



# PKU

## ASSOCIATION OF IRELAND

*Raising awareness and supporting the PKU community in Ireland*

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20th January 2020

Dear Mona Baker CEO,

I am writing to you to draw your attention to the scandalous situation faced by patients with PKU throughout Ireland, their carers and community as a whole, whereby the HSE approved medicine Kuvan is not being made available to children with PKU at Temple Street Hospital.

Kuvan is the only licensed medicine for children with PKU. It originally gained regulatory approval in 2009, but was only approved for reimbursement by the HSE in **January 2019** following public and political outcry. We are now faced with the unacceptable situation whereby the Care Pathway for Kuvan cannot be implemented by the country's only children's metabolic service at Temple Street, and only adult metabolic service at The Mater Hospital. Neither can provide families and/or patients with any timelines whatsoever for when they can be tested to see if they respond to Kuvan.

After an extremely protracted, frustrating and exhausting highly publicised process by the PKU community to finally get the HSE to approve the medicine, we feel that we have no further option but to highlight the systematic obfuscation resulting in children being denied access to a medicine to which they are officially entitled by HSE processes. This will involve engaging with the broad stakeholder group of journalists, policy makers and legislators who supported the approval of Kuvan, and whom we are sure will be perplexed and frustrated as to why it is still not being made available to Irish patients after all the years of delays. In order to establish whether patients will respond to Kuvan, and its potentially life-changing benefits, some have no option but to travel to another country to have the testing done.

The current care pathway stipulates that patients must be admitted for 48 hours to have blood spot tests done at regular intervals. These are the same tests that we do on a weekly basis at home, so we see no reason whereby the 48 hour tolerance/loading test can be done in the home setting? This would at least overcome the obstacle of requiring a hospital bed, which we all know is a very significant rate limiting step in our healthcare system.

The PKU community has been patient and respectful and feels deeply that after waiting for over 10 years, they can wait no longer.

I look forward to hearing from you. Warm regards,

*PP Keren Willetts*

PKU Association of Ireland – Fergus Woodcock Chairperson

CC.

Eilish Hardiman, CEO Children's Hospital Group  
Prof Ellen Crushell  
Simon Harris, TD, Minister for Health  
Joanne Lonergan, Gov  
Gino Kenny, TD  
Paul Reed, HSE  
Metabolic Department, Temple Street  
Prof Michael Barry, NCPE  
John Brassil, TD  
Senator John O'Mahony  
Jim Breslin, Secretary General, Health