E.S.PKU

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

Annual Report 2011

Mission accomplished?

> **Obstacles are those frightful things**
> **you see when you take your eyes off your goal.**
>
> Henry Ford

Words of gratitude:

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

Content

Part I: Executive Board Report 2011

Administration

No one is infallible. A liability insurance was selected and signed to cover the most important risks of the voluntary work of ESPKU officers and the association itself in time of increasing responsibilities.

Executive Board Communication

Since the last Annual Conference 2010 in Denmark, 2 board-meetings have been held to coordinate the daily work of the Executive Board Members. Both meetings have been held in Warszaw, Poland. During the year, details have been discussed and appointed via e-mail and phone calls.

The ESPKU Executive Board forms a team. Repeatedly board members filled a gap left by a colleague who was unexpectedly overloaded with problems out of private reasons. This became possible since the fields of duty have been redifined: Responsibilites are not longer attached to the position of each board member, but rather specifically appointed in particular cases.

Nevertheless, the implementation of an assessor as additional member of the Executive Board is strongly recommended due to the increasing duties of ESPKU and their importance.

External Relations

EURORDIS

The membership application of ESPKU in EURORODIS (European Organisation for Rare Diseases) has been accpted on March 4th, 2011. EURORDIS brings together more than 460 national and international operating member organisations, which represent the interests of patients with rare diseases.

In long term, we expect to profit by the experiences of other EURORDIS Members and by the support of a strong and well known organisation with numerous opportunities.

Communication with patients and relatives

ESPKU News

Due to an unforseeable health problem in his family, the editor of ESPKU News was unable to publish another issue of this magzine in 2011. The next issue is planned to be distributed in full color at the beginning of 2012.

ESPKU website

The ESPKU website (www.espku.org) was constantly updated, including an improvement of the conference registration module. Nevertheless, especially this module is subject to be improved by an ongoing learning process in cooperation with the local conference organisers.

Via the ESPKU website an increasing number of individual requests were addressed to ESPKU, mostly answerd by providing contacts to the national ESPKU member associations or other institutions.

Additionally, we have been asked for information on European level regarding different subjects, such as

- Military services and PKU,
- Reimbursement policy for food, drugs and dietary products for special medical purposes,
- Nutritional content of regular and low protein food.

International PKU Adults Meetings

Two international meetings for adult patients have been organised in 2008 and 2009 by individual patients on private basis at the venue of the Annual ESPKU Conference in 2008 and 2009.

For 2011 a closer cooperation has been appointed with ESPKU: The content quality of the meetings shall be improved by organisers and logistical support will be returned by ESPKU.

This year's meeting took place from 9th unitl 13th October in Warsaw. A group of adult PKU patients stayed at appartements in the city of Warsaw, experienced not just fun events but also discussed different topics in workshops and cooked together.

Communication with ESPKU members

Delegates Meeting

As suggestedc at the General Meeting 2010, a meeting with delegates of the ESPKU member associations took place in April 2011 in Warsaw. In a very intimate and informal atmosphere delegates from six countries discussed different topics, such as

- Past and upcoming Annual ESPKU Conferences
- Actual individual main duties and activities in the national associations
- The need for unified international treatment guidelines for PKU

Above all content, the most important aim of the meeting was to carry forward the association renewal of ESPKU to a real community of cooperating partners, which was identified to be a key for succuess for the future of ESPKU at the General Meeting in 2010.

Newsletter

Another suggestion at the General Meeting 2010 was to implement a newsletter to update ESPKU members with available actual information on science and politics as well as on activities of ESPKU, members and other institutions. The newsletter offers basic information without going into details, whereas magazines or websites present detailed articles on a specific topic.

Two issues have been edited with support by Timon Schaffer (Austria, layout) and circulated amongst the ESPKU member delegates. The Newsletter shall be established to provide requested contents on regular basis. All inputs are welcome.

Political work

Benchmark report

At the Annual Conference in 2011, we will present a benchmark report on the management of phenylketonuria within EU healthcare economies, titled

PKU – a pan-European Perspective

The aim of this survey was to pave the way towards a more consistent management of PKU across all European nations. It is designed to prepare the basical work for up-to-date pan-European guidelines on diagnosis, initial treatment, monitoring and continuing care of PKU. It also seeks to investigate access to care, reimbursement policies and how these issues influence the patient’s perspective on their condition.

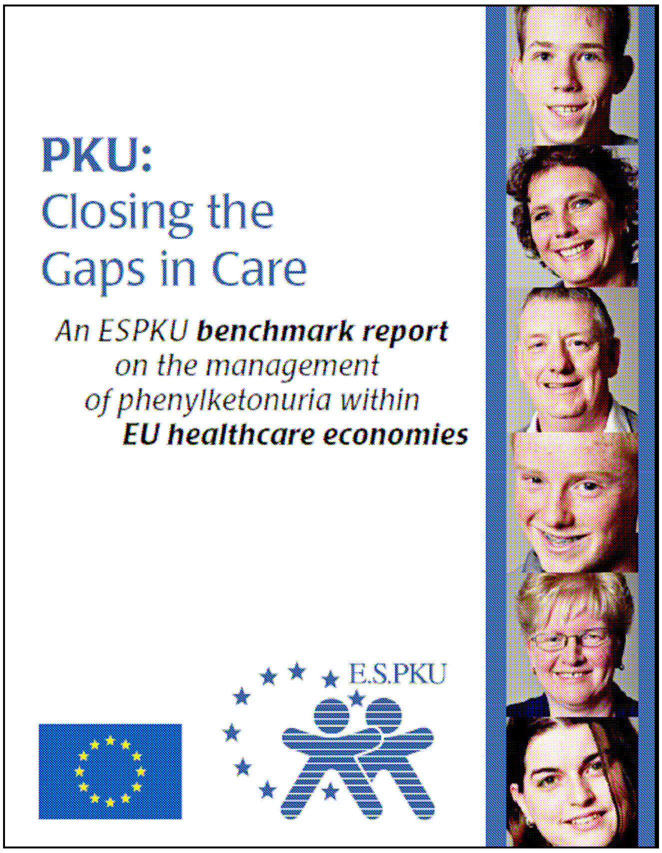
By focusing on the management policies within five EU member countries, the survey presents a benchmark whereof the progress of PKU care during the next few years wil be judged.

As it’s starting point the survey takes the well-documented observation that European PKU management is currently disparate and inconsistent. Therefore, it seeks to highlight good practice and provide advice on how this might be disseminated and adopted more widely.

The survey also presents the views of relevant stakeholders such as health authorities, health care professionals, patients and carers on national and international level.

Based on the survey analysis, the report will draw conclusions and present recommendations for consideration for the European Council, national governments and professional medical bodies.

This project is supported by a restricted educational grant from Merck Serono.



PKU:
Closing the
Gaps in Care

An ESPKU **benchmark report**
on the management
of phenylketonuria within
EU healthcare economies

E.S.PKU

Consensus paper on good PKU management across Europe from patient's perspective

There are only established PKU guidelines in three countries, including UK, Germany and France. There is no dedicated scientific society that is responsible for establishing international standards. Therefore, a dedicated group, the EPG (European Phenylketonuria Group), was founded and is currently led by Professor Nenad Blau. Up to now, there has neither been any consensus amongst professionals about the content of medical guidelines nor about some basic metrics e.g. target phe plasma concentration.

During an SSIF (Serono Symposia International Foundation) meeting, the EPG achieved some agreements on PKU standards which are, in a first step, being pulled together into a consensus statement and planned to be published by the end of 2011.

In the development of such medical guidelines, the ESPKU would like to play an active role by providing input about good PKU management from a patient's perspective and raise the urgency for guidelines.

The kick-off event for this longterm-project took place at the delegate's programme of the Anuual ESPKU Conference 2011 in Warsaw, Poland. Structure and content of the consensus paper have been appointed, an ESPKU Consensus Paper working group was elected, and consensus on representatives of a Steering Committee Group was achieved.

European Parliament contacts

During 2011, contacts to two Members of the European Parliament have been made. At personal meetings in Brussels, both became interested for the issues of ESPKU.

Both are willing to offer support and open doors for further useful contacts to other MEP's.

Beside some minor suggestions, one result of the meetings was that ESPKU may be able to present the benchmark report at some stage in Brussels and can put some emphasis on the inequality of treatement on a disease like PKU within Europe.

Part II: A review of 3 years 2008-2011

Starting point

After decades of successful work of ESPKU, a new executive board was elected at the General Meeting 2008 in Iceland. At this time, ESPKU was mainly established as a conference association promoting small networks amongst members and giving some political support on European level.

Existing relations to medical and nutritional professionals were based on the individual activities of the Scientific Advisory Committee Board.

The biggest issue of ESPKU was to provide exchange of information between member associations.

Development since 2008

The Annual Conferences

The conference structure has been changed from alternating "small and big meetings" to open conferences with parallel programmes for patients and families, professionals and member association representatives. The attendance increased to a stable level of about 300 participants as a result of the increasing conference standard.

Due to that fact the conference organisation has been reformed: Responsibilities of local organisers and ESPKU have been fixed in a manual and technical tools have been implemented for a more professional conference organisation.

Industries

Topical partnership instead of pure economic sponsorship is the strategic guideline for our relations to the nutritional and pharmaceutical industries in the field of PKU. Based on these partnerships some projects have been executed successfully on a professional level.

Coevally, ESPKU remained independend with regards to content and economics.

Major projects

Apart from the Annual Conferences, the focus of ESPKU work turned from the member associations to the patients:

A multilingual book "Living with PKU" was edited and published. It includes real stories of people from around the world, who live with PKU and shared their experiences to make this rare condition less isolating.

An online survey of ("Take charge of PHE") was carried out to understand the attitudes and behaviour of patients, families and healthcare professionals treating PKU. It identified gaps in service provision and opportunities to improve services for people living with PKU.

A benchmark report on the management of phenylketonuria within EU healthcare economies was published (see page 7).

Improvement of internal cooperation and networking

By involving patients and delegates from different countries in the major projects, the ESPKU work became more observable. Parallely, more and more adult patients are taking over responsibilities in their national associations. The interrelation between these facts led to a first level of real networking and concerted action.

Strategic alignment

It is the most important purpos of ESPKU to become recognised as the strong partner for patients and their representatives in the national patients associations, as well as for health care providers, industries, insurance companies and political decision makers.

The more successful we strive for this goal, the more successful we can protect the interests of PKU patients.

Part III: Prospect

The mission is not accomplished!

In the era of the internet as most important source of information it is not sufficient to provide basic information and organise an annual conference. Some first important steps have been made to “turn the ship” and to design sustainable means of existance. This is a promising start of a development which has to be carried on in the future.

A voluntary working board of officers, composed by patients and relatives guarantees high engagement and personal identification. Nevertheless, the implementation of an assessor as additional member of the Executive Board is strongly recommended due to the increasing duties of ESPKU, their importance and complexity.

In long term it has to be considered how this voluntary engagement has to be complemented with professionalism, political knowledge and effective methods of modern communication.

Impressum

This Annual Report 2011 was presented to the delegates of the attending member associations of E.S.PKU at the General Meeting 2011 held on October 14th in Warsaw, Poland. It gives insight into the main working-fields of E.S.PKU from November 2010 until October 2011 without laying claim to completeness.

This Annual Report 2011 is published at www.espku.org

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

Tobias S. Hagedorn

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.

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

- Austria
- Croatia
- Czech Republic
- Denmark
- Estonia
- Germany
- Hungary
- Iceland
- Ireland
- Italy
- Latvia
- Lithuania
- Moldova
- Norway
- Poland
- Portugal
- Romania
- Serbia
- Montenegro
- Slovakia
- Slovenia
- Spain
- Sweden
- Switzerland
- The Netherlands
- Turkey
- United Kingdom

Executive Board:
President: Mr. David Abeln, The Netherlands
Vice-President: Miss Dinah Lier, Switzerland
Treasurer: Mr. Maarten Criem, Belgium
Secretary: Mr. Tobias S. Hagedorn, Germany

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

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