



Report:

Welcome, introduction and rationale:

Sean Kelly MEP welcomed participants, underlining that International PKU Day provided the perfect context to discuss the Alliance's priorities, strategy, and agenda.

The Alliance is already supported by 17 MEPs (with another 13 MEPs who endorsed its Call to Action); this is a higher number than for many other health-related groups active in the Parliament and an indication of the importance of addressing a rare disease such as PKU.

Since its expansion, the Alliance has not yet had an opportunity to discuss its specific priorities, its activities, and potential output, hence the focus of this meeting. Now that the COVID-19 restrictions are gradually being lifted, it will be easier to organise face-to-face meetings, facilitating communication and exchange.

The COVID-19 pandemic has made clear that more health coordination and cooperation at EU level is needed; for example, the EU can support coordination around how to better manage rare conditions such as PKU, which require treatment and care throughout life.

Eric Lange (President, ESPKU) thanked Sean Kelly MEP for hosting the event and for his other recent efforts to make PKU visible at EU level. It is important for the PKU community to engage with the wider community, with the aim of making 'the invisible, visible'.

Since the Alliance's inception in March 2020 many activities have taken place. Three events were organised (one 'live' and two virtual meetings). MEPs sent several Written Questions to the European Commission, addressing access to neonatal screening, life-long treatment, and care, and the recently adopted EU Disability Strategy.

A comprehensive Call to Action was developed, outlining the unmet needs in PKU and the specific and concrete policy solutions that could and should be taken to address these.

A formal response to the Commission's consultation on the Green Paper on Ageing was prepared. A position paper on the revision of the orphan and pediatric medicine legislation as well as letter in response to the European Strategy on the Rights of the Child are being developed.

With an average of two ESPKU/Alliance tweets per week, social media visibility is also increasing. An article co-signed by 4 of the Alliance supporters addressing mental health and PKU was posted on the ESPKU website.

Internal Alliance business;

Bernadette Gilroy (Chairperson of the PKU Association of Ireland) warmly thanked all MEPs supporting the Alliance as well as those endorsing the Call to Action for their continued involvement. This support helps to raise awareness of the unmet needs in PKU and to champion policy action to help improve the lives of people living with PKU and their families.

With respect to the Alliance's chairmanship, Bernadette Gilroy informed the meeting of Billy Kelleher MEP's nomination; the meeting unanimously endorsed Billy Kelleher's appointment.

Bernadette Gilroy also invited other Alliance supporters to express their interest in the position of co-chair and noted that it would be useful to have a representative group of co-chairs as the Alliance grows (i.e., representing different countries and different political groups).

Turning to the Alliance's working methods, a proposal was circulated prior to the meeting. As no comments from Alliance supporters were received, this proposal was adopted.

Bernadette Gilroy then invited Billy Kelleher MEP to make a short statement.

Mr. Kelleher MEP thanked his colleagues for his appointment and stated that his previous experience working in health policy has provided significant insights into the daily challenges faced by people living with rare diseases. Access to treatment remains an issue as rare and genetic diseases are not always prioritised as an area for research or patient access; as a result, people living with these diseases risk losing out when it comes to accessing innovative treatment. While taking note of the reluctance of the Member States to let go of their competence in relation to health policy, he stated that the EU should look to further incentivise innovation in relation to rare and genetic diseases as there are key needs as well as opportunities in this area.

Furthermore, a novel approach to reimburse these innovative medicines should be considered, to provide equal access for all patients across the EU. Member States should be obliged to create capacity within their health budgets to earmark support for rare disease therapies and appropriately compensate companies that innovate and invest in this area.

When diagnosed early, people with PKU can live perfectly normal lives - but only if they have the possibility to manage their condition and have access to the best means to do so.

Setting Alliance priorities:

Tobias Hagedorn (Secretary General of ESPKU, DIG-PKU) presented an overview of issues and unmet needs that should be addressed, linking these to specific items on the current EU policy agenda.

He stated that the Cross-Party Alliance on PKU was set up to work with the PKU community to improve the policy framework to the benefit of those EU citizens impacted by PKU – both people living with PKU as well as their families and loved ones.

While there are limits to the extent to which the EU can shape healthcare provision at national level, the European Parliament does have a role in the development and oversight of EU legislation. It can provide important input into policies that have a bearing on better health care for PKU patients, such as those related to the revision of the orphan (OMP) and pediatric medicines legislation (), the EU Disability Strategy, and many others.

In relation to the ongoing consultation on the revision of the OMP legislation, Tobias Hagedorn noted that the European Commission's Inception Impact Assessment, which outlines possible policy changes, considers changing the prevalence criteria for rare diseases and shifting the focus to diseases deemed to have "unmet medical needs" – but without any concrete details.

He questioned whether this could mean that PKU would not be considered 'rare' in the future and whether this would mean that new treatments would no longer be incentivized. He called on the Alliance's support required to ensure that the OMP legislation will support all rare disease patients.

The first generation of early diagnosed patients are now approaching 65; and the lack of adequate provisions for the transition from adult to old age care is deeply concerning. Therefore, universal access to dietary and innovative care throughout life needs to be part of the implementation of the Green Paper on Ageing. In addition, even in countries with a positive history of early diagnosis, one third of patients are diagnosed late; screening systems are not as resilient as they should be. Late diagnosed patients are often forgotten, while they suffer the most. A European Strategy on Care, as championed by the EPP, is therefore welcome as the burden of care falls often on families.

Tobias Hagedorn concluded by thanking the MEPs on behalf of those citizens impacted by PKU for listening and working to close the gaps in PKU care.

Eric Lange then invited MEPs and MEP assistants present to provide their views on the following questions:

- How can the current policy agenda 'serve' to address the unmet needs in PKU?
- What could you personally do to advance this agenda (written questions, formal EP initiative, hearing, host events, help bring MEPs from the larger countries on board and national MPs on board...)?

Billy Kelleher MEP stated that, given the ongoing revision of the EU OMP legislation, the Alliance should prioritise ensuring that all patients living with PKU across Europe have timely access to innovative therapies and other technological advances. He noted that Member States are often reluctant to support EU policies in these areas due to local budget impacts; therefore, a key question is how to ensure that Member States guarantee local capacity to support the development and reimbursement of rare disease therapies in a manner that provides timely patient access.

He cautioned against rare disease therapies – including for PKU – being assessed based on cost rather than on their health outcomes. He also lamented the fact that patients – including PKU patients – in some countries often have to wait much longer than patients in other EU countries to access approved therapies. In Ireland, for example, PKU patients had to wait 10 years longer than other European patients for access to an approved therapy.

He underlined that the Parliament must be strong and proactive when it comes to developing a supportive legislative framework on this matter; he proposed creating a small internal working group of MEPs to actively contribute to the revision of the OMP legislation in connection to PKU and other rare diseases.

Sean Kelly MEP stated that awareness of and citizens' interest in health is currently higher than ever. The Horizon Europe Programme will have an emphasis on health research and there are opportunities where patients' views are required to ensure the right emphasis and focus. Ensuring appropriate lifelong care is one topic that could be addressed. More awareness and sharing of solutions and good practice is required.

The proposed 'EU Health Union' should also address rare diseases and work towards greater equality. Equal access is a good theme for the Alliance as it touches upon many of the unmet needs in PKU.

Deirdre Clune MEP expressed her support for all that had been said and underlined the current policy opportunities as useful 'hooks' to keep the spotlight on PKU. There are currently many ways to highlight the issues and COVID-19 is helping to create the right context for greater focus on health at the EU level.

Asked whether MEPs present would be willing to increase support for the Alliance from MEPs representing the non-represented countries, they agreed to explore possibilities to do so, making colleagues in their respective political groups aware of the Alliance and inviting them to support it.

Conclusions

Closing the meeting, Sean Kelly thanked ESPKU for its coordinating work and his colleagues for their support. He noted that the meeting resulted in a clear direction for the Alliance, and that it will continue to work to make sure that PKU gets the attention it merits in relevant policy discussions, including by involving additional colleagues from other countries.