*Date*

*Dear [TD] /*

I am writing to you for your help and support to intervene where an opportunity to dramatically improve the lives of people with PKU is about to be lost forever.

(My child / my grandchild XXX/ I) was born in (year XXX) and was diagnosed with phenylketonuria or PKU, through the national newborn blood-screening (heel prick) test after birth, for which I am eternally grateful.

PKU is a rare genetic disorder that affects (my / my child’s / my grandchild) metabolism. People with PKU are unable to break down an amino acid called phenylalanine (phe), a natural substance found in food. This results in a build up of phe in the blood and in the brain, which can cause serious health problems, including if untreated, severe brain damage.

The only medical treatment for PKU, Kuvan, can dramatically help some people with PKU vastly improve their quality of life. The drug enables more ‘normal’ food to be eaten, and critically improves cognitive functioning ‘reducing’ the real threat of long-term irreversible brain damage.

Kuvan received EU regulatory approval in 2009 and is currently approved for use and reimbursed in: Austria, Belgium, Bulgaria, Czech Republic, Estonia, France, Germany, Hungary, Italy, Netherlands, Luxembourg, Norway, Portugal, Romania, Russia, UK (maternal), areas of Sweden, Lithuania and Denmark.

**So why not in Ireland?**

The PKU community cannot see any justification for the drug not being available in Ireland on such a relatively small scale, when it is widely available across EU, the rest of Europe and worldwide*.*

Since 2010 Kuvan has been assessed by the NCPE twice and rejected on both occasions (2009 & 2017). The PKU community are frustrated with this process and feel it’s unfair, as it’s designed to fail ‘orphan drugs’, which don’t have the necessary data. It is an expensive and lengthy process for small organisations such as PKUAI, while also costly for the HSE (please see full process breakdown below).

**How can you help?**

We call on you to demonstrate political leadership and mobilise resources around this issue and intervene on behalf of all those struggling to live with PKU every day, within your community and throughout Ireland. The ultimate decision regarding drug funding availability for PKU in Ireland rests with the Minister for Health and the HSE.

I urge you, as my local representative, to support the PKU community and request the HSE to consider Kuvan for funding, weighing up the significant beneficial outcome in long-term health improvements for the lives of so many people with PKU, with a moderately small overall impact on budget.

I would appreciate you taking this up with the relevant government minister on my behalf, and I look forward to hearing your own, and the government’s thoughts on the matter soon.

Should you require any further detail information about my story or PKU, please do not hesitate to let me know. Please review the attached information below.

Yours sincerely,

XXXXX

*Attached information below*

* *National Media coverage – RTE Radio 1 & The Irish Times*
* *Newborn screening*
* *Classic PKU*
* *Flawed NCPE Test Process*
* *Media release re: NCPE reject Kuvan – PKU Only Treatment*

**PKU information;**

**Recent NATIONAL media COVERAGE**

* ***Interview with RTE Radio 1 Ray D’Arcy show***

***Monday 25 September, 2017***

*20 minute interview with Ray and PKU parent: real day-to-day battle*

* + [*https://www.rte.ie/radio1/ray/programmes/2017/0925/907408-ray-darcy-monday-25-september-2017/?clipid=102613523#102613523*](https://www.rte.ie/radio1/ray/programmes/2017/0925/907408-ray-darcy-monday-25-september-2017/?clipid=102613523#102613523)
* ***Irish Times News***

***Tuesday 26 September 2017***

***Article highlighting NCPE rejecting only drug to assist PKU***

* + <https://www.irishtimes.com/news/health/pku-case-study-ciara-9-and-luke-4-willetts-1.3233504?mode=amp>
  + https://www.irishtimes.com/news/health/drug-for-pku-to-be-withheld-by-hse-on-cost-grounds-1.3233471?mode=amp

**Newborn screening**

Irish policy makers and clinicians introduced screening for newborns in the 1960’s, as without radical intervention from shortly after birth, babies with PKU would be extremely brain damaged and require huge levels of care.

**Classic PKU**

Harshest strain of PKU, common to Ireland. People with Classic PKU can eat the least amount of protein for life.

**NCPE Drug Test Process**

* The drug review with NCPE is set up to fail ‘orphan drugs’ - these drugs are put through endless assessments, which they can never win due to their poor scoring 'QALY' - quality of adjusted life.
* To get a reliable QALY you need big clinical trials - common for things such as oncology or heart disease.
* In simple terms, for example; a cancer drug could give an additional year of life for €10,000 so the cost of that QALY is €10,000.
* Sadly PKU and other rare diseases cannot show examples for orphan drugs because you simply can't do the clinical trials to do good QALY.
* There is a misconception that these orphan drugs are just expensive which sometimes is the case, while the reality is often the fact that the QALY is unreliable, but this will always be the case in orphan drugs because you can't get reliable clinical trials with small amounts of patients/data.

**Health PRESS Release**

**From: PKUAI – PKU Association Of Ireland**

**NCPE REJECT RARE DISEASE PKU ‘WONDER’ DRUG With Flawed Test**

**- PKU Patients Denied Only Treatment for Lifelong Incurable Condition**

The only drug treatment available for people with Phenylketonuria (PKU), a rare metabolic condition has been rejected by the NCPE.

The National Centre for Pharmacoeconomics (NCPE) denied approval for the drug Kuvan after a protracted process. For people living with this challenging condition, the drug can significantly increase their quality of life and assist with the prevention of severe brain damage.

PKU is a rare metabolic condition for which Ireland has one of the highest rates, with most severe cases\* in Europe.  Approximately 1 in every 4,500 babies is diagnosed with PKU, and everyone born with the condition must adhere to a strict low-protein diet or irreversible mental and physical disabilities will develop.

Kuvan is widely available throughout Europe since 2009 and critically allows users increased amounts of ‘normal daily food’ to consume and improves immediate and long-term mental functioning.  Kuvan is currently approved for use and reimbursed in: Austria, Belgium, Bulgaria, Czech Republic, Denmark. Estonia, France, Germany, Hungary, Italy, Netherlands, Luxembourg, Norway, Portugal, Romania, Russia, UK (maternal), areas of Sweden, Lithuania and Denmark.

Speaking about the result, Fergus Woodcock, Chairman, PKUAI (PKU Association of Ireland) said:

 “***We are hugely disappointed Kuvan, the only drug treatment for PKU, has been rejected after such a lengthy review.   PKUAI are frustrated with the NCPE drug process as it is fundamentally designed to fail vital rare disease drugs, such as Kuvan****”.  He added: “****PKUAI call on Minister for Health Simon Harris, TD, and the HSE to make the right decision and approve funding, weighing up the significant outcome in long-term health improvements for the lives of so many PKU people, with a moderately small overall impact on budget***”.

A PKU diet removes foods most people take for granted, including meat, fish, poultry, cheese, milk, bread, flour, rice, pasta, yoghurt, chocolate and all products containing the widely used sweetener aspartame.   In addition, all other foods consumed must be strictly monitored for protein levels.  Diet is then balanced out with protein free foods and specially manufactured foods where the protein has been removed.

***Harsh reality***

Even when the grueling PKU diet is maintained, people managing the condition can still suffer long-term and permanent health conditions such as neurocognitive deficits, neurological side effects, growth defects, bone mass reduction, and obesity (which can be assisted with Kuvan).

**Acclaimed metabolic Consultant Gregory Pastores**, based in Adult Metabolic Service at Mater, Misericordiae University Hospital, with extensive experience working with PKU condition, commented:

“*Patients with PKU require a strict and highly restricted diet to be maintained for life, which is a constant daily challenge. Uncontrolled, elevated Phe levels can have an adverse impact on short and long-term health, particularly on brain function. Moreover, affected women with poor control during pregnancy can lead to unfavourable consequences for their child. I am hopeful groups of patients with* *the greatest and most urgent clinical need and at highest risk of adverse outcomes are given the opportunity to avail all options to optimize their care”.*

The PKUAI strongly urge the HSE and Government to engage with Kuvan manufacturers and approve funding for the only drug treatment available for PKU. It is already available in 9 other EU countries.  Giving access to this life-changing drug for all eligible to transform the future health of PKU people throughout Ireland.

**ENDS.**

**PKUAI PR contact: Emer O’Reilly @ +353 86 859 3658**

**For further information on PKU check out** [**PKUAI web site**](http://www.pku.ie)