



Ein cyf/Our ref VG/00148/20

**Llywodraeth Cymru**  
**Welsh Government**

Angela Burns AM  
National Assembly for Wales  
Cardiff Bay  
CF99 1NA

10 March 2020

Dear Angela,

I am writing further to our exchange in plenary on phenylketonuria (PKU) on 12 February 2020.

I understand from officials that NICE started to appraise Kuvan™ (sapropterin), but the manufacturer withdrew from the appraisal on 31 October 2019 so the appraisal is suspended. Therefore, a clinician would need to make an individual patient funding request (IPFR), if they believe their patient would benefit from accessing this drug, as NICE could not recommend this medicine without appraising it.

In relation to the diet, as you highlighted People with PKU can only eat a very limited diet. They are unable to eat many foods such as meat, fish, eggs, cheese, milk, nuts, ordinary bread, pasta and other cereal products. There are few normal foods they can eat, therefore, the diet requires supplementation with many low protein foods that are available on prescription.

The Advisory Committee on Borderline Substances (ACBS) is responsible for advising on the prescribing of nutritional and dermatological products for use in NHS primary care. There are a wide range of protein substitutes available in different forms such as infant 'milks', drinks and gels in different flavours and also tablets and capsules. There are over 100 low protein foods available on prescription. Those approved can be found under the borderline substances listed in the drug tariff. Patients with PKU should discuss accessing these products with their dietician or GP as part of a personalised, balanced diet.

Finally, in relation to National Society for Phenylketonuria recommendations as I stated in plenary if you write to me I would be happy to ask my officials to share these with the rare disease implementation group for their consideration.

Yours sincerely,

A handwritten signature in black ink that reads "Vaughan Gething".

**Vaughan Gething AC/AM**  
**Y Gweinidog Iechyd a Gwasanaethau Cymdeithasol**  
**Minister for Health and Social Services**

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Rydym yn croesawu derbyn gohebiaeth yn Gymraeg. Byddwn yn ateb gohebiaeth a dderbynir yn Gymraeg yn Gymraeg ac ni fydd gohebu yn Gymraeg yn arwain at oedi.

We welcome receiving correspondence in Welsh. Any correspondence received in Welsh will be answered in Welsh and responding in Welsh will not lead to a delay in responding.