



International PKU Day: European Parliament Cross-Party Alliance on PKU and ESPKU jointly launch campaign to ensure all rare disease patients continue to benefit from future innovation

Today, International PKU Day, marks the launch of an advocacy campaign to ensure that the foreseen revision of the EU Orphan Medicinal Products (OMP) Regulation will continue to benefit all people living with rare diseases, including Phenylketonuria (PKU). This campaign is led by the European Parliament Cross-Party Alliance on PKU and the European Society for Phenylketonuria (ESPKU)¹.

Prior to the existence of the OMP Regulation, only 8 medicinal products were available for patients living with a rare disease. However, due to the incentives provided to companies to develop therapies for rare diseases (OMPs) – which are characterised by small patient populations and limited basic science – research and development of medicines in this area has been stimulated – and has made a real difference to people living with a rare disease: there are now over 200 products.

This Regulation is currently being revised and the advocacy campaign addresses two major concerns.

Firstly, we need to avoid a legally binding definition of ‘unmet medical need’. Given the complex nature of this concept, agreeing on a definition that takes all these relevant and determining factors into account would be virtually impossible.

Secondly, we need to maintain the current prevalence threshold for a rare disease; this threshold determines whether a particular rare disease is eligible for support under the Regulation.

‘Both these issues relate to the crucial need to ensure that the revised legislation does not exclude any rare disease patients from future therapeutic innovation and lead to unintended negative consequences’, said Eric Lange, President of the ESPKU.

Billy Kelleher MEP, co-chair of the European Parliament Cross-Party Alliance on PKU, stated that *‘the Alliance intends to continue to work to ensure that people with PKU and their families have lifelong access to the services, therapies and support to best manage the disease. The revision of the OMP Regulation provides a welcome opportunity to further improve the lives of people living with PKU and other rare diseases’*.

For more information:

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¹ This initiative is enabled by support provided by BioMarin

Notes for editors:

- 1 The OMP policy statement can be found at
<https://www.espku.org/wp-content/uploads/2022/02/OMP-advocacy-document-final.pdf>
- 2 The ESPKU was founded in 1987 as a patient-driven non-profit umbrella organisation representing PKU organisations across Europe. it currently consists of 41 national and regional associations from 31 countries. <https://www.espku.org/>
- 3 The European Parliament Cross-Party Alliance on PKU was launched in March 2020 with the aim to
 - bring together MEPs (and relevant) stakeholders to raise awareness of and propose and advocate specific policy initiatives to better address unmet needs in PKU across the EU.
 - ensure that individuals affected by PKU and their families have lifelong access to the services, therapies and support to best manage the disease and its daily impact.

It is coordinated by ESPKU and currently supported by 18 MEPs, with a further 13 MEPs endorsing a recent comprehensive Call to Action on Unmet Needs in PKU

<https://www.espku.org/cross-party-alliance/>