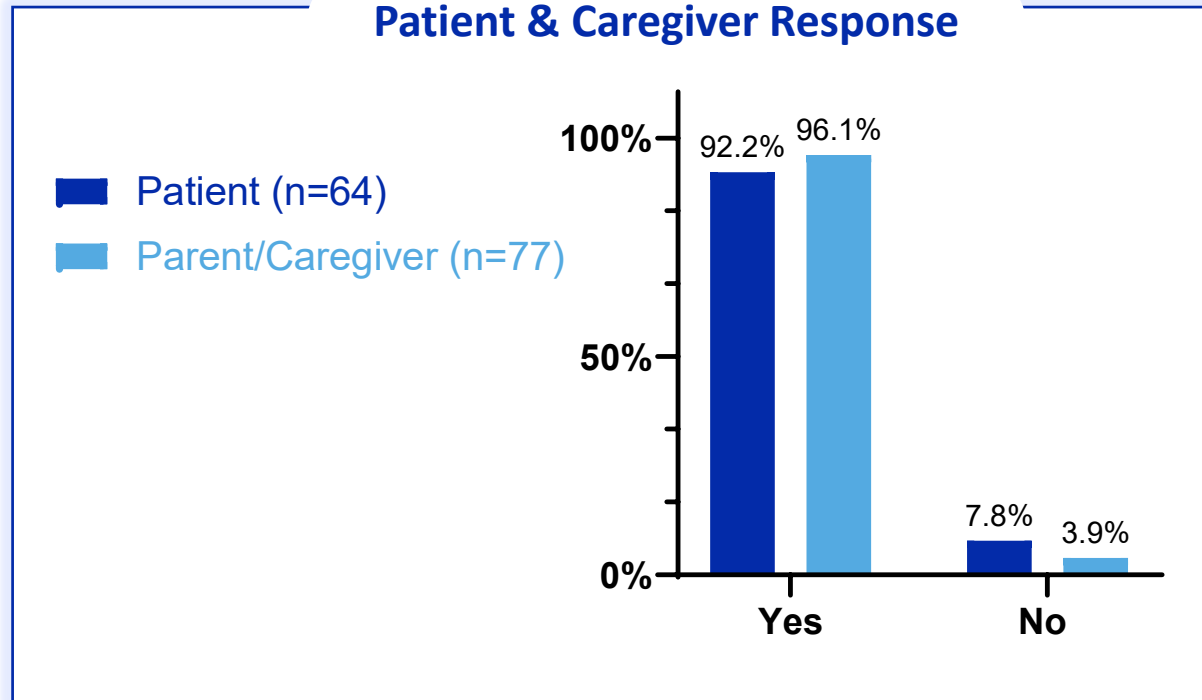


The PKU diet is clearly difficult to prepare and lacking in variety

Would you like to increase the amount of natural protein in your diet?

A small amount (2-3g) of additional natural protein is considered meaningful

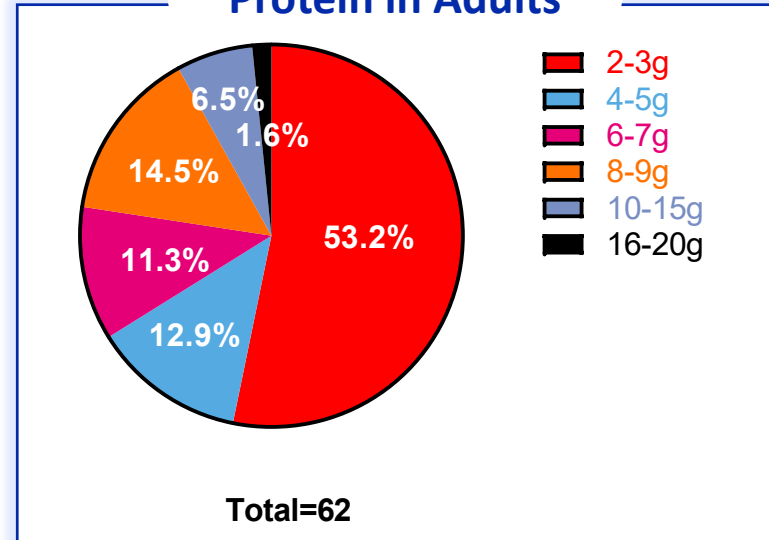
Patient & Caregiver Response



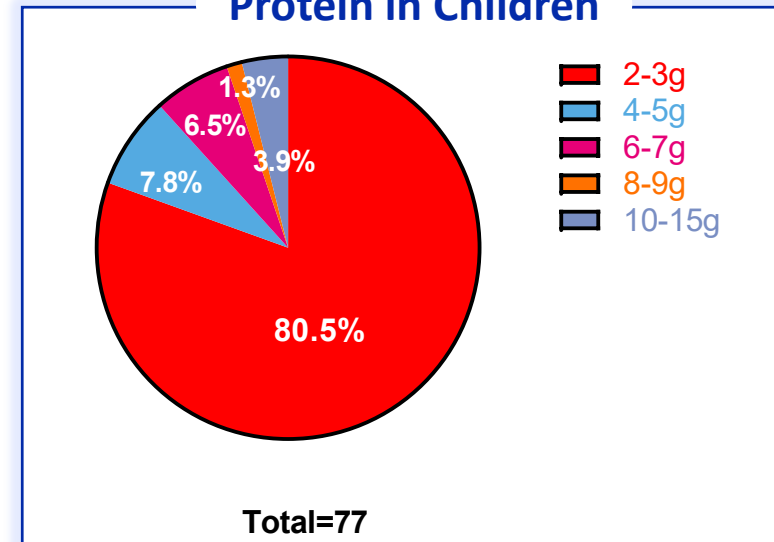
Examples of foods with 2-3g of protein:

1 slice white bread, 1/2 cup broccoli, 1/2 cup regular cereal, 1 medium potato

Protein in Adults



Protein in Children



Key takeaways

Conducted together with National PKU Alliance and Canadian PKU and Allied Disorders

A significant proportion of both adult and pediatric PKU patients are not well controlled (Phe levels >360 $\mu\text{mol/L}$)

Virtually all PKU Patients (or their caregivers) want to increase the amount of natural protein in 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)

Thank You!

Find us on ClinicalTrials.gov

ct.gov #: NCT04534842

Visit our study website

<https://pkuresearchstudy.com/>

Contact us at

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