ENABLING A LIFELONG APPROACH TO ACCESS TO CARE IN PHENYLKETONURIA (PKU)

IN CELEBRATION OF INTERNATIONAL PKU DAY 2020

ONLINE POLICY ROUNDTABLE

HOSTED BY MEPs FRANCES FITZGERALD & TOMAS ZDECHOVSKY

TUESDAY 30 JUNE 2020

EXECUTIVE SUMMARY

Meeting Objectives

- To raise awareness of the importance of transition and access to adult care, and of the urgent need to enact good practices in that field
- To review specific needs and gaps and to formulate recommendations for solutions
- To contribute to the development of a "Call to Action" on this topic, to be used for advocacy by/with MEPs and other policymakers, at the EU and national levels
- To forge closer links and cooperation between all participating organisations

Meeting Highlights

- PKU is not just a paediatric disease – it requires lifelong treatment, and inappropriate or no treatment will lead to poor and unacceptable outcomes, e.g. intellectual disability
- Adult patients are not "big children" – multiple morbidities, mental health, adherence to diet and treatment, and other issues can impact them differently and all dimensions of their lives should be better taken into account
- A smooth transition from child to adult care, as well as access to appropriate adult care services, are crucial to lifelong well-being
- There is a risk of losing people as they move from child to adult services – in some countries the proportion could be as high as 80% - and this should be avoided at all cost
- The first generation of PKU patients is growing older and health care systems need to plan for this
- Adult PKU patients have the right to expect the same standards of organisation and quality of care as children – where no clinics, policies and procedures for the care of adult PKU patients exist, they need to be developed
- Tailored transition pathways should be developed in each country jointly by patients and health care professionals based on existing guidelines or standards, but also factoring in national situations and resources
- Patients need access to the most suitable and effective form of treatment for them
- The EU Health Programme could support work on developing and exchanging views and experiences on mechanisms to support better transition of care

MEETING REPORT

WELCOME AND INTRODUCTIONS

ERIC LANGE

PRESIDENT, ESPKU (UNITED KINGDOM)

Eric Lange welcomed all participants and thanked the 17 Members of the European Parliament (MEPs) supporting the meeting, which was attended by nearly 50 participants from 18 European countries. He then reminded the audience that the meeting aimed to:

- raise awareness of the importance and of the urgent need to ensure good practice in relation to transition of care and access to adult care;
- formulate the specific needs/gaps and recommendations for solutions;
- contribute to the development of a Call to Action on this topic, which could be used for advocacy by/with MEPs and other policy makers (at EU and national level);
- forge closer links and cooperation between the participating organisations.

STATEMENTS BY PARTICIPATING MEPs

Frances Fitzgerald MEP (European People's Party, Ireland) also welcomed participants and underlined that this meeting was taking place at a highly opportune moment, i.e. within the context of International PKU Day. She noted that the topic chosen for this meeting is highly relevant, also from an Irish point of view – although it is by definition a rare disorder, phenylketonuria (PKU) has one of the highest prevalences in the world in Ireland. Clearly, a smooth transition of care from child to adult care as well as access to appropriate adult care services are crucial to lifelong well-being. Unfortunately, both are sadly lacking across the European Union, with large differences between and even within EU member states.

Ms Fitzgerald then informed the audience that she is currently working on the development of a European Carers Strategy, which will aim to better address the gaps in care and resources so clearly highlighted by the COVID-19 pandemic. This is a priority for her political group, and the need is widely recognised as urgent to invest to a greater extent in collecting, disseminating and sharing best practices and also setting up support and exchange networks in the area of long-term care.

Ms Fitzgerald further noted that about 80% of all long-term care today is being provided by informal carers – mainly women. It would be useful to highlight the specific aspects of care provision related to PKU and to explore what the EU can do in this particular area. This could also be a coherent way of obtaining funding.

Ms Fitzgerald expressed once again her unflinching support for the European Parliament Cross-Party Alliance on PKU, as well as her willingness to help take forward the major outcomes of the meeting – in particular a parliamentary Call to Action.

Tomáš Zdechovský MEP (European People's Party, Czech Republic) underlined that International PKU Day represents a useful opportunity to put the spotlight on PKU once again, as PKU is still not today a mainstream health policy issue. He mentioned his interest for situations when people are at an increased risk of vulnerability as they move between different parts of the health care system, and remarked that it is all too easy to lose sight of people when they move from child to adult services, especially as this happens at an age when many other transitions also take place – e.g. changing or leaving schools, entering the world of work, leaving home...

Mr Zdechovský expressed his view that **health care systems need to do a better job altogether to support better care transitions** and, in doing so, to keep a clear focus on the needs of patients and of their carers at all time.

Mr Zdechovský welcomed the strong involvement and support of all participants to this meeting and to the Cross-Party Alliance in general, and he renewed his offer to help with any follow-up actions to be decided during the meeting.

WHY FOCUS ON TRANSITION OF CARE AND ACCESS TO CARE IN ADULT PKU?

MALGORZATA HENEK
BOARD MEMBER, ESPKU (POLAND)

Eric Lange then announced a prerecorded video by **Malgorzata Henek (ESPKU).** Speaking as an adult PKU patient born and raised in Poland, she stated that in her country adult PKU patients continue to be treated in the same hospitals and by the same doctors and dietitians as children with PKU.

Ms Henek affirmed that **PKU does require lifelong treatment**. Inappropriate or no treatment will inevitably lead to **poor and unacceptable outcomes**, such as intellectual disability, mental retardation and poor executive functioning.

Once they reach a certain age, **all patients should be able to transition effectively from paediatric to adult care**. Treatment of children with PKU is generally very well organised and there is no reason why adult patients should not be able to expect similar levels of organisation and quality of care. Unfortunately, this is far from always being the case. **Many adult PKU patients find themselves lacking access to adult clinics**, a fact only compounded by the rising number of adult PKU patients year after year. Health care systems need to plan for this. Not providing access to adult clinics can have a negative effect on PKU adults and by extension social and economic implications for the country where they live. Successfully managed adult PKU patients can work, pay taxes, build families and live like any other person. **If care for adult PKU patients is not adequate, then governments must take action:** where no adult metabolic clinics, or no policies and procedures for the care of adult PKU patients exist, they need to be developed.

Ms Henek further commented that a narrow focus on PKU therapy and treatment options only does not suffice to address the actual needs of patients: all dimensions of the life of an adult living with PKU should be better taken into consideration. What are the difficulties they are facing, can they overcome them, what is the patient’s level of compliance to his/her treatment and how does the condition influence his/her mental health day-to-day? The doctor and other members of the medical team should also have a broader knowledge of any other diseases and disorders that are common in non-PKU patients of a similar age and sex group, as these co-morbidities may in turn influence the management of PKU therapy.

The transition process should be organised and conducted very carefully, jointly managed and agreed between patients, paediatricians, adult care physicians and dietitians. Other health professionals may be required as well, e.g. psychologists or social workers. The purpose of this process is to ensure the best possible care and not to lose sight of the patient. **The risk of a patient being lost during transition is high; this should be avoided at all cost.**

TRANSITION OF CARE: ENSURING APPROPRIATE CARE IN ADULT PKU

A PANEL DEBATE

DR. JAMES O'BYRNE AND PROF. EILEEN TREACY
MATER HOSPITAL, DUBLIN (IRELAND)
DR. JOHANNES KRÄMER
CENTER FOR RARE DISEASES, UNIVERSITY HOSPITAL OF ULM (GERMANY)
PROF. DR. MARTIN MERKEL
MEDICAL DIRECTOR, ENDOKRINOLOGIKUM HAMBURG (GERMANY)

Eric Lange then gave the floor to the first panelist, **Prof. Eileen Treacy** (Mater Misericordiae University Hospital, Dublin, Ireland), who briefly introduced PKU as **a complex and challenging lifelong rare condition with great impact on those affected and their families**.

The incidence of PKU is high in Ireland – 1 : 4,500 births as opposed to, for instance, only 1 : 10,000 in the neighbouring United Kingdom. Currently, there are approximately 600 individuals with PKU actively attending specialised services in Ireland. Classical PKU is the most common form owing to the relative high prevalence of the *R408W* mutation.

A newborn screening programme has been in place in Ireland since 1966, as the first European country to introduce this screening policy. Since then, 3.5 million infants have been screened for PKU. **The newborn screening programme for PKU is a success story in Ireland**, which has driven major cost-efficiencies for the state by helping prevent mental retardation and associated costs. **Ireland has also actively promoted a 'Treatment for Life' policy**, which has been in place since the programme's inception.

Until 2014, all PKU care was provided to children, adolescents and adults at one centre only, the National Centre for Inherited Metabolic Disorders at the Temple Street Children's Hospital. It is only as of 2014 that another adult hospital-based service started at the Mater Hospital in Dublin. There are now well over 360 adult patients attending this service.

While the treatment of PKU should be seen as a good example for other treatable rare genetic diseases, **there are still significant unmet needs for adult care**, to include for community care for untreated/late treated patients.

Prof. Treacy emphasised that PKU is not just a paediatric disease – it is lifelong and affects adults too. There are a number of existent issues which need to be taken into account in relation to adult PKU patients, such as **the presence of multiple morbidities, mental health issues, adherence to diet and treatment, as well as the challenges of phenylalanine (Phe) control during pregnancies and managing the potential risks to the unborn children.**

The need for multidisciplinary care and for sustainable funding of adult services matched with paediatric care pathways is clear. The need to have infrastructures in place such as registries and agreed outcome data sets as well as patient-centred outcome measures to generate the evidence to support the reimbursement of new therapies was also mentioned.

Looking at EU-level solution, Prof. Treacy suggested to look at **a better implementation of the European PKU treatment guidelines across countries, and at the development of consensus care pathways for implementation and outcome monitoring** including a transition pathway – something for which a European Reference Network such as MetabERN[1] will be essential.

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[1] https://metab.ern-net.eu/

The second panelist, **Dr. Johannes Krämer** (Children's Hospital, University of Ulm, Germany), stated that he is currently treating both children and adults in the metabolic centre of his hospital, even if it is clear that paediatricians are not the practitioners best suited to treat the more complicated needs of adult patients.

Dr. Krämer noted that it is often complicated to know where to send adult patients for their care and treatment. In fact, he found that, even as he tried to develop a structured transition pathway to other physicians, nearly all patients came back to his clinic asking for continued treatment there. Most adult patients did not find elsewhere the multi-disciplinary teams of dietitians, social workers, psychologists, etc.… which they could access at the Ulm Children's Hospital.

Recent studies show that some 20% of adult patients are lost when making the transition from child to adult services and this is worrying. Dr. Krämer commented that it means that it is better to keep the patients in the children's clinics in order not to lose them and expose them to a situation of no or inappropriate care.

He agreed that the lack of access to adult services and to a more structured transition is a clear and significant unmet need which should be addressed as a matter of urgency. In that respect, it would be helpful if the standards of medical education would focus more on PKU and its management, as at the moment there is a profound lack of knowledge of the condition amongst clinicians generally. Dr. Krämer also manifested his willingness to exchange information on how transition is structured and available in other countries.

The third panelist, **Prof. Dr. Martin Merkel** (Endokrinologikum Hamburg, Germany) started by expressing his agreement with the previous speakers on the need to put in place structured transition pathways from children to adult care as well as access to adult treatment services.

However, he underlined that in his opinion, even more patients are lost than the 20% referred to by Dr. Krämer – in his own view, the figures could be as high as 60% to even 80%.

This is totally undesirable and to demonstrate this, Prof. Dr. Merkel provided the example of one of his patients, who had been diagnosed with PKU as a child and received good care and treatment until she was 17 years old. However, she did not experience any form of transition to adult care. Hence, at the age of 51, she started suffering from a severe neurological decline. Her condition was not recognized, so she did not receive the appropriate treatment and as a result she became highly dependent, hardly mobile and afflicted with speech difficulties. After only 6 months of intensive dietary treatment, complemented with vitamins and amino-acid supplements, the patient experienced a remarkable health improvement: she was able to leave her wheelchair, to drive to her own care and even to apply for a job. In other words, this person had retrieved a new – and normal – life.

Prof. Dr. Merkel stated that in Germany about 200 babies are diagnosed with PKU and Hyperphenylalaninemia (HPA) every year. Most of these individuals will be lost for follow-up when they move into adult age. There needs to be a clear understanding that adults are not "big children", and that taking care of adult PKU patients in paediatric facilities is only the second-best choice. Knowledge about rare metabolic diseases needs to become a greater part of the adult medicine curriculum. Today, the provision of adult services in well-structured and organised metabolic centers simply does not exist in Germany – this needs to change.

Prof. Dr. Merkel concurred that EU-level actions could help improve this situation, for instance by supporting data gathering through the organisation of Europe-wide clinical investigations on how to improve the quality of life of people with rare disorders, including PKU. Driving consensus towards the establishment of valid and widely accepted medical goals for adult treatment could also be helpful.

THE PERSPECTIVE OF A PATIENT

LOIC LALIN

PATIENT ADVOCATE, LES FEUX FOLLETS (FRANCE)

Eric Lange then introduced a pre-recorded testimonial by **Loïc Lalin** (Association Les Feux Follets, France). The situation presented by Mr Lalin was full of contrasts. On the one hand, French PKU patients are probably some of the most protected in the entire European Union as they enjoy full reimbursement for amino-acid mixtures, low-protein food and other medical procedures related to PKU. The health care system for patients living with rare disorders is organised by a Rare Disorder Network commissioned by the Ministry of Health, which categorises hospitals as reference centers or centers of expertise. As far as PKU is concerned, there are reference centers all across the country, which work hard to apply and disseminate the latest French PKU protocols for diagnosis and care, which were updated only two years ago to ensure good alignment with European guidelines.

However, even in such a privileged situation, there are still problems when it comes to the transition from paediatric to adult care.

First of all, **medical teams with “old school" practitioners are sometimes more reluctant to embrace the benefits of lifelong treatment**, or to be proactive in giving their patients greater leeway in managing and piloting their own treatment. A lack of funding or structures to receive adult PKU patients in dedicated services instead of paediatric services also plays a role.

A second issue relates to those adult patients who are lost to follow-up. In the past, when there was only dietary treatment, PKU families and patients were told they could safely stop treatment as of 8 years of age. That threshold was then successively pushed to 12 and then 18 years, and today the new consensus is that treatment should be continued through life. **Many older patients who would need to resume their treatment have unfortunately been lost in the meantime** (no up-to-date contact details, etc).

A third issue relates to **the difficulty of the French system to adjust itself to meet the new and burning needs of adults with PKU**. Many adults stop following their diet therapy for various reasons, all of which are well-known e.g. the difficulty of the diet therapy, the unavailability of these products, the stigma, etc. These patients need either innovative food products (called FSMP) or other forms of treatment, e.g. pharmacological therapies. **They urgently need to be able to have access to them**, and at the moment in France the situation is not optimal from that point of view.

Mr Lalin’s organisation, “Les Feux Follets”, works closely with hospitals and institutions to enhance the care and quality of life of adult PKU patients. It carries out awareness campaigns and therapeutic education to develop partnerships with doctors who may be hesitant to apply new protocols. The organisation could certainly do with help to continue advocating for better transition to adult services.

Mr Lalin concluded by noting that International PKU Day is a good time to celebrate the positive achievements relating to PKU care – but also to highlight the gaps that are still being faced in France like in the rest of Europe.

AUDIENCE DISCUSSION BASED ON PRE-SHARED QUESTIONS

The audience debate, moderated by **Eric Lange**, focused on one central question: **how can good transition of care be ensured to improve adult access to care and treatment in PKU?**

Dr. James O’Byrne (Mater Misericordiae University Hospital, Dublin, Ireland) shared a number of points that would help to enable a life-long approach to access to care in PKU:

- Tailored transition pathways should be developed in each country based on existing guidelines/standards but also factoring in national situations and resources.
- As these transition pathways develop and evolve, oversight committees should be more pro-active in incorporating new developments and novel innovative treatments. Currently, it seems like the existing systems are catching up with developments rather than being prepared for these.
- In terms of setting medical goals, a standard outcome registry should be developed and put in place to record generated evidence on treatment and care. All patients of all ages should be included in it, and a European network like MetabERN could play a role in rolling out and harmonising such registries across countries.
- With the genomics revolution upon us, and at a time when countries are increasingly developing their genomic strategies and integrate them into healthcare systems, PKU as a treatable genetic disorder should be part and parcel of these conversations and plans, and so should be the need for patients to have access to up-to-date treatment.
- Finally, better access for practitioners to education and information in the area of rare diseases – incl. PKU – is also a must. This could involve greater exposure to PKU in academic settings (e.g. medical schools) or the development of information centers where appropriate information could be accessed in a user-friendly way by patients, clinicians and the general public alike. In Ireland, one such center exists, but it requires further development.

Vinciane Quoidbach (European Brain Council, Belgium) updated all participants on the progress of the European Brain Council's "Value of Treatment" study, one research project of which is dedicated to PKU. That project looks at service use and patient outcomes, and focuses on care pathways and indicators related to access to personal care. The study will be finalised nearer to the end of this year.

Kirsten Ahring (ESPKU, Denmark) indicated that work is ongoing to update existing guidelines on PKU treatment and to touch specifically on the issue of transition of care. She underlined that adult patients clearly do not belong in children's' hospitals. However, while some adults may object to being treated in a children's clinic, others actually do not mind as it is familiar to them and also as the physicians and other health care professionals at hand there know the patient very well.

Prof. Julio Rocha (NOVA Medical School, Lisbon, Portugal) remarked that transition is a very important aspect of proper access to lifelong care, and referred to a paper currently being developed at one of the treatment centers for PKU in Portugal, and which is almost ready for publication. In Portugal, the proportion of adult PKU patients "lost to follow-up" is lower than in Germany, and in the region of 6% only.

Prof. Rocha further commented that one of the key aspects of a successful transition is *continuity*: while a treating physician may change, it is important to keep at least the same nutritionist and psychologist involved. Even within the structure of a multi-disciplinary team, keeping some of the familiar faces in touch with the patient is key.

Tobias Hagedorn (DIG-PKU, Germany) opened his comments by acknowledging the important support offered by the MEPs, both those present at the meeting and those not able to attend but still associated to the work of the Cross-Party Alliance.

Mr Hagedorn underlined that screening was introduced some 50 years ago and that, in many countries, paediatric care is well to very well organised. However, such continues not to be the case for adults. This unmet need is becoming more pressing as, the more time passes, the more adult PKU patients we will see in our societies. The current situation where patients end lost to follow up is not acceptable. Also, the advice given to patients can be conflicting as many are told they can come off diet in their teens – which is not quite correct. There needs to be more research into the condition as the effect of the strict diet on the ageing body is not clear either.

Mr Hagedorn urged national health systems to invest in staff training and education in relation to adult PKU and to ensure access to care and treatment irrespective of a patient's age, and he called on European institutions to play as much of a role as possible in supporting those changes.

Dr. Greg Pastores (Mater Misericordiae University Hospital, Dublin, Ireland) remarked that the issues addressed in the meeting apply to many other rare disorders as well, and suggested that there might be merit in joining forces with representatives from other such conditions to drive change and get the proper services put in place.

Another issue relates to the multi-morbidity of PKU patients as they get older. Adult patients will require access to multidisciplinary teams. Comprehensive care is key. Dr. Pastores finally referred to the very welcome development of a medical curriculum on adult metabolic medicine in Ireland.

Niko Costantino (Cometa ASMME, Italy) outlined the current issues faced in Italy with respect to brain disorders, especially in COVID-19 times. Rare diseases are much less of a priority, which has been frustrating. He informed the audience of a specialist curriculum for rare metabolic diseases developed by the University of Padova – a very positive step.

While **Johan Prévôt's** organisation (IPOPI, the International Patient Organisation for Primary Immunodeficiencies) addresses a different audience, many commonalities were found, e.g. the crucial importance of transition pathways. The French model of La Suite-Necker[2] was mentioned as an example of a sound, structured and stakeholder-driven transition model and, based on this like other examples, advocating for the creation of specific centers for paediatric and adult care could also be a good model for the PKU community to pursue. Another common issue is newborn screening. IPOPI has recently launched a platform called "Screen4Rare" as part of the European Health Policy Forum. Mr Prévôt mentioned that ESPKU would be most welcome to join this platform. Lastly, he also invited ESPKU's German members to join an event due to take place in November at the German Parliament, organised by IPOPI, and which will focus on newborn screening and transition.

Mary Ellen O'Shea (Institute of Technology, Tralee, Ireland) briefly introduced a research project in which she is involved, which aims to elicit the experience of adults living in Ireland with an early diagnosis of PKU on long-term dietary therapy. Ireland was one of the first countries to introduce neonatal screening in 1966 and dietary therapy shortly afterwards. This research represents the "lived experience" of some of the oldest adults with PKU in the world without any apparent cognitive or developmental delay. Participants identified the needs of an adult with PKU to be different from those of a child, particularly with regard to the provision of healthcare support.

This research shows that transition is important, but also that it can be challenging for a lot of people. Many individuals feel at a loss and don't know where to turn for help while waiting to be transitioned to adult services. It is also unclear what the future holds for PKU patients and the impact of the condition on the ageing body. Specialised teams and psychological support can truly benefit patients.

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[2] http://www.la-suite-necker.aphp.fr/

Laura Petreus (Asociația Aproteica a Bolnavilor de Fenilcetonurie, Romania) underlined the need for European collaboration between the PKU community and other representative organisations. The EU Health Programme could support work on developing and exchanging views and experiences on mechanisms to support better transition of care. The European Council and Parliament should be involved and mobilised with a common Manifesto, which could be elaborated and advocated with the active support of ESPKU in all member countries.

CONCLUDING REMARKS AND NEXT STEPS

ERIC LANGE AND PARTICIPATING MEPs

Alex Agius Saliba MEP (Socialists & Democrats, Malta) thanked ESPKU for taking the initiative to coordinate the European Parliament Cross-Party Alliance on PKU and for organising the meeting. He underlined the importance for MEPs to be involved with important issues such as these and to work in direct contact with stakeholders, getting their direct input and feedback on the issues that matter to them. He expressed his view that this type of meeting is a great way to raise awareness of the issues faced by a community, and doing so in the context of International PKU Day was most useful.

Mr Saliba summarised the various interventions and comments, which all demonstrated the serious impact that PKU has on the lives of those affected as well as of their families – most specifically because of the effect on brain development – but also the need for seamless, appropriate treatment provided at the right time. Regretfully, this is by no means the case across the EU today.

In turn, Mr Saliba reaffirmed that mental health is and will be one of his main priorities for this current legislative term – and that he had no doubt after today that the impact but also the relevance of PKU for mental health is considerable.

A Call to Action, developed with input from expert stakeholders and highlighting the steps and principles that should be in place to guarantee this, could go a long way to help those living with PKU in a practical sense. It would be a useful advocacy tool for all organisations present at the meeting, but would also find applications outside the European Parliament, and away from Brussels. It would also have the potential to set a precedent and serve as a beacon for other conditions as well.

Mr Saliba closed his intervention by expressing his outright support for any follow-up work, underlining again the need for stakeholders to work hand-in-hand with policymakers.

Sean Kelly MEP (European People's Party, Ireland) also thanked ESPKU for working collaboratively with the European Parliament. Awareness of conditions such as PKU is important to get policymakers moving and Mr Kelly found it to be fully in the purview of organisations such as ESPKU to ensure that this topic does not get lost in the midst of all the other emergencies of the present time (e.g. COVID-19). Speaking from his long experience as a policymaker, he recommended that, to really change and improve situations, *influence, pressure and profile* are all required – and this is exactly what meetings such as this are about.

Mr Kelly underlined again all previous remarks about the need to have systems better cater for access to PKU treatment and care at all ages. Ideally, transitional care should be based on a comprehensive care plan, co-created by clinicians, patients, and carers, taking account of the individual circumstances of the patient. This would include logistical arrangements, education of the patient and family, and coordination among all health professionals involved in the transition. He addressed a word of encouragement to all participants, noting that it does matter to policymakers and to the health care system alike to ensure that nobody is left behind, irrespective of their condition, their age, or any other personal aspect.

On this, Mr Kelly referred to the United Nations Sustainable Development Goals, which prioritise healthy lives and promote well-being for all. He commented that it is not just about the well-being of the one person living with a condition: **systematically identifying patients who are most likely to be at risk of a poor transition of care enables better and more efficient use of scarce health care system resources as well.**

As a closing comment, Mr Kelly underlined the important of addressing **insurance** as part of a comprehensive care plan. Like all his colleagues before him, he expressed his interest in the Call to Action to be developed as a result from this meeting, his willingness to be involved in any follow-up and his steadfast engagement onboard the Cross-Party Alliance.

Eric Lange thanked speakers, participants as well as supporting MEPs and closed the meeting by indicating that **the Cross-Party Alliance will meet again later in 2020**, at a date to be announced in due course.

Annex

Registered Participants

First Name	Surname	Country	Organisation
Alex	**Agius Saliba**	Malta	MEP and Member of the Cross-Party Alliance on PKU
Kirsten	**Ahring**	Denmark	Kennedy Institute, Glostrup
Barry	**Andrews**	Ireland	MEP and Member of the Cross-Party Alliance on PKU
Deniz	**Atakay**	Turkey	PKU Family Association of Turkey
Agata	**Bak**	Spain, Poland	Federación Española de Enfermedades Metabólicas Hereditarias (FEEMH)
Marc	**Boënne**	Belgium	Belgische Org. voor Kinderen en volwassenen met Stofwisselingsziekten (BOKS)
James	**Bonham**	United Kingdom	International Society for Neonatal Screening (ISNS)
Sara	**Cerdas**	Portugal	MEP
Anastasia	**Cojocaru**	Belgium	Global Alliance of Mental Illness Advocacy Networks (GAMIAN-Europe)
Niko	**Costantino**	Italy	Cometa ASMME
Rachel	**Finnegan**	Belgium	FIPRA
Frances	**Fitzgerald**	Ireland	MEP and Member of the Cross-Party Alliance on PKU
Mari	**Fresu**	Belgium	European Psychiatric Association (EPA)
Bernadette	**Gilroy**	Ireland	PKU Association of Ireland
Maria	**Gizewska**	Poland	Pomeranian Medical University, Szczecin
Hülya	**Gökmen Özel**	Turkey	Hacettepe University, Ankara
Judy	**Gore**	Greece	European Federation of the Associations of Dietitians (EFAD)
Lara	**Grosso Sategno**	Belgium	European Psychiatric Association (EPA)
Tobias	**Hagedorn**	Germany	Deutsche Interessengemeinschaft Phenylketonurie (DIG PKU)
Małgorzata	**Henek**	Poland	ESPKU (VIA VIDEO TESTIMONIAL)
Alvaro	**Hermida Ameijeiras**	Spain	Congenital Metabolic Diseases Unit, Horpital Clinico Universitario de Santiago
Christina	**Katsagoni**	Greece	Eur. Federation of the Associations of Dietitians (EFAD) + Agia Sofia Children's Hospital, Athens
Sean	**Kelly**	Ireland	MEP and Member of the Cross-Party Alliance on PKU
Péter Béla	**Kovacs**	Hungary	Hungarian PKU Association
Johannes	**Krämer**	Germany	Center for Rare Diseases, University Hospital of Ulm
Loïc	**Lalin**	France	Les Feux Follets **(VIA VIDEO TESTIMONIAL)**
Eric	**Lange**	United Kingdom	ESPKU
Anita	**MacDonald**	United Kingdom	Birmingham Children's Hospital
François	**Maillot**	France	Universite de Tours
Christine	**Marking**	Belgium	Marking Public Affairs
Martin	**Merkel**	Germany	Endokrinologikum, Hamburg
Birgit	**Mueller**	Germany	Deutsche Interessengemeinschaft Phenylketonurie (DIG PKU)
James	**O'Byrne**	Ireland	Mater Misericordiae Hospital, Dublin
Nigel	**Olisa**	Belgium	Global Alliance of Mental Illness Advocacy Networks (GAMIAN-Europe)
Emer	**O'Reilly**	Ireland	PKU Association of Ireland
Mary Ellen	**O'Shea**	Ireland	Institute of Technology, Tralee
Greg	**Pastores**	Ireland	Mater Misericordiae Hospital, Dublin
Florentina	**Perić**	Croatia	Croatian PKU Association
Sanja	**Perić**	Croatia	Croatian PKU Association
Laura	**Petreuş**	Romania	Asociatia Aproteica a bolnavilor de Fenilcetonurie
Johan	**Prévôt**	United Kingdom, Portugal	International Patient Organisation for Primary Immunodeficiencies (IPOPI)
Vinciane	**Quoidbach**	Belgium	European Brain Council
Júlio César	**Rocha**	Portugal	NOVA Medical School (Faculdade de Ciências Médicas), Lisbon
Yvonne	**Rogers**	Ireland	National Centre for Inherited Metabolic Disorders, Temple Street, Dublin
Jean-Louis	**Roux**	United Kingdom	BioMarin
Allison	**Sheerin**	Ireland	Mater Misericordiae Hospital
Serap	**Sivri**	Turkey	Hacettepe University, Ankara
Celine	**Stenson**	Ireland	Children's University Hospital, Temple Street, Dublin
Crina Mihaela	**Taloş**	Romania	Asociatia Aproteica a bolnavilor de Fenilcetonurie
Eileen	**Treacy**	Ireland	Mater Misericordiae Hospital, Dublin
Esma	**Uygur**	Turkey	Cerrahpaşa Faculty of Medicine, Istanbul University
Jaap	**Van der Zijp**	Netherlands	Permanent Representation of the Netherlands to the European Union
Olivia	**Walsh**	Ireland	Children's University Hospital, Temple Street, Dublin
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