



European Society for Phenylketonuria
and Allied Disorders treated as Phenylketonuria
(ESPKU)

Annual Report 2012

Advocacy for a common goal



Annual Report 2012

**The secret of success in life
is for a man to be ready for his opportunity when it comes.**

Benjamin Disraeli (1804-81)
British Prime Minister 1868 / 1874-80

Words of gratitude:

Thanks to all who supported the work of ESPKU this year and are not mentioned in this report.



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1. Meetings and Conferences

ESPKU General Meeting 2011

October 14th, 2011, Warsaw (Poland)

Regular board elections have been on the agenda of the 2011 General Meeting in Warsaw (Poland). President David Abeln decided not to go for another period in office. The ESPKU member delegates followed the advice of 2011 Annual Report to extend the Executive Board by two assessors. The new Executive Board is composed of:

President:	Eric Lange, United Kingdom
Vice-President:	Dinah Lier, Switzerland
Treasurer:	Maarten Criem, Belgium
Secretary:	Tobias S. Hagedorn, Germany
Assessors:	Thomas Møller Nielsen, Denmark Gregor Hammerschmidt, Austria

Executive Board Meetings

Since election, the Executive Board came together in two meetings and one unofficial meeting to coordinate the daily work. The meetings were held in Warsaw (Poland), Brussels (Belgium) and Gent (Belgium). During the year, details have been discussed and appointed via E-Mail and phone calls.

Delegates Meeting 2012

May 19th, 2012, Gent (Belgium)

The delegates of 7 ESPKU member associations and the Executive Board of ESPKU met to discuss the political activities of ESPKU and the actual political climate in Europe with regard to healthcare policies and rare diseases.

After the reports of the attending delegates of ESPKU members about the situation in their countries, ESPKU is alerted: Instead of fighting for improvements, National PKU associations find themselves more and more in a defensive position, aiming to keep at least the status quo of social support for patients and families. ESPKU members fear a worsening in PKU care due to socio economic change.

A new supportive project was appointed: An overview of treatment costs shall be researched in the ESPKU member states and published on the ESPKU website.

Annual Conference 2012

October 18th-21st, 2012, Liverpool (United Kingdom)

The ESPKU Annual Conference 2012 was hosted by ESPKU member association NSPKU, United Kingdom, and took place in the Adelphi Hotel in Liverpool from October 18th until October 21st, 2012. The local organizing committee expected an attendance of about 400 patients, relatives, professionals and delegates of ESPKU member associations.

In accordance to the ESPKU political activities, the key programme was about international guidelines for the management of PKU (professionals programme) and developing local advocacy programmes (programme for ESPKU members delegates). The patients/families programme was headlined "PKU's got talent" and demonstrated that PKU's with good access to good treatment can live their lives to the full. On the final day, there was a common programme for all attendees with presentations and lectures.

Children's entertainment, creche, kids-outings as well as cultural trips and a conference dinner were included in the social programme of the conference. An industrial exhibition gave the opportunity to inform about latest product and service developments of international low-protein-food distributors and amino-acid-mixture-producers or dedicated pharmaceutical companies.

2. Political work



ESPKU Poster "Closing the Gaps in Care"

February 2012

Beginning of 2012, ESPKU presented a poster on its long-term and multi-level programme called "Closing the Gaps in Care".

Scientific research shows that there are major inconsistencies in treatment and access to treatment across Europe. Due to differences in social impact of PKU, treatment and care, as well as PKU health policy agendas, the quality and success of treatment are matters of the patient's birthplace. These were results of an ESPKU online questionnaire in 2009 and an ESPKU benchmark report published in 2011/2012.

The poster presents the structure of a multi-level programme of ESPKU and its members, which aims to minimize these inconsistencies:

Phase 1: In 2009, 214 patients and their families answered an online questionnaire. Their answers have been compared with the results of a

parallel survey among 28 leading health care professionals. One key result was that care is not at the same level in all European countries.

Phase 2: In 2011, ESPKU commissioned qualitative research in five European countries to provide insight from patients, health care professionals and policy makers. The survey sought insight to social impact of PKU, treatment and care and health policy agendas. In conclusion to this report, ESPKU presented several recommendations and current initiatives.

Phase 3: With these findings, ESPKU approached the European Community and raised awareness on the disease and the needs of patients among members of the European parliament. Parallel, ESPKU member associations started lobbying initiatives on national level, supported with a toolkit.

Phase 4: Parallel to the political lobbying, ESPKU members agreed in a consensus paper on optimal PKU treatment from patient's point of view.

Phase 5: ESPKU and its Scientific Advisory Board invited leading scientists to set up and implement international treatment guidelines.

European Parliament Luch Debate*

February 29th, 2012, Brussels (Belgium)

E.S.PKU Urges Policy Makers to Close Significant Gaps in Care for Patients with Phenylketonuria (PKU) Across Europe at the European Parliament



Eric Lange, Maarten Criem, Dr. med. Maria Gizewska, Tobias S. Hagedorn, David Abeln at the EU Parliament

On World Rare Disease Day, the European Society for Phenylketonuria and Allied Disorders (ESPKU) urged policy makers to bridge significant gaps in Phenylketonuria care across Europe during a lunch debate at the European Parliament. The ESPKU presented a set of recommendations based on the first PKU benchmark report "Closing the Gaps in Care", launched on this occasion. The event was hosted by Members of the European Parliament (MEP) Esther de Lange and Antonyia Parvanova.

In front of more than 50 attending members of the European Parliament, Scientists and representatives of other advocating associations, Esther de Lange, MEP, said: "Unfortunately, for many diseases, as for PKU, vast differences exist between European Union countries. Therefore the EU Council Recommendation on the implementation of rare disease plans is a welcome signal. I invite national governments to include PKU as an example in these plans, including strong guidance on high standards of screening, care and treatment."

"The level of health inequalities faced by patients suffering from PKU in Europe is unacceptable. Debating these with members of the Parliament in Brussels is a crucial step to draw attention to the condition which is one of the most common rare diseases in Europe", said Eric Lange, President of ESPKU. "Through this initiative, we call on European policy makers and other decision-makers to help us give our PKU patients an equal chance of living a healthy and productive life, no matter where they live."

Maria Gizewska, Paediatrician, Poland, stressed: "The medical community is well aware of their responsibility and role in finding broader consensus on therapeutic threshold, treatment targets and advice. Despite recent advances in many aspects of PKU, there is however still a lack of baseline data on the condition globally, and more clinical evidence is needed to overcome this knowledge gap."

Eric Lange, ESPKU, concluded: "I call on those here in the room today to include PKU in activities undertaken at EU level such as the upcoming initiatives on Newborn Screening, and the Implementation Report on National Plans. Further, I would like to encourage healthcare professionals in particular to continue their endeavours in developing much-needed clinical guidance to ensure that all PKU patients receive the optimal care they deserve."

The debate is the first of a series of E.S.PKU initiatives to prioritize PKU as a rare disease on national health agendas, address unequal access to treatment and gaps in reimbursement.

Consensus paper on optimal PKU care across Europe from patient's perspective

Ongoing project

Since delegates of 26 ESPKU members have appointed structure and content of a consensus paper on optimal PKU care at a workshop during the ESPKU Annual Conference 2011 in Warsaw (Poland), a dedicated workgroup took over to adapt the draft paper with the comments of the workshop.

The workgroup was composed by delegates of different ESPKU member associations: Paul van Berkel (NPKUV, The Netherlands), Gregor Hammerschmidt (ÖGAST, Austria), Marketa Lhotakova (NSPKU, Czech Republic), Rosalia Pasqual Saludes (FAE PKU, Spain), and Tobias S. Hagedorn (DIG PKU, Germany and ESPKU).

Their results have been presented to the participants at the ESPKU Delegates Meeting in Gent (Belgium) on May 19th, 2012 for a final review.

The paper summarizes in a descriptive way all aspects of PKU care which are important from patients point of view, from screening to care, follow-up and monitoring.

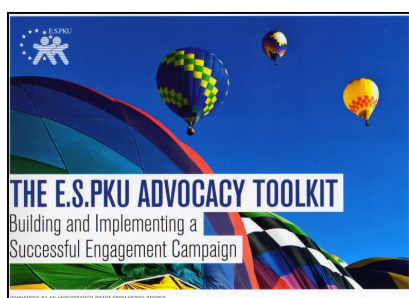
The paper raises the urgency of international guidelines and provides solid input from patient's perspective for those scientific professionals invited to set up these guidelines within the coming two years.

The paper will be peer-reviewed and finally published in a scientific journal.

The ESPKU Advocacy Toolkit*

Published May 2012

In order to arm its members with sufficient knowledge, ESPKU has edited a guide for successful advocating. This advocacy toolkit encompasses a simple approach to developing an advocacy campaign, providing guidance on how to initiate policy initiatives to raise awareness of the challenges that many people with PKU face with the ultimate objective of shaping the national health agenda to better serve PKU patients.



The objectives of the toolkit are to

- assist members of ESPKU in raising awareness of PKU at a national level
- help coordinate efforts across Europe thereby reducing the inequalities between countries on issues such as access and availability to all treatment options and care and support for families, which in turn facilitates exchanging best practices between national organisations.

The toolkit is divided into 10 steps. For each of the 10 steps, ESPKU member associations will find guidance on how to implement them combined with some practical examples and useful templates and other materials in the annex.

"We shall advocate for a common goal: More comprehensive and equal PKU services, including diagnosis, treatment and after-care", Eric Lange (President of ESPKU) said in the foreword.

The Advocacy Toolkit is available printed and digitally via ESPKU website www.espku.org.

ESPKU Nomination for membership of scientific committee of IRDiRC

ESPKU nominated the secretary of its Scientific Advisory Committee, Kirsten Kiaer Ahring, R.D., to participate as member in the Therapies Scientific Committee of IRDiRC (International Rare Diseases Research Consortium, settled at the European Commission, DG Research and Innovation). The nomination has not been successful. Not all candidates of a high number of outstanding nominations could be appointed by the Executive Board of IRDiRC.

3. Relations to other associations

A. Memberships

After being accepted as member of EURORDIS in 2011, this year ESPKU applied for membership in two further organisations:

ORPHANET

Orphanet is the reference portal for information on rare diseases and orphan drugs, for all audiences. Orphanet's aim is to help improve the diagnosis, care and treatment of patients with rare diseases.

EPPOSI

Epposi (European Platform for Patients Organisations, Science and Industry) is an independent, not-for-profit, partnership-based and multi-stakeholder think tank based in Brussels (Belgium). Their goal is to work at the "cutting edge" of European health policy-making, providing members and the wider public with high quality independent research, capacity-building, knowledge exchange and dissemination with the aim of bridging the gap between innovation and improved public health outcomes. In order to fulfil their mission, Epposi enables consensus-driven, equally-weighted outcomes between the different stakeholder groups of its membership: Patients' organisations, science and industries.

National PKU Association of America-2nd Bi-Annual Meeting Philadelphia

It is the aim of the ESPKU Board to be involved with other organisations to facilitate a better exchange of information & understanding in the world of PKU. This was the first part of an exchange visit. The NPKUA Executive Director will be attending the 2012 ESPKU Conference in Liverpool.

It was a pleasant surprise to see our Croatian Delegate -Sanja Peric-on holiday in the US-at the same meeting.

B. Participations in other meetings and conferences

ESPKU representatives took part in different meetings to build and maintain personal relationship and trustful cooperation:

EPPOSI Stakeholder Day, Brussels (Belgium), April 19th, 2012

The Epposi stakeholder day was an open day for all Epposi members and interested stakeholders to hear the latest updates on Epposi's four programme areas and discuss their development:

- Chronic Conditions Management
- Health technology Assessment
- Innovation in Healthcare
- Rare Diseases

EURORDIS: European Conference on Rared Diseases and orphan Products, Brussels (Belgium), May 24th–25th, 2012

The event was entitled "A better future for patients: Shaping together the Agenda 2020". Delegates from 55 countries took part, close to half of them were patient representatives.

The presence of EU Commissioner for Heath, John Dalli, highlighted the commitment of the European Union to the field of rare diseases.

The conference was structured around seven thematic pillars of strategic importance to the European rare disease community, including national plans for rare diseases and patients empowerment.

Canadian PKU and Allied Disorders Inc., Annual General Meeting, Alliston (Ontariom Canada), July 13th–15th, 2012

The relationship between CanPKU and ESPKU is historically good. CanPKU President John Adams regularly participates in ESPKU Conferences. CanPKU is focussing more on future therapeutic approaches on PKU, wheras ESPKU is concentrating on political issues. At the event, CanPKU released a "PKU and the brain", a new 44-page booklet about latest research findings about the effects of PKU.

4. Summary and conclusions of the ESPKU year 2012

ESPKU is consequently following its roadmap to "Closing Gaps in Care of Phenylketonuria": After having researched and proved the inconsistencies in treatment and access to treatment in 2010 and 2011, the results have been presented with the ESPKU benchmark report to EU healthcare politicians as well as international scientists, combined with some clear messages and call-to-actions:

With the lunch debate at the European Parliament, PKU is known at the EU level as an example for rare diseases and the need to prioritise the needs for PKU and rare diseases in the EU and national health policy agendas.

With the invitation to dedicated international scientists, a major step has been taken to reach scientific consensus and to set up international guidelines on PKU management from diagnosis to treatment and after-care.

By describing the optimal care from patient's perspective with a consensus paper, ESPKU member associations showed their readiness to give valuable input and support to this process.

It must be stressed, that the entire campaign is based on close cooperation between ESPKU members and with ESPKU as a real community. Together, we can make a difference to the quality of life of all the people with PKU and their families and provide a united political front across Europe.

5. A look into future

Closing Gaps in Care – the next steps

After having reached consensus on optimal care of PKU amongst the ESPKU member associations, about 15 – 20 dedicated professionals have been invited by the board of the ESPKU Scientific Advisory Committee, to find consensus on international guidelines on the management of Phenylketonuria, including diagnosis, treatment and after-care.

The publication of these guidelines is expected for the rare disease day on February 28th, 2014.

Additionally, ESPKU and its member associations will put efforts in a coordinated advocacy campaign on European and national / regional level for a more comprehensive and equal PKU services and access to all treatment options.

Future ESPKU Conferences

Since Austrian member ÖGAST withdrew their application to host the ESPKU Annual Conference in 2013, Belgium member association BOKS stepped in and will host the 2013 Annual Conference.

Spanish member association FAE PKU has applied to host the 2014 Annual Conference.

German member association DIG PKU is considering to host the Annual ESPKU Conference at the occasion of their 40th anniversary year 2015.

The upcoming annual meetings of ESPKU member's delegates in spring will take place in Belgium.

6. Impressum

This Annual Report 2012 was presented to the delegates of the attending member associations of E.S.PKU at the 2012 General Meeting of E.S.PKU, held on October 19th, in Liverpool, United Kingdom.

It gives insight into the main working fields of E.S.PKU from November 2011 until October 2012, without laying claim to completeness.

This Annual Report 2012 is published at www.espku.org

On behalf of the E.S.PKU Executive board:


Eric Lange, President
Tobias S. Hagedorn, Secretary

**European Society for Phenylketonuria
and Allied Disorders treated as Phenylketonuria
(E.S.PKU)**

is a non-profit organisation promoting exchange of information about phenylketonuria (PKU) and allied disorders of amino acid metabolism.

Address of correspondence:

Mr. Tobias S. Hagedorn (Secretary)

Wilhelm-Mellies-Strasse 10

32120 Hiddenhausen

Germany

www.espku.org

*The European Parliament Lunch Debate is the first of a series of E.S.PKU initiatives to prioritize PKU as a rare disease on national health agendas, address unequal access to treatment and gaps in reimbursement. These initiatives are supported by an unrestricted grant from Merck Serono.

*The ESPKU Advocacy Toolkit "Building and Implementing a Successful Engagement Campaign" is sponsored by an unrestricted grant from Merck Serono.

The European Society for Phenylketonuria and allied disorders treated as Phenylketonuria is a non-profit organisation with members in the following countries:

Austria	Hungary	Portugal
Belgium	Iceland	Slovakia
Bulgaria	Italy (3)	Slovenia
Croatia	Latvia	Spain
Czech Republic	Lithuania	Sweden
Denmark	Moldova	Switzerland (2)
Estonia	Montenegro	The Netherlands
Germany	Norway	Turkey
	Poland (2)	

Executive Board:

President: Mr. Eric Lange, United Kingdom
 Vice-President: Miss Dinah Lier, Switzerland
 Treasurer: Mr. Maarten Criem, Belgium
 Secretary: Mr. Tobias S. Hagedorn, Germany
 Assessor: Thomas Moller Nielsen, Denmark
 Assessor: Gregor Hammerschmidt, Austria

Board of the Scientific Advisory Committee:

Chairman: Dr. Francjan J. van Spronsen, The Netherlands
 Vice-Chairman: Dr. Maria Gizewska, Poland
 Secretary: Mrs. Kirsten Ahring, Denmark

Bank Account:

IBAN: BE66 7360 0196 7043

BIC: KREDBEBB