Date

Re: Please help my son/daughter/Friend/colleague/neighbour with PKU

Dear Minister Harris,

I am writing to ask for your help and for a meeting with you urgently regarding my son/daughter/Friend/colleague/neighbour, NAME (optional) who has PKU.

Please support the PKU community in their mission to gain HSE access to Kuvan, a medicine that can dramatically improve the quality of life for some PKU patients.

My son/daughter/Friend/colleague/neighbour, NAME (optional)   was diagnosed with PKU after birth, following the heel prick test. PKU means NAME (optional) is unable to break down an amino acid called phenylalanine (PHE), a natural substance found in food.  The result is a build-up in the blood and brain, which can cause serious health problems, including severe brain damage.

PKU occurs in 1 in every 4,500 live births in Ireland- one of the highest rates of PKU in the world. It’s estimated that there are over 700 people living with PKU in Ireland today. Most of these cases are associated with specific ‘Celtic’ gene mutations.

Thanks to political and clinical vision and leadership in the 1960’s, Ireland was at the forefront of PKU, through pioneering newborn screening and dietary intervention. This helped avoid some of the most severe complications for people with PKU, whilst also dramatically lowering the costs of long term care and medical complications.

However, Ireland now trails behind all developed country’s healthcare systems in terms of modern standards of care for people with PKU. It is now recognised in the international PKU research community that dietary intervention alone is inadequate. Despite enormous effort by people with PKU, their families, carers and clinicians, it is documented that most people (70%) with PKU fail to adequately adhere and cope with the gruelling restrictions, resulting in dramatically reduced quality of life, as well as inadequate control of their PHE levels, leading the long-term brain damage. Indeed, the highly restricted diet itself is associated with additional long-term complications such as osteoporosis, diabetes, renal disease, as well as neurocognitive effects such as depression, anxiety, poor concentration, mood swings, agitation etc.

The NCPE recently concluded its 2nd Pharmacoeconomic evaluation of Kuvan and has considered it not to be cost effective, at the original price submitted. We understand that the report now goes to the HSE for assessment and price negotiations with the manufacturer. I urge you to intervene as a matter of urgency to ensure access to Kuvan for Irish patients.

It is now widely acknowledged that the specific process used by the NCPE (QALY/ ICER measurement) to assess medicines for rare diseases is flawed. Rare diseases, by their very nature, are very challenging to research and are therefore unable to satisfactorily provide the inputs necessary to prove cost effectiveness. Kuvan has been assessed twice by the NCPE: 1st in 2009 and again just recently between July 2015 and Sep 2017. This process is unfair to rare diseases and both protracts and detracts resources from contributing to actual PKU care.

The recently published European Society for PKU (ESPKU) guidelines state that all people with PKU should undergo a trial of Kuvan to assess if they can benefit from it. Unfortunately, most ‘Celtic’ gene variants do not respond well. However, this means that the budget impact of introducing Kuvan in Ireland will be small as the number of responders will be small. It should also be noted that the list price cost of Kuvan is other countries where it is available is not in any way comparable to recent high profile new rare disease medicines such as Soliris or Orkambi.

Kuvan received EU regulatory approval 8 years ago in 2009 and is currently reimbursed in:

* Austria
* Belgium
* Bulgaria
* Czech Republic
* Denmark
* Estonia
* France
* Germany
* Hungary
* Italy
* Netherlands
* Lithuania
* Luxembourg
* Norway
* Portugal
* Romania
* Russia
* UK (maternal PKU)
* Sweden

**Why not in Ireland?**

The PKU community does not understand why the drug is not available in Ireland, when it is widely available across the EU and worldwide?

**We need your help?**

I am appealing to you to as Minister for Health to show leadership and compassion around this issue on behalf of the PKU community and to intervene with the HSE.

I look forward to hearing from you and would be very keen to meet to discuss the above.

Kind regards,

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NAME