Polka

a project by Eurordis
and its partners

Patients' Consensus on Preferred Policy Scenarii for Rare Diseases

Partners

EURORDIS Rare Diseases Europe

Rare Disorders Denmark

Fundació Doctor Robert

NHS

A project financed by

Executive Agency for Health and Consumers

CSL Behring

NOVARTIS

sigma-tau Rare dedication

Polka in a few words

Overall objectives

Polka is a project conducted by Eurordis and its partners. It is co-financed by the EU Public Health Programme 2008-2013, DG Sanco. It is a 3-year project, September 2008 to September 2011.

The central idea of this project is to foster the opinion of patient representatives on future European policies for rare diseases, or to collect their views on existing ones. For the former, entertaining sessions to familiarise patient representatives with complex scientific issues are developed, the "PlayDecide sessions". Once opinions have emerged, a more-in-depth exercise will help patient representatives to define their preferred policy (Delphi-like method). For policies that are already in place, the project will focus on the policy for European Reference Networks and Centres of Expertise for rare diseases. It proposes to evaluate these networks three years after the first ones received support from European institutions. All these themes and policies will be debated during the 5th European Conference on Rare Diseases, ECRD 2010 in Poland, which is the third pillar of the Polka project.

Strategies and plans for rare diseases are currently being developed by the European Union and many of its Member States. Eurordis and its partners believe that patient input into this process is of the utmost importance. Polka, a new project launched in September 2008, has been set up to respond to this objective. The project will facilitate the consultation of the European rare disease community at large, with the aim of building consensus on preferred public health policy scenarios for rare diseases: genetic testing, cost of treatments, xenotransplantation, telemedicine…
Five to seven topics will be selected and two different methods will be used to address them: patients' deliberative sessions using an entertaining approach developed by Ecsite, the European network of Science Museum: PlayDecide sessions. This new tool was developed to learn more on complex, scientific and controversial issues and was inspired by the democs.gov in the US: PlayDecide sessions, a playful approach to form an opinion.

1. *PlayDecide at the European Commission*

2. *PlayDecide at Eurordis*

In parallel to the PlayDecide sessions, we will also organise Delphi-like consultations to develop common policies on given topics.

Other important aspects of the project, are the European Reference Networks for Rare Diseases and the 5th European Conference on rare diseases 2010 in Poland.

Deliberative patients' debates

To build consensus on preferred scenarii for rare diseases

This activity represents a modern and entertaining method to empower patients and their representatives to become advocates for their cause.

The European network of Science Museums "Ecsite" has developed a new tool to learn more on complex, scientific and controversial issues, inspired from the democs.gov in the US: PlayDecide sessions, a playful approach to form an opinion. Sessions on xenotransplantation, pre-implantation diagnosis, stem cells etc. have already taken place in Europe, with the general public as participants.
Eurordis and its partners have decided to use these subjects, and to create new ones of particular importance for rare diseases. These subjects will be translated and adapted to most EU languages so that patients, families, parents, policy makers, health care professionals can participate.
Moderators in various Member States will organise debates with up to eight patient representatives per debate. In total, the project should facilitate between 600 and 1 000 discussions across 27 countries, in 21 languages, with a minimum of 80 participants per country !
Eurordis team has already "play-decided" and all reported the session to be very interesting.

After the debates, how to go further ?

These debates triggered by the PlayDecide sessions are meant to inform participants about topics, concepts and definitions they might know little about. For patients' representatives who would like to continue after the PlayDecide sessions, Polka proposes another method to help patients' representatives communicating on these issues when meeting with health authorities or health care professionals.

Participants who want to continue the debate and exchange more views and opinions will meet with experts, and reach a broader consensus with a larger section of the rare disease community.

Polka proposes to help this process via a Delphi-like method (a method to arrive at a consensus among experts), in two rounds:

1. *First round: team leader with participants (20 groups of 10 to 12 participants).*
   The team leader will present the goal of the exercise and summarise the debates, prepare materials. He/she will lead the discussion, collect opinions, observe diverging points of view, and propose a consensus. At the end, he will write a synthesis document that prepares the second round
2. *Second round: adapted questionnaire to a larger group of patient representatives to comment on the synthesis document*
   The questionnaire will present the options set out during the first round, highlighting convergence.

This part of the Polka project is lead by ***Lene Jensen,*** *Director, Rare Disorders Denmark.*

Bridging the gap between patients' representatives and European Reference Networks for rare diseases

What we want to achieve

Centres of Expertise

European Reference Networks for rare diseases are networks of centres of expertise for rare diseases established in various member states. European Reference Networks for rare diseases gather specialised centres catering for a rare disease, or a group of rare diseases. By working together at the European level, they can support health care professionals in making the right diagnosis, in agreeing on standard of care, in accessing and generating information and knowledge on the disease. Their objective is the improvement of patient health outcomes.

To support and guide the implementation of the EU policy for European Reference Networks from the patients' perspective, the project aims at involving patients and their representatives in its development.

1. *We will disseminate a Declaration of common principles on Centres of Expertise and European Reference Networks for Rare Diseases (see below)*

2. *From these principles, a Charter of relations between patient organisations and reference networks will be tested in real practice*
3. *We will propose an evaluation of Reference Networks from the patients' perspective, in close relation with the EU High Level Group and the Rare Disease Task Force*

The 9 European Reference Networks

The 9 European Reference Networks which we will work with are:

1. ***ECORN-CF*** *(European Centres of Reference Network for Cystic Fibrosis)*
2. ***DYSCERNE*** *(European Network of Centres of Reference for Dysmorphology)*
3. ***RARE CARE*** *(rare cancers)*
4. ***EPNET*** *(European Porphyria Network)*
5. ***PAAIR*** *(Patient Associations and Alpha1 International Registry)*
6. ***European Network of Rare Bleeding Disorders***
7. ***European network of paediatric Hodgkin's lymphoma***
8. ***NEUROPED*** *(European Network of Reference for Rare Paediatric Neurological Diseases)*
9. ***EURO HISTIO NET*** *(A reference network for Langerhans cell histiocytosis and associated syndrome in the EU)*

The Declaration of Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases

(See insert 2)

This part of the Polka project is lead by ***Dr Edmund Jessop,*** *Advisor, National Commissioning Group, NCG NHS UK.*

5th European Conference on Rare Diseases 2010 in Poland

2010 Krakow
European Conference on Rare Diseases

The 5th European Conference on Rare Diseases 2010 will take place in Krakow on the following dates:
13-15 May 2010
The two co-chairs are Torben Gronnebaek, President of Rare Disorders Denmark, and Prof Josep Torrent I Farnell, Director of the Fundacio Doctor Robert and Committee for Orphan Medicinal Products EMEA.
A preliminary programme will be available soon.

Contact Eurordis: kasia.peala@eurordis.org to learn more about the conference and how to register.

1.
Rynek square, Krakow

The Motto of the European Conference for Rare Diseases

The European Conference on Rare Diseases is a unique platform/forum across all rare diseases, across all European countries, bringing together all stakeholders (academics, health care professionals, industry, policy makers, and patients' representatives).
It covers research, development of new treatments, health care, social care, information, public health and support at European, national and regional levels.
It is a biennial event, presenting the state-of-the-art environment in the field of rare diseases.

Dates	**13-14-15 May 2010**
Place	**Krakow**
Participants	**400-600**

Interpreted in 5 languages for some sessions: English, French, German, Polish and a fifth one, to be defined at a later stage.

Main themes

1. **National strategies and plans for rare diseases**
2. **European Reference Networks and Centres of Expertise for rare diseases**
3. **Services to patients, families and carers**
4. **Rare diseases in Central/Eastern Europe**
5. **Translational research in rare diseases**
6. **Health education, information, patient empowerment**
7. **Policy scenarii for rare diseases (WP4 Polka)**

Programme Committee

*This part of the Polka project is lead by **Prof Josept Torrent I Farnell,** Director, Fundacio Doctor Robert, who is also co-chair of ECRD 2010 in Krakow.*

***Torben Gronnebaek,** Rare Disorders Denmark, is co-chair of ECRD 2010.*

Members of the Programme Committee

Patients' representatives

Torben Gronnebaek	Rare Disorders Denmark
Dorica Dan	Romanian Alliance of Rare Diseases Organisations
Pawel Wojtowicz	Cystic Fibrosis Poland
Raynald von Gizycky	Proretina Europe
John Dart	Debra United Kingdom
Lia van Ginneken	European Myeloma Platform

Health Care Professionals

Prof Josep Torrent i Farnell	Fundacio Dr Robert, COMP
Dr Ségolène Aymé	Orphanet, Rare Disease Task Force
Prof Tomasz Grodzicki	Faculty of Medicine Krakow
Dr Bozenna Dembowska-Baginska	COMP member, Poland
Dr Frits Lekkerkerker	NDA, The Netherlands
Dr Kerstin Westermark	COMP Chair, EMEA

About the project

Steering committee and partners

Associated beneficiaries:

Eurordis

Rare Disorders Denmark

Fundacio Doctor Robert

National Commissioning Group NHS UK

Collaborating partners:

Dutch Steering Committee on Orphan Drugs and Rare Diseases

MPA – Medicinal Products Agency, Sweden

Project co financed by the EU Public Health Programme 2008-2013

Important dates

Polka Project starts	Sep. 1st, 2008	
1st Donors Committee meeting	Dec. 11th, 2009	Paris
1st Polka Project Steering Committee	Jan. 15th, 2009	Paris
Programme Committee ECRD	Aug., 2009	Krakow
1st Polka project interim report	Aug. 31st, 2009	
ECRD 2010 Programme Committee	Oct., 2009	Krakow
5th European Conference on Rare Diseases ECRD 2010 in Poland	May 13-15th, 2010	Krakow
2nd Polka project interim report	Aug. 31st, 2010	
Polka project ends, final report	Aug. 31st, 2011	

Contacts

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CSL Behring

Novartis

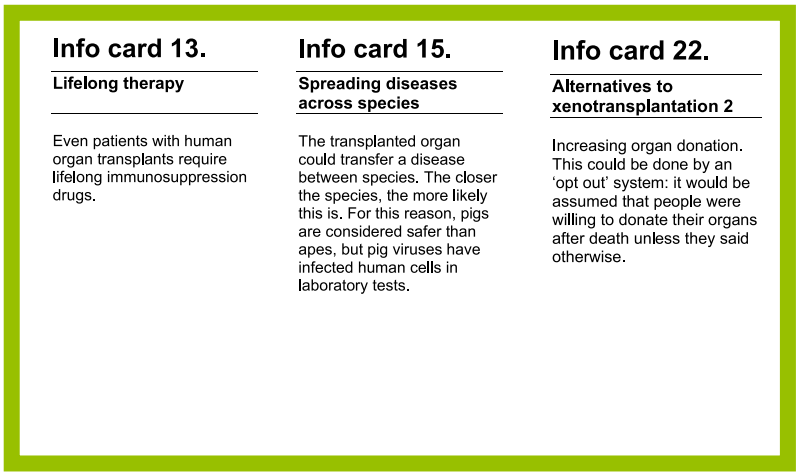
Sigma Tau

graphic design: http://bapliste-ferrier.fr

1. Information cards

A set of 24 infocards are prepared by experts, written in lay language. They set the scene. Everyone takes the time to read them, and they are followed by group discussion.

1.
Examples of issue cards from the Xenotransplantation Decide game



Info card 13.

Lifelong therapy

Even patients with human organ transplants require lifelong immunosuppression drugs.

Info card 15.

Spreading diseases across species

The transplanted organ could transfer a disease between species. The closer the species, the more likely this is. For this reason, pigs are considered safer than apes, but pig viruses have infected human cells in laboratory tests.

Info card 22.

Alternatives to xenotransplantation 2

Increasing organ donation. This could be done by an 'opt out' system: it would be assumed that people were willing to donate their organs after death unless they said otherwise.

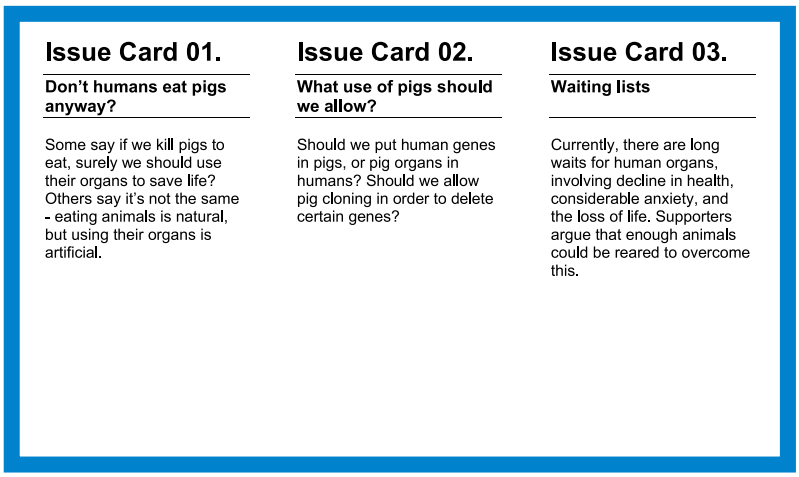
1

insert 1

2. Issue cards

A second set of 24 cards is read about issues. Participants pick up 3 issues they would like to discuss. All can react.

2.
Examples of issue cards from the Xenotransplantation Decide game



Issue Card 01.

Don't humans eat pigs anyway?

Some say if we kill pigs to eat, surely we should use their organs to save life? Others say it's not the same - eating animals is natural, but using their organs is artificial.

Issue Card 02.

What use of pigs should we allow?

Should we put human genes in pigs, or pig organs in humans? Should we allow pig cloning in order to delete certain genes?

Issue Card 03.

Waiting lists

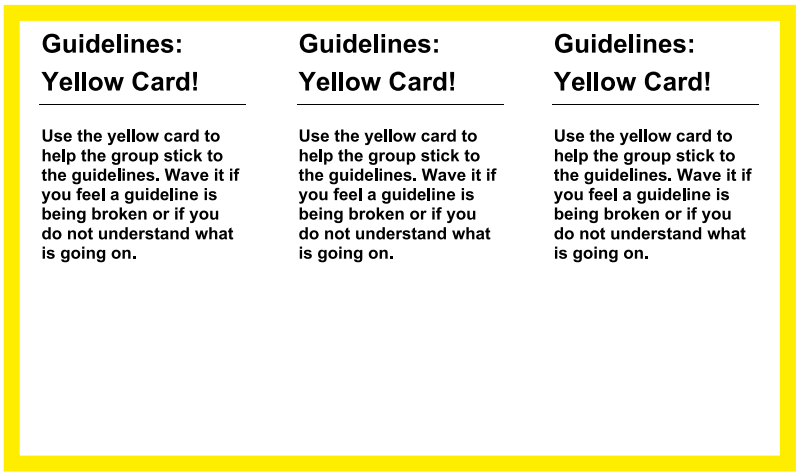
Currently, there are long waits for human organs, involving decline in health, considerable anxiety, and the loss of life. Supporters argue that enough animals could be reared to overcome this.

2

3. Yellow cards

Yellow cards are for the moderator to keep the participants focused and fair.

3.
Examples of yellow cards of a Decide game



Guidelines: Yellow Card!

Use the yellow card to help the group stick to the guidelines. Wave it if you feel a guideline is being broken or if you do not understand what is going on.

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3

4. Challenge cards

16 cards used by the moderator to address all challenges raised by the subject and to give a new turn to the discussion.

4.
Examples of challenge cards from the Xenotransplantation Decide game

insert 1

Challenge Card

Can we justify spending money on this research given the inequalities in health care between Europe and developing countries?

Challenge Card

Do you think that human needs are more important than the needs of those without a voice- nature, animals, embryos?

Challenge Card

"We should maximise human life and pursue all avenues of research to help people who are ill."

Do you agree with this statement?

4

5. Story cards

Because the issues are not theoretical, true life stories help participants to measure the consequences of their choices.

5.
Examples of story cards from the Xenotransplantation Decide game

Story card 01.

Sophia's story

I am an animal technician. Its amazing how attached you can get to the pigs we breed. It is hard to see them go, but this has to be done. What would be really wrong would be to treat these animals badly just because we know that in the end they are going to be put down as part of an

Story card 02.

Dr. Sharon Taylor

I've worked for ten years to enable pig hearts to be successfully transplanted into humans. I've seen the terrible suffering at first hand of people born with congenital heart diseases. My son, Toby, was born with a weak heart. He could barely walk and couldn't play

Story Card 03.

Ben's story

I'm waiting for a heart transplant. My heart has been dodgy for years, but a car accident last summer was such a shock to my system that it has been erratic ever since. Doctors say that the likelihood of finding a donor heart is slim. I've heard about xenotransplantation -

5

6. Policies

The group decides on a possible policy scenario, or proposes its own policy, with or without consensus.

6.
Examples of policies from the Xenotransplantation Decide game

Policy positions for Xenotransplantation

Position

1. It is ethically wrong to transplant live animal organs into human patients.

2. It is too risky to transplant live animal organs into human patients.

3. Only transplant animal organs into human patients if the benefits are sure and very large and if the benefits definitely outweigh the risks.

4. Transplant animal organs into human patients provided the risks are shown to be reason-able.

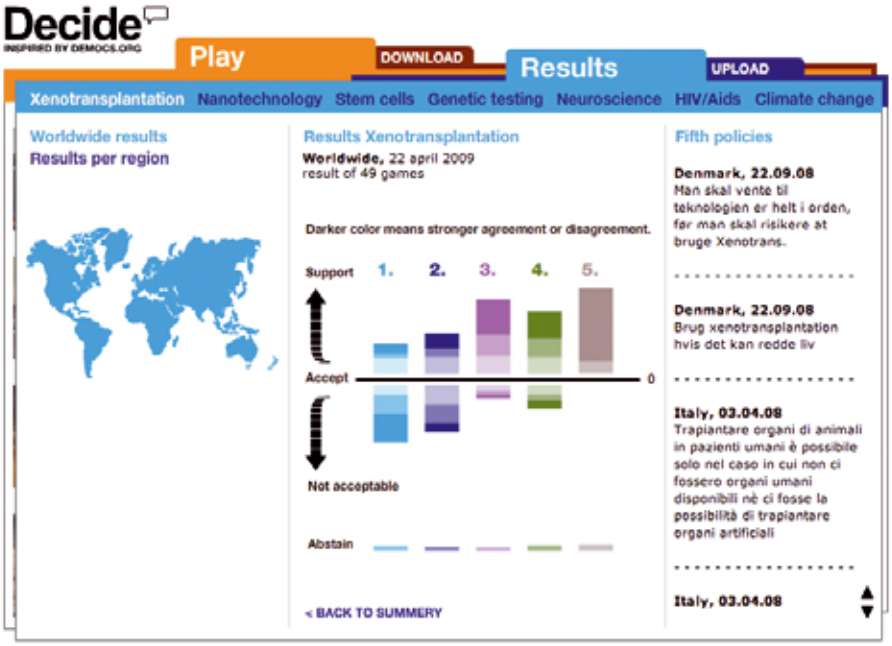
5. This group's own policy position response:

6

7. Results

When all members have voted, the results are uploaded in a database and compared to the votes of all other sessions that have taken place Europe-wide.

7.
Example of results

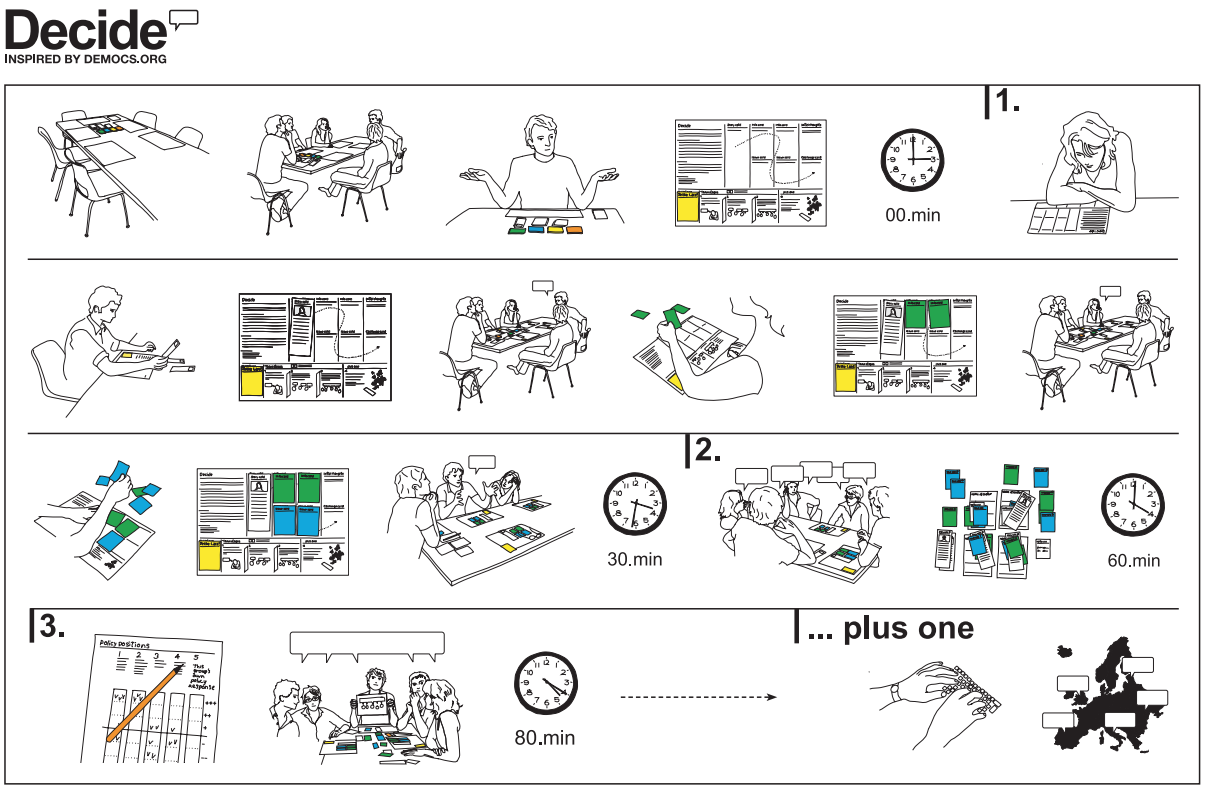


7

insert 1

All steps

8. *all steps of a PlayDecide game*



8

Specific objectives

When the topics will be selected, we will prepare materials (cards) with experts in the field. Our objectives are to involve as many patient organisations as possible to run sessions locally in all 27 European member states.
By participating in these sessions, patient representatives will be informed and will have exchange ideas and opinions on controversial and complex issues. Doing so, they will be in a better position to be the voice of the rare disease community in other fora.

insert 1

The Ecsite Network

Ecsite- the European Network for Science Centres & Museums - is the European-wide network organisation that links science centres and museums, natural history museums, zoos, aquariums universities, research organisations in Europe as active members. The common thread uniting all Ecsite members is a commitment to public engagement to communicate science through accessible, interactive exhibits and programmes.
Ecsite covers a network of 385 members worldwide, about 180 Science centres and museums in Europe visited by 40 millions persons per year and many more through their websites.
To learn more, visit: www.ecsite.net

Contacts

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How you can participate

Who you can participate?
Even though Polka is intended for the rare disease patient community, everyone can participate: families can organise sessions, or children at school, health care professionals, policy makers, employees of health industries, general public…
You can organise your own PlayDecide sessions very easily: it is no more complex than organising a Monopoly™ game with your friends.
All you need to do is:

1. *Identify five other persons to participate (this is the optimum number. The maximum participants would be eight).*
2. *Invite them to spend two hours for one session.*
3. *Identify one of them who could be the moderator, or you can volunteer to be the moderator.*
4. *Log on www.playdecide.org*
5. *Select the topic you wish to organise a session about. Topics specifically created for the rare disease community within the frame of the Polka project will be indicated.*
6. *Then select the language and download the kit : materials, and instructions.*
7. *Print the materials using coloured paper (green, yellow, white, blue) as indicated.*
8. *Read the instructions, or listen to the demonstration on www.playdecide.org*
9. *Explain the simple rules to the participants.*
10. *And you can immediately distribute the Information cards.*
11. *At the end of the session, upload the results in the database.*

The Declaration of Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases

In 2008, EURORDIS adopted the Declaration of "Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases", in order to improve patient care throughout Europe.
Rare disease patients call upon National Health Authorities to endorse, publicise and implement the following Declaration to contribute to the identification of Centres of Expertise and to support them financially.

Rare diseases are often complex diseases

1. *Centres of Expertise shall aim at providing a multi-disciplinary approach [1, 2].*
2. *Centres of Expertise shall aim at providing patient centred-care. Multidisciplinarity shall be managed in a coordinated manner [3], and shall not result in disconnected medical services.*
3. *Centres of Expertise shall represent a reliable source of accurate diagnosis, and shall include genetic testing and genetic counselling.*
4. *Centres of Expertise shall share their competences at both national and European levels [4] and shall endeavour to constantly increase and update their level of expertise.*
5. *Centres of Expertise should join in European Reference Networks for Rare Diseases.*

Rare disease patients are too often excluded from health systems and socially marginalised, in spite of their tenacious personal commitment [5, 6]

6. *Centres of Expertise shall be places where patients feel welcome and safe [7] and where patients are received by knowledgeable and understanding professionals.*
7. *Centres of Expertise shall facilitate and improve the autonomy of the patient.*
8. *Centres of Expertise shall provide access [8] to social assistance [9], which respond to the special needs of the disease [10].*

Centres of Expertise shall not only be "care giving structures", but shall also engage in the following activities

9. *Centres of Expertise and European Reference Networks shall actively involve patients and their representatives in the establishment and performance, management and evaluation of the centre [11]. These evaluations should be made publicly available.*
10. *Centres of Expertise shall exchange information with local professionals [12], including general practitioners [13].*
11. *Centres of Expertise and European Reference Networks shall disseminate information on the diseases to social and other relevant stakeholders involved [14].*
12. *Centres of Expertise shall provide training [15] to all stakeholders involved, including health care professionals, patients and their representatives*
13. *Centres of Expertise and European Reference Networks shall provide guidelines on the most appropriate care management for patients, closely integrating both medical and social aspects. They should involve patients and give them an active role as recognised partners at all stages.*

14. *Centres of Expertise and European Reference Networks shall facilitate the coordination of both basic and clinical research activities and infrastructures, including clinical trials, registries, biobanks, exploration of innovative techniques, etc. They should also be required to publish and disseminate research results, irrespective of whether the results are positive or negative*

15. *Access to Centres of Expertise must be ensured to all patients, regardless of their country or region of origin*

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insert 2

The following figures are based on the EurordisCare3 Survey on access to health services, for which a total of 5995 responses were received from 22 countries for 16 diseases, thanks to the active involvement of 130 patient organisations.

1. Each patient went through an average of four different types of medical consultation, three kinds of examination and 2.4 types of treatment over the last two years, in relation to his/her disease.

2. During the same period, almost half of these (47%) spent time in hospital for an average of three times for 20 days in total.

3. 94% of patients consider that "coordinating the sharing of medical information on the patient between all professionals who care for him/her in the specialised centre" is essential (70%) or useful (24%).

4. 95% of patients consider that "communicating with other specialised centres and professional networks to harmonise treatments and research at the national and European level" is essential (67%) or useful (28%).

5. An average of 59% of the respondents (up to 64% for the low income group) had to reduce or stop their professional activity because of their disease or to take care of a relative affected by a rare disease.

6. On average, 16% of patients (up to 24% for the low income group) were forced to move house because of their disease.

7. One out of 5 patients (18%) experienced rejection linked to their disease from healthcare professionals. The patient perceived reason of rejection is linked to the disease (80% of cases due to reluctance because of the complexity of the disease), and/or to the physical conditions of the patient : 10% for disease-related behaviour, 11%, for communication difficulties and 15% for physical aspect.

8. Every year, 28% of the patients needed the assistance of a social worker. For about one-quarter of these, access to this assistance was difficult : difficult access (18%), very difficult (9%) or even impossible (4%).

9. 92% of patients consider that "informing patients about their rights and guiding them toward social services, schools, leisure activities or vocational guidance" is essential (55%) or useful (37%).

10. Globally, social assistance services respond inadequately to the expectations and needs of rare diseases patients (only 37% of patients are satisfied), especially when the demands are specific to the disease : 27% for assistance to obtain exceptional financial support, such as the purchase of a wheel chair, 32% for assistance with social integration, school, leisure or professional integration. This inadequacy of the social assistance is more severe for the low income patients (only 26% of satisfied).

11. 96% of patients agree that "a specialised centre should involve patient organisations in order to benefit from their knowledge of daily life and needs of patients".

12. 90% of patients consider that "creating material for teachers, employers, social services, insurance companies and the general public to inform them about patients' needs and improve social integration of patients" is useful or essential

13. 44% of patients disagree that "the role of general practitioners consists mainly in looking after health problems not related to the rare disease".

14. 95% of patients consider that "coordinating the sharing of medical information between health professionals of the specialised centre and local health professionals" is useful or essential.

15. 93% of patients consider that "training local professionals to respond to the specific needs of patients" is useful or essential.