



European Society for Phenylketonuria and allied disorders treated like Phenylketonuria

www.espku.org

President

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To whom it may concern,

About ESPKU:

ESPKU was found in 1987 and represents the interests of afflicted patients from 41 countries, including Ireland. The society's aims and objectives are to promote educational and social welfare of persons affected of PKU, and to stimulate scientific and medical research. Accordingly, we support every improvement of treatment and care, and we support all activities to establish access to all available information and care.

About Phenylketonuria:

PKU is an inherited disorder of protein metabolism.

If left untreated, PKU results in increased phenylalanine concentrations in blood and brain, which cause severe, irreversible intellectual disability, epilepsy and behavioural problems

Management of PKU is a strict low - phenylalanine diet in combination with Phe-free L-amino acid supplements, which allows patients to have a near normal development. This therapy avoids the worst complications of PKU. There is however a growing body of evidence that there are still very significant side effects and limitations associated with it. For some patients the constraints of dietary treatment make it difficult to adhere to, particularly during adolescence. The control of phenylalanine intake must be strict and it is important to remember that the dietary limitations not only affect the patient but their families as well.

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In addition to this established low protein diet, there exists a pharmaceutical treatment option (BH4, brand name "Kuvan®"). This allows responding patients to have a better control on their phenylalanine levels and / or to follow a less strict diet and have a better quality of life.

About the situation in Ireland:

Ireland is one of the few countries where this is unavailable. This pharmaceutical product is indicated for the treatment of PKU. We have been invited by the representatives of the PKU Association of Ireland to support their campaign for a decision to reimburse the costs of this pharmaceutical treatment. The PKU Association of Ireland informed us, that so far there is no final decision to reimburse or subsidize Kuvan® in Ireland

Conclusions:

The PKU patient community is convinced of the benefits provided by Kuvan® in the management of the disease and for the improved quality of life. There is a growing body of evidence that there may be neurocognitive benefits, independent of dietary liberalisation, across the spectrum of PHE tolerance. Clinical studies have so far demonstrated the efficacy and the utility of Kuvan® for the treatment of PKU.

We strongly believe, that you do not neglect rare diseases, and ensure that patients in Ireland

for whom Kuvan® could be beneficial will be identified and have access to medication and that this will be fully reimbursed

Yours sincerely

Eric Lange
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