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Impact of the Formula Shortage on Patients with Inborn Errors of Metabolism (IEM) & Dietitians

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BACKGROUND

Patients with Inborn Errors of Metabolism (IEM)

- Inborn errors of metabolism (IEM) represent a large group of rare, inherited genetic disorders.
- Rare diseases but collectively common (more than 500 types of IEMs, Incidence rate of ~ 1 in 2500 births).
- Mostly diagnosed in newborn screenings, but can be present at any age.
- The most frequent type of IEMs is Phenylketonuria (PKU).
- PKU patients are lack of the gene necessary for enzyme production, which is responsible for breaking down phenylalanine.
- A dangerous Build-up of phenylalanine in the body can develop when a person with PKU eats foods that contain protein or aspartame, an artificial sweetener.
- This can eventually lead to serious health problems, for instance, irreversible brain damage, intellectual disability, neurological problems, behavioral, emotional, developmental problems.
- Currently no cure for IEM patients, early recognition of the condition and prompt intervention can help.
- Specialized medical formulas or supplements play a crucial role in providing nutritional support for patients with IEM.

Medical Formula Shortage Since 2022

- Unexpected medical formula shortage happened in part by voluntary recalls of several formulas produced by Abbott Nutrition since the shutdown of the plant in Michigan in February 2022 due to bacterial contamination (United States Food and Drug Administration, 2023), and ongoing supply chain interruptions resulting from the COVID-19 pandemic (Calder et al., 2021).
- To date, no study focused on the impact of medical formula shortage on patients who are dependent on metabolic formulas and their dietitians.



RESEARCH QUESTIONS

- What is the long-term psychosocial implications of the formula shortage on patients with IEM?
- Does the experience of cutting back on medical formula due to the unexpected formula shortage affect patients' negative weight perception via patient-dietitian relations?

METHODS

Recruit Participants and Survey Data

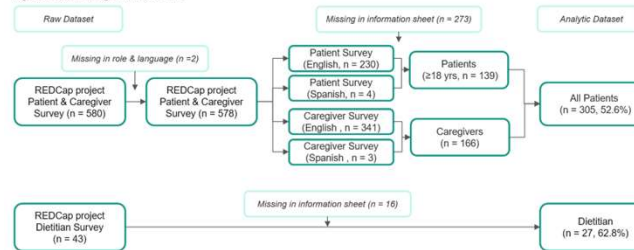
- Interested patients with IEM and metabolic dietitians in the US were invited to the survey between December 2023 and January 2024.
- Participants provided informed consent prior to completing a brief survey, including questions about demographic information, type of metabolic disorders, role of caregivers (for caregiver survey only), and language they choose to response.
- Survey responses from patients and dietitians with two language options (English/Spanish) were separately collected into REDCap database.

- The patients/caregiver survey asked about psychosocial impact, medical formula access and alternatives, formula shortage related symptomology, clinic-patient relations, and PKU-specific question.
- The dietitian survey asked about emotional burden, clinical burden, dietitian-patient relations, and practical impact of the formula shortage.

Data Management and Wrangling

- The raw survey data for patients (18 years-old or older) /caregivers and dietitians were separately exported from REDCap database with information sheets as csv files.
- R software was used for all data management and wrangling procedure as well as statistical analyses.
- After removing missing responses in information sheet, the analytic data for all patients consists of 305 responses (52.6%) in total. For dietitian data, 27 responses (62.8%) are retained from the raw data (Figure 1).

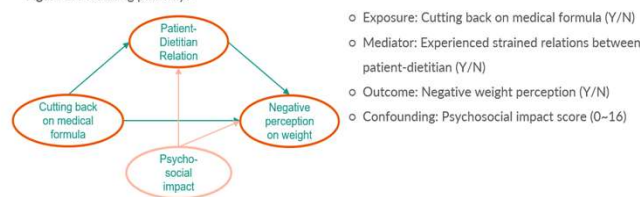
Figure 1. Data management flow chart



Statistical Analysis

- To assess nutritional and psychosocial impact on patients for type of metabolic disorders, mean response score of each question was analyzed using two-sample t-tests.
- To see whether there is a difference in perception of loss of trust between patient-dietitian, the same question was asked to both patients and dietitians. The frequency of responses for two groups was analyzed with Chi-squared test.
- To investigate the association between patients' negative perception on weight from cutting back on medical formulas, potentially through a mediating variable of patient-dietitian relations, a series of logistic regression models were performed.
- The path from cutting back on medical formula to negative perception on weight is the direct effect, while the path from cutting back on medical formula to negative perception on weight through patient-dietitian relation is the indirect effect (Figure 2).
- The mediate package in R was used for modelling with complete dataset.

Figure 2. Mediating pathways



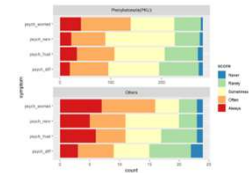
RESULTS

Psychosocial Impact for Type of Metabolic Disorders

- Patients with other type of disorders showed relatively higher mean response scores compared to PKU patients for all questions asking about psychosocial impact, but those differences between two groups were not statistically significant.

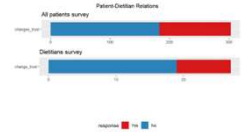
Impact score	Type of Metabolic disorders		p-value ²
	Phenylketonuria (PKU) N = 277	Others N = 23	
Nervous	2.17 (0.88)	2.52 (1.08)	0.15
Worried	2.42 (1.00)	2.83 (1.11)	0.10
Difficult to handle	2.06 (0.96)	2.09 (1.20)	0.93
Frustrated	2.19 (1.03)	2.43 (1.24)	0.37

Mean (SD)
¹ Welch Two Sample t-test



Perception of changes in trustful relations

- About 40% of patients responded they had experienced loss of trust in relations with dietitians. For dietitians, it was about 30% and the difference was not statistically significant (Chi-squared test, p-value = 0.42).



Mediation Analysis

- Patient's experience of formula cutbacks was related to strained patient-dietitian relations, adjusting for psychosocial impact (OR = 2.94, 95% CI = 1.75, 4.96).
- Strained patient-dietitian relation was significantly associated with negative perception on weight (OR = 2.86, 95% CI = 1.53, 5.44).
- The experience of cutting back on formula was not significantly associated with negative perception on weight, adjusting for psychosocial impact and strained relation (OR = 1.84, 95% CI = 0.92, 3.84).
- Finally, there was a significant indirect effect of formula cutbacks on negative weight perception via strained patient-dietitian relations (OR = 1.04, 95% CI = 1.01, 1.07). A complete mediation was detected.

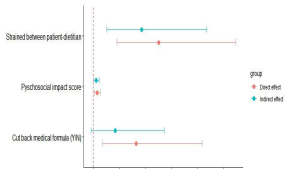


Figure 3. Forest plot comparing direct vs. indirect pathways

DISCUSSION

- The study addressed the adverse impact of unexpected medical formula shortage on patients' mental and emotional health, regardless of the type of metabolic disorders.
- Medical formula cutbacks due to the unexpected shortage may have had negative impacts on patients' weight perceptions via their increasingly strained relationships with dietitians.
- These results have implications for dietitians, highlighting the importance of maintaining positive relationships with patients which may improve nutritional outcomes of patients.

REFERENCES

1. Medical Formula for Inborn Errors of Metabolism, Medical Policy, Blue Cross
2. Mayo Clinic, Phenylketonuria (PKU)
3. Mothers' Experiences During the 2022 Infant Formula Shortage in Washington D.C., Sylvestry et al.
4. Causal Mediation Analysis in Pediatric and Perinatal Epidemiology: Maternal Obesity and Childhood Asthma, Natalie A. Rosenquist et al.
5. Review: mediation Package in R, Adam C. Sales, Journal of Educational and Behavioral Statistics
6. Incidence and genetic variants of inborn errors of metabolism identified through newborn screening: 7-year study in eastern coastal areas of China, Shuai Men et al., Molecular Genetics & Genomic Medicine