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A three-month trial of PKU sphere™ to assess potential clinical benefits in comparison to taking an amino acid based protein substitute.

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Patient Details & Medical History

Age: Adult

Gender:

Diagnosis: Diagnosed with PKU on newborn screening.

Initial Presentation: Patient attended the metabolic clinic reporting symptoms including fatigue and mild anxiety.

Relevant History

Dietary: Patient followed the low protein diet strictly during childhood, but in adulthood found adhering to protein substitutes and the low protein diet increasingly difficult.

Dietetic Assessment

GMP-based protein substitutes were newly available and have been suggested to improve dietary adherence in adult PKU patients.[1,2]

Overall aim

A three-month trial of PKU sphere was conducted to assess what (if any) clinical benefits were experienced by the patient, compared to when taking his usual amino acid-based protein substitute at baseline.

Dietetic Intervention

Assessments: All parameters were completed at baseline (whilst taking usual protein substitute) and after 3 months of PKU sphere intake including:

- **Biochemical parameters:** plasma phenylalanine: indicating metabolic control and vitamin B_{12} and folate: indicating nutritional status.
- **Dietary adherence and nutritional intake:** dietary assessments completed by specialist metabolic dietitian.
- **Psychosocial outcomes:** Fatigue Severity Scale [3], SF-36 [4], assessing quality of life, cognitive failures questionnaire [5] assessing cognitive functioning, Hospital Anxiety and Depression Scale [6].
- **Palatability and tolerance:** self-reported by patient during dietary assessments.

Results

Parameter	Reference Range	Baseline	Following 3 month trial	Clinical Interpretation
Plasma phenylalanine (µmol/L)	120 - 600	1297	560	Clinically significant improvement
Plasma Vitamin B_{12} (pg/mL)	197 - 771	264	416	Clinically significant improvement
Plasma Folate (ng/mL)	2.9 - 26.8	5.1	10.6	Clinically significant improvement
Protein intake (g/day)	n/a	<0.5g/kg/day	>1g/kg/day	Clinically significant improvement
Fatigue Severity Scale	n/a	22	12	Clinically significant improvement
Quality of life (SF-36)	n/a	88 (45th %ile)	98 (75th %ile)	Stable
Cognitive failures questionnaire	n/a	26/100	3/100	Clinically significant improvement
Hospital Anxiety and Depression Scale	n/a	A - 9 (86th %ile, mild anxiety) D - 6 (64th %ile, normal range)	A - 2 (18th %ile, normal range) D - 1 (28th %ile, normal range)	Clinically significant improvement in anxiety symptoms

Discussion

The patient reported that PKU sphere was more palatable and tolerable compared to his previous amino acid-based protein substitute. This is undoubtedly important for patients who are expected to follow a restrictive and difficult lifelong diet to effectively manage their condition.

Overall, use of this product has allowed the patient to achieve considerably better metabolic control and achieve the target for phenylalanine recommended in European guidelines for PKU [7]. His nutritional status has also improved, as evidenced by improvements in biochemical markers and dietary assessment of total protein intake.

The patient completed several validated, self-reported questionnaires to assess psychosocial outcomes (on advice from a neuropsychologist). All assessments revealed improvements in fatigue, quality of life, cognitive functioning and anxiety after taking PKU sphere for 3 months.

Following the 3 month trial, the patient continued to take PKU sphere. Bloodspot results remained much improved (655µmol/L and 475µmol/L) compared to their baseline plasma level (1297µmol/L) whilst taking the previous protein substitute. This demonstrated that the patient was able to achieve longer-term adherence, whilst taking PKU sphere as part of a therapeutic diet.

Conclusion

The data presented supports that PKU sphere improved clinical outcomes and dietary adherence for this patient, compared to when taking their previous amino acid-based protein substitute.

Note:
This was not a controlled study and so it is not possible to demonstrate a causal relationship between the patient's change in diet and improvement in self-reported symptoms. In addition, this informal process of trialling a new product and recording several outcomes before and after was developed in conjunction with the patient. This was to support the case for access to his preferred protein substitute from his healthcare provider. This type of external motivation can sometimes affect results.

References:

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