

Summary of the ESPKU Members' Representatives "Spring Meeting" on April 6th, 2019 at the Swissotel in Izmir



Attendance:

Laura & Ion Petreus (Romania), Lal & Deniz Atakay, Ege Erucar (Turkey), Michael Bechstein (Germany), Stian Skarelvén (Norway), Loic Lalin (France), Laura Momme (Denmark), Florentina Peric (Croatia), Eric Lange, Tobias S. Hagedorn, Gosia Henek (ESPKU Executive Board), Kirsten Ahring (ESPKU SAC Board)

Top 1: Welcome and overview on the agenda

ESPKU President Eric Lange opened the meeting, presented the agenda and asked all participants for a brief introduction.

Top 2: ESPKU Commitments 2019

ESPKU Secretary introduced the topics that have been identified by the Executive Board as their six priorities:

- The EU All Party Parliamentary Interest Group
- The ESPKU Manifesto
- The ESPKU White Paper on Transition
- The ESPKU Conference Organisation Help-Book
- The ESPKU Guidelines Cookbook
- Sheila Jones Award 2019

The first Sheila Jones Award Winner Laura Petreus (Romania) explained that the Sheila Jones Award has an impact on health policies in Romania and helped her to open some doors. Tobias Hagedorn supported this statement as a good example of how the ESPKU can support its members in their own political activities. He said that the ESPKU is not a service provider that solves local problems. However, ESPKU can very effectively support pro-active patient advocacy groups to identify and close gaps in PKU care.

Tobias Hagedorn presented the draft ESPKU White Paper on Transition and Adult Care that was adopted by the attending delegates and will now be lay-outed and published at the ESPKU website.

Top 3: The political environment and its impact on PKU

It is remarkable that the tolerances of nutritional information on food labels are very large compared to the required accuracy of the composition of technical products. Standards for food labelling differ between EU- and non-EU-countries.

The EU Commission Regulation 2016/128 on specific composition and information requirements on food for special medical purposes came into effect on February 22nd, 2019 should have no negative impact on the accessibility of amino acid mixtures and low-protein special foods and their reimbursement by national social and health systems. However, due to different interpretation problems cannot be excluded when the EU legislation is transposed into national rules.

Additional to insufficient food labelling rules, sugar tax is an issue for PKU, since sugar may be replaced by Aspartame in many food products or beverages that are not considered “light” or “diet” products.

The ESPKU does not expect any serious impact of Brexit on the availability of amino acid mixtures and other FSMPs. In the worst case, there may be short-term bottlenecks when products need to be exported or imported to or from England. The local subsidiaries of the manufacturers should be prepared for this.

Top 4: The Value of Treatment

During the Delegates’ Programme at the 2018 ESPKU Annual Conference, Edmund Jessop (Medical Advisor, NHS England) gave a brief overview on challenges and opportunities of Health Technology Assessments. Eurordis’ call on the EU Parliament to allow patients voices to be heard in HTAs was rejected with reference to a possible influence of pharmaceutical companies on affected patient representatives.

Eric Lange introduced a research Project of the European Brain Council. “The Value of Treatment” This will focus upon Phenylketonuria and evaluate coordinated care for PKU. Clinical specialists and patients’ association representatives will identify and analyse treatment gaps and unmet needs, and estimate the socioeconomic impact and health gains from best practice healthcare interventions in comparison with current care. The study will explore the potential benefits (consequences) of coordinated care examining health services, patient outcomes and patterns of multidisciplinary care.

With reference to emerging therapies and new product developments, Tobias Hagedorn emphasized that patient representatives will likely be confronted with HTA’s in the future, and that it will be most

important to understand their methodology to defend challenges and to take advantage on opportunities.

Top 5: ESPKU Consensus Paper: Time to revisit?

The ESPKU Consensus Paper “Requirements for a minimum standard of care for Phenylketonuria: The Patients Perspective” was published in the Orphanet Journal of Rare Diseases in 2013. With this publication, the ESPKU called for comprehensive guidelines on internationally harmonized PKU care. Since then, the European Guidelines on diagnosis and management of PKU have been published. More than that, with emerging therapies and new product developments, the PKU environment is constantly changing.

As a consequence, it was agreed that the ESPKU Consensus Paper should be updated and answer (1) whether the patients’ requirements are covered by the European Guidelines and whether these are being implemented into daily practice across Europe, and (2) whether there are new aspects that should be included into a minimum standard of PKU care.

Tobias Hagedorn, Stian Skarelvén and Loic Lalin have offered their cooperation in the project, further support from other representatives of ESPKU members is welcome. A first workshop will take place during the 2019 ESPKU Annual Conference and focus on objectives (what do we want to achieve) and procedures and working format (what strategy will the working group use to achieve results).

Top 6: The ESPKU Manifesto

Eric Lange outlined the idea of an ESPKU manifesto as a public statement of primarily political goals and intentions in a concise and understandable manner, created to attract the attention and support of political decision makers. This manifesto is essential to successfully establishing an EU All Party Parliamentary Interest Group that identifies with our goals and intentions and politically advocates at EU level.

In a brainstorming session, the attending delegates collected some headlines for the manifesto, including

- Transition and adult care
- Identification and care for undiagnosed and late diagnosed patients
- Low Protein Food Logo
- Prevention is better than cure!
- Constant training of specialists

It was also discussed whether ESPKU should offer PKU as an example for rare diseases or better focus on the number of carriers to gain political attraction (“are you one of the 2 %? Roll the dice!”)

During a coffee break, questions have been collected that could be asked at the EU parliament. Most proposed questions focussed on neonatal screening, PKU treatment and political awareness.

Top 7: The ESPKU Research Priority List

In a thoroughly philosophical discussion, the participants identified a list of questions for research that they believe should be prioritised, including treatment simplification, economic and social implications, treatment side effects and quality of life issues. Mental health issues were recognised

to be most important for patients of all ages, siblings, parents, grandparents, carers, professionals etc.

There was broad consensus that databases should not be owned by industries, but should be created by health professionals and patients associations as joint effort. Access to databases should be granted to everybody based on transparency and a clear purpose and with respect to data protection regulations.

Top 8: The 2019 ESPKU Annual Conference

The Executive Board briefly introduced some cornerstones of the draft conference programme, focussing on the programme for delegates. With regard to the limited hotel capacities, it is likely that the conference will be sold out quickly. Therefore the Executive Board advised the attending delegates to book their rooms in time.

Top 9: Any other business

During the discussions on the previous topics, there were many contributions and focussed discussions on different aspects of PKU, e.g.

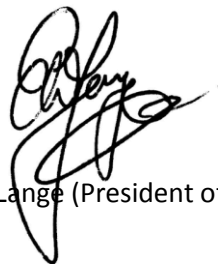
- Can PKU patients donate blood, organs, eggs or semen?
- Do PKU patients need an emergency card with digital health data?
- Being off diet does not mean being off treatment!
- etc.

When there were no further contributions from the participating delegates, Eric Lange and Tobias Hagedorn closed the meeting at 17:00. Compared to previous spring meetings, this year the attendance was relatively small. However, the quality of the contributions was remarkably high. There is a promising young generation of PKU patients coming up who are increasingly taking the lead in national and international patients advocacy work for PKU.

Recorded on April 21st, 2019



Tobias S. Hagedorn (Secretary of ESPKU)



Eric Lange (President of ESPKU)