



The EU Strategy on the Rights of the Child

Statement by:

ESPku (European Society for Phenylketonuria and Allied Disorders Treated as Phenylketonuria) European Parliament Cross-Party Alliance on PKU

SUMMARY

What can the EU Strategy on the Rights of Child mean for children living with PKU?

- Support universal access to new born screening, to enable a healthy start in life and prevent avoidable neurological/ mental health issues later on
- Promote implementation and monitoring of European guidelines on minimum standards of care for children living with PKU
- Facilitate equal access to appropriate dietary and pharmacological therapy and innovation in order to ensure the highest standards of health
- Work towards early access to appropriate care regimes as well as appropriate transition from children's to adult treatment
- Facilitate clear and accurate protein food labelling to facilitate adherence to the required diet and healthy nutrition

Introduction

The European Society for Phenylketonuria and Allied Disorders Treated as Phenylketonuria (ESPku) and the European Parliament Cross-Party Alliance on PKU warmly welcome the proposed EU Strategy on the Rights of the Child, which states that 'children's rights are human rights, at the core of the EU objectives, ensuring all children can fulfil their potential'.

We can only applaud the Commission's ambition to 'build the best possible life for children in the European Union and across the globe to respect, protect and fulfil the rights of every child, and to build - together with children - healthier, resilient, fairer and equal societies for all'.

This document sets out our views in relation to the Strategy's relevance to children living with PKU and the rights of children living with chronic and/or rare conditions should be part of this initiative.

Who are we?

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.

The ESPku acts as the coordinator of the European Parliament Cross-Party Alliance on PKU, which 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 disorder and its daily impact.

What is PKU?

There are currently approximately 45,000 people in Europe living with PKU - a rare metabolic condition diagnosed through new-born screening. PKU affects a person's ability to break down phenylalanine, an amino acid present in protein. When phenylalanine builds up in the bloodstream, it can lead to intellectual and physical disability. Treatment consists of a protein restricted diet for life; however, research has demonstrated that adherence to dietary therapy as well as access to the foods required is a challenge for many people living with PKU. Pharmacological therapies with proven efficacy are available - but not to all individuals with PKU.

If not diagnosed and treated early in life, PKU can have lasting neurological, emotional, mental health, behavioural consequences and comorbidities – osteoporosis, high blood pressure, obesity, asthma, chronic ischemic heart disease – both in childhood as well as later in life.



The Strategy and Children Living with PKU

The EU Strategy on the Rights of the Child proposes actions across six thematic areas as well as the mainstreaming of children's rights across all relevant EU policies; it aims to take account of the specific needs of certain groups of children.

One group of children with specific needs, which could benefit from the proposed actions and principles proposed by the Strategy, are children living with PKU, and the Strategy contains a number of themes and principles which are highly relevant for this group, i.e.

- ✓ The section entitled 'Socio-economic inclusion, health and education' states that 'each child has the right to the highest attainable standard of healthcare, irrespective of their background and where they live.'
- ✓ In addition, the European Child Guarantee – part of the Action Plan on implementing the European Pillar of Social Rights and complementing this Strategy – calls for specific measures by Member States to guarantee access to quality key services for children in need, such as healthcare and nutrition.
- ✓ The Strategy also aims to complement ensure complementarity with the European Strategy for the Rights of persons with Disabilities to respond to the needs of children with disabilities and provide better access to mainstream services and independent living. This Strategy explicitly states the need to '*raise awareness and develop support strategies for patients with disabilities related to rare diseases and identify ways of facilitating access to state of the art treatment*'.

While the above is extremely welcome, we would like to highlight some specific concerns in relation to the rights of children living with PKU:

- ✓ The Strategy states a *'healthy diet, together with regular physical activity, is vital to children's full physical and mental development'*; also, it is said that the Commission will propose *'harmonised mandatory front-of-pack nutrition labelling to facilitate informed, healthy food choices'*.

In this context it is essential to bear in mind the importance of appropriate nutrition as part of treatment and dietary therapy – which can be crucial to children's health. To comply with dietary treatment, consistent, detailed and comprehensive nutrition labelling is required.

However, there is a lack of harmonised EU guidance and control on protein substitutes and low protein special foods – essential for those living with PKU. While the EU regulates for gluten, allergens, lactose, weight control foods, infant formula and baby food, this is not so for people depending on low protein diets. This adds to the burden (of caring) for children living with PKU and adherence to the required strict diet.

Moreover, in some countries it is difficult to obtain the specific low protein foods required for dietary therapy. These foods are often expensive and in many cases not reimbursed. This means that some children do not have access to the healthy diet they require.

- ✓ The document also underlines that childhood is crucial in determining future physical and mental health. It lists isolation, education, environment, social inclusion and poverty as important factors determining mental health. However, it is important to note that in some cases, mental ill health can be caused by other health conditions, such as PKU. Moreover, these types of mental ill health can be prevented by early diagnosis (in the case of PKU by newborn screening), followed by appropriate treatment. A healthy start early in life, - diagnosis, treatment, access to and adherence to diet will enable better health in later life.
- ✓ As part of ensuring the implementation of the stated right to the highest attainable standard of healthcare and access to key services, it is important to take account of existing treatment guidelines, specifying minimum treatment standards and promoting their implementation across the EU. In this context, access to and reimbursement of innovative treatment should also be facilitated.
- ✓ ESPKU and the Cross-Party Alliance on PKU caution against any changes to the EU Orphan Medicinal Products and Paediatrics Regulations that would hinder access to innovative treatments for children living with PKU or other rare diseases.
- ✓ The Commission intends to ensure 'that a children's rights perspective is mainstreamed in all relevant policies, legislation and funding programmes and to create a child-friendly culture in EU policy-making'. The Commission's Coordinator for the Rights of the Child will play an important part in this respect, along with a future EU Network for Children's Rights, which is to be established by the end of 2021
- ✓ ESPKU and the Cross-Party Alliance on PKU call on the Commission to take explicit account of the special needs, rights and requirements of children with chronic and rare conditions and ensure that their voice is represented in this Network. This is crucial if the Strategy is 'inclusive by design and in its implementation', as stated by the Commission.

Conclusions

The ESPKU and the European Parliament Cross-Party on PKU would like to thank the Commission once again for this initiative. We call on the Commission to take note of our suggestions, which are based on our Call to Action (<http://pku.at/unmet-needs>).

We would be happy to provide any more information and data required and work with the Commission to ensure the best possible life and future for **all** children in the EU.