



Children's Health Ireland

Sláinte Leanaí Éireann (SLÉ), Herberton, Siúlán San Séamas, Rialto, D08 HP97, Éire  
Children's Health Ireland (CHI), Herberton, St James's Walk, Rialto, D08 HP97, Ireland

14<sup>th</sup> December 2020

Deputy Paul Murphy,  
Leinster House,  
Kildare Street,  
Dublin 2.

**PQ 40672/20**

"To ask the Minister for Health the status of the availability to patients with PKU in Dublin and throughout Ireland of the HSE-approved medicine kuvan; the reason kuvan it is not being made available to children with PKU at Temple Street Hospital or adults with PKU at the Mater Misericordiae University Hospital; the reason the care pathway stipulates that patients must be admitted for 48 hours to have blood spot tolerance tests done since these are the same tests that patients do on a weekly basis at home leaving some with no option but to travel to another country to have the testing done; and if he will make a statement on the matter".

Dear Deputy Murphy,

I refer to your parliamentary question above to which I have been asked to provide a response.

The National Centre for Inherited Metabolic Disorders (NCIMD) is located in Children's Health Ireland (CHI) at Temple Street. Since August 2019 any newborns diagnosed with PKU have been tested in CHI at Temple Street and treated, when responsive, with Kuvan. In addition, a number of other known responsive patients have been treated. CHI looks forward to expanding the Kuvan treatment programme when additional resources are in place to support the pre and post phases of this new drug treatment regime. The additional key resources required are dietetic, nursing, laboratory and medical to especially assist at the pre-testing, testing and early treatment phases of Kuvan treatments. CHI has put in place an interim plan to support Kuvan treatment in 2020 while we seek to secure the required resources to extend this treatment. In the meantime, working within our current staffing levels (medical, nursing and dietetics) in CHI at Temple Street, Kuvan has been available and trialled on all newly diagnosed infants with the condition Phenylketonuria since August 2019. A number of patients (known patients established on diet) have been highlighted by the team based on need and these children have also been trialled on the medication.

In March 2020, due to the COVID-19 pandemic and national guidance, all elective admissions for trial of Kuvan were placed on hold, both newly diagnosed infants and established patients. The Metabolic team continued to prepare our patients for trialling of the medications however and have been working to establish a suitable pathway for elective admissions, taking into account the need to ensure physical distancing and other Infection Prevention and Control guidance.

**Mission – To promote and provide child-centered, research-led and learning informed healthcare, to the highest standards of safety and excellence. We do this in partnership with each other, with children, young people and their families through a network of children's services in Ireland.**

**CRA Registered Charity Number (RCN): 20202295 Revenue Charitable Status CHY: 22235**

**Board Members:** Prof James Browne (Chairman), Prof Niamh Brennan, Mr Con Cronin, Ms Mary Cryan, Mr Liam Dowdall, Prof Martin Elliott, Ms Catherine Guy, Dr Anne Kilgallen, Dr Gavin Lavery, Ms Brigid McManus, Mr Turlough O'Sullivan, Mr Sean Sheehan



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There are three cohorts of PKU patients to consider :

1- All neonates who are diagnosed with PKU via the National Newborn Bloodspot Screening Programme since August 2019 when Kuvan (Sapropterin Hydrochloride) became available have a Kuvan trial and if found responsive, they continue on treatment.

2- In early 2020 we commenced admissions for established patients where we cannot predict the response to Kuvan from the genotype for a Kuvan trial over 48 hours. This involves 2 doses of the drug and 7 blood samples for Phenylalanine analysis (time: 0, 8, 16, 24, 32, 40, & 48 hours). All elective admissions were stalled for a number of months due to Covid 19 pandemic but are again underway.

3- There is also a small cohort of patients who we know to be fully responsive based on their genotype, these patients are being started on Kuvan directly as an outpatient without the need for admission.

With cohort 2 and 3 there is a significant outpatient workload involved from dietetic, nursing and medical services preparing patients prior to initiating testing / treatment. All as per HSE protocol /guidelines for Kuvan, 42 patients have already been admitted, with further patients scheduled to come in.

Yours sincerely,

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