



Joint Declaration “Rare Diseases as an
International Public Health Priority”
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A common paper from RD patients at International level

2004 EURORDIS → invited by IAP0 contribute to the WHO Priority Medicines Report for Europe and the World → essential medicines list.

Main concerns:

- Confusion in terms & concepts (rare & neglected diseases)
- Definition of “essentiality” of a drug based on the size of the population affected rather than on “unmet medical needs”

Removal from the list of clotting factor concentrates. Then re-integrated after International patients group pressure.

→ December 2004 → EURORDIS Position Paper

A common paper from RD patients at International level

(EU) EU/HEALTH: Eurodis critical of WHO report on priority medicines for Europe and world

Brussels, 06/01/2005 (Agence Europe) - The European Organisation for Rare Diseases (Eurodis), which brings together 220 associations from 21 countries has recently given a severely critical assessment of the WHO's recent report on European and international priority needs for medicines. Eurodis balked at the "absurdity" of the geographical cut limiting the problematic of rare diseases to the EU and USA alone (rare diseases affect less than 1 in 2000 people). Eurodis also criticises the absence of a world-wide reflection on Orphan drugs and the access of all populations to medicines.

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A common paper from RD patients at International level

- Importance of “Essential Medicines list”: governments refer to WHO recommendations when making decisions on health expenditures.
- Need for an international initiative of some kind became more and more clear and we decided - after talking with colleagues - in the US to do “something” based on the WHO founding principle that access to life-saving medicines is a fundamental human right.

EURORDIS – which is an international umbrella organisation already - decided to launch the initiative of a Joint Declaration.

A common paper from RD patients at International level

In 2009-2010 → EURORDIS drafted a paper on “RDs as an International PH priority” that mirrors the existing paper at European level.

This process was originally linked with ICORD but then exclusively with other **POs** in order to make it the outcome of a **RDs patients movement at global level**.

The draft went out for consultation to all our International contacts over the Summer 2011.

Then re-circulated in November 2011 and the last contributions were received until May 2012.

International Survey

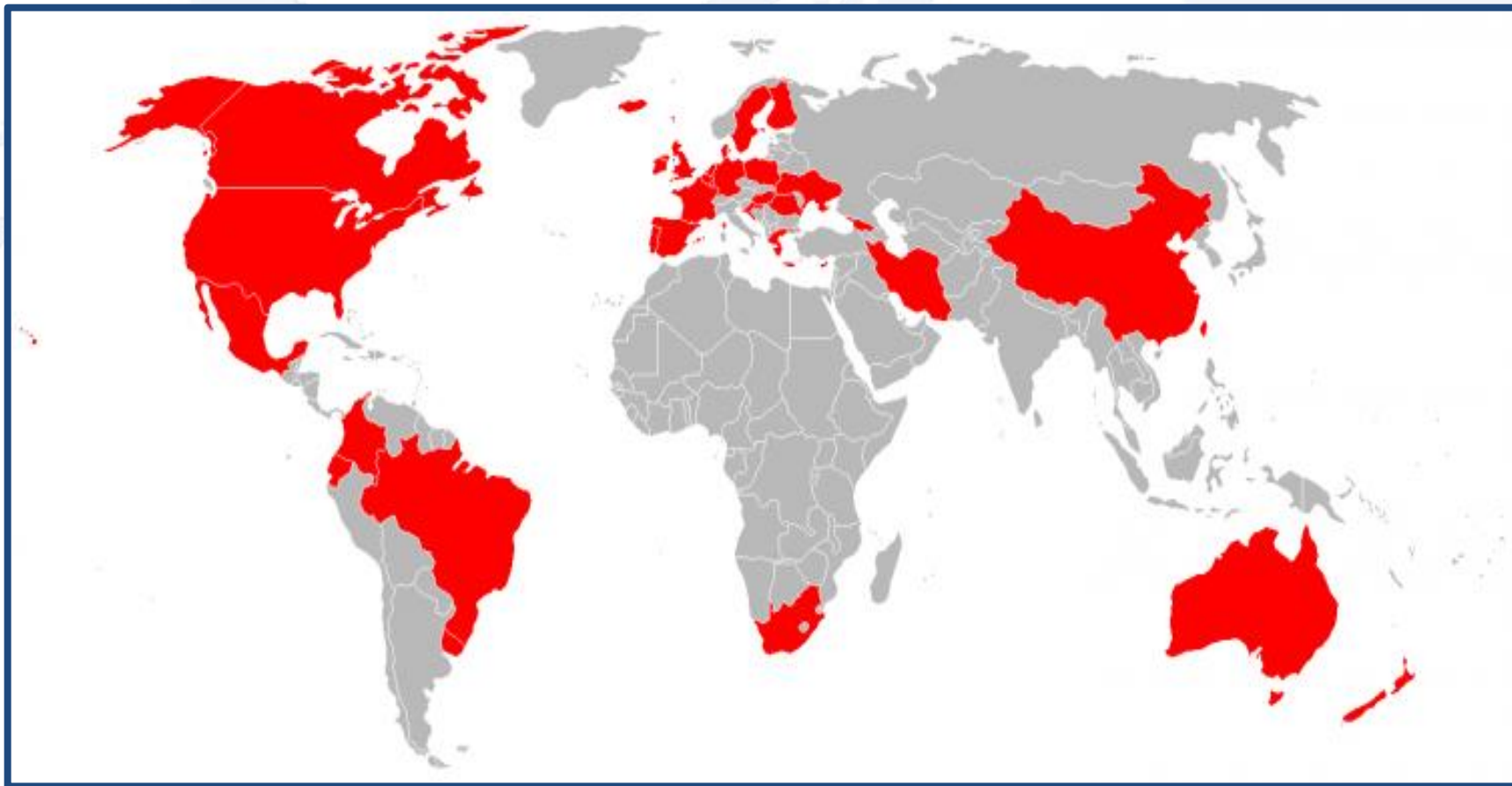
After a meeting in June 2012 with IAP0 in London, it was decided to launch a survey to increase the sense of appropriation by POs involved.

The survey was drafted and launched in September 2013.
Responses were received until December 2013.

The survey aimed to:

- Gather more information regarding the international situation of the RD field
- Assess interest of patient groups in the Rare Diseases International initiative
- Use responses to revise/update the Joint Declaration “Rare Diseases: an International Public Health Priority”

64 respondents from 37 countries around the world



Speak with one single voice: Joint Declaration “Rare Diseases as an International Public Health Priority”

- Executive Summary of the 10 Recommendations
- Part I - Development of each of the ten Recommendations
- Part II – The background paper:
 1. Objectives of the Declaration
 2. What is a rare disease?
 3. Consequences of rarity for patients and their families
 4. Clarification of related concepts
 5. RDs and national, regional, international levels
 6. Political principles (social justice and social rights)

Joint Declaration: Executive Summary and explanation of the ten Recommendations

Following the Survey we were able to extract 10 Key Recommendations on the following 5 themes:

1. **Visibility** of Rare Diseases
2. Patient **Empowerment**
3. Universal **Access** to Healthcare
4. **Research**
5. Cooperation in **policy shaping** at International level



RARE
DISEASES
INTERNATIONAL

Joint Declaration

Visibility of Rare Diseases

1. Enhance visibility of rare diseases at an international level in terms of public awareness and within the healthcare system as a human rights issue and a public health priority. A better classification and codification of rare diseases can support the process towards better recognition of rare diseases worldwide.

Survey:

- Respondents rated the level of RD Public Awareness as medium (2.6 / 5) and RD visibility within the healthcare system as below medium (2.3 / 5)
- Ways to increase visibility of rare diseases in respondents' countries and welfare systems included: Awareness campaigns through media for the general public (including TV spots, Campaigns in Schools etc.), National Plans, Specialised training for Healthcare Professionals
- Better codification and classification of RDs was ranked as important (scoring 4/5)

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Patient Empowerment

2. Support and empower patients and families through different capacity building activities adapted to the specific national needs

Survey:

- Most comments stressed that POs are currently facing a lack of funding that limits their operations and there is therefore a need for an increase in PO fundraising skills
- Respondents stated it is necessary for POs to be equipped with necessary advocacy tools to be able to approach decision makers and influence policy
- A desire to increase capacity to further engage in international activities, meetings, conferences was stressed

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Patient Empowerment

3. Develop, gather and disseminate information on rare diseases

Survey:

When asked what would be the best ways to make use of limited existing knowledge & disseminate accurate information on RDs at an international level 83.9% of respondents replied *Coordination of the information that POs provide on their websites at international level* followed by *Expanding the Orphanet Database worldwide* (76.8%)

Comments stated that more important than expanding Orphanet would be making it more accessible to patients; giving access to other sources of information; increasing quality and ensuring validity of data

Translation of existing information resources into more languages was also deemed important

4. Promote international cooperation in the field of services to patients and families

More funding sources to ensure financial security and participation of patient representatives in international events, capacity building and training, as well as more cooperation between patient groups at international level.

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Universal Access to Healthcare

5. Improve prevention, screening, and timely diagnosis

Survey:

- 80% respondents rated as very important international cooperation between health professionals in order to improve timely diagnosis
- Cooperation between HC professionals to improve diagnosis is expected to focus as a priority on CoE and International Reference Networks by 76% of respondents followed by newborn screening policies (75.5%) and genetic diagnostic testing (68%)

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Universal Access to Healthcare

6. Facilitate universal access to high quality healthcare and treatments for rare diseases patients, including surgeries, special diets, transplants, and medical devices, common and orphan medicinal products.

Survey:

Respondents rated as fundamental aspects of healthcare: access to OMPs and training of HCPs (97%), followed by capacity-building on disease management for patients and carers (95%) and habilitation/rehabilitation services (93%)

In this context (improving universal access to high quality care), international cooperation linking European and International Reference networks is considered as very important by 60.5% of respondents (important 35%)

This cooperation is expected to aim as a priority to develop International Guidelines for specific RDs or groups of RDs (70%) and to join EU and Intl Reference Networks (70%)

Joint Declaration

Universal Access to Healthcare

7. Support policy on rare diseases by promoting specific measures in regional / national / international strategies, including identification and support of specialised expert providers as well as their national and international networking

Survey:

In order to support RD patients worldwide, the adoption at international level of common priorities for national strategies was favoured as the best approach by more than 88% of respondents

8. Promote recognition that rarity requires increased international cooperation and mobility of experts as well as of patients when expertise is not available locally.

Survey:

In order to ensure universal access to treatment more than 71% of respondents believe that the country of origin should bare the costs of patients mobility at international level (64% an International Fund)

An increased level of transparency should be introduced in value based pricing of OMPs (agree or strongly agree more than 97%) as the best approach to enhance access to OMPs

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Research

9. Coordinate worldwide research efforts on rare diseases through IRDiRC and other comparable research initiatives, as well as networking of patient registries

Survey:

In order to improve research outcomes for patients, research efforts should be coordinated at international level (68% respondents) mainly through joining IRDiRC (more than 90%)

- 72% of respondents ranked as very important the networking of patients registries at international level.
- The results of research efforts should be widely disseminated as a matter of high priority for more than 78% of respondents and Public-Private Partnerships should be encouraged for more than 76% respondents.

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Cooperation in policy shaping at international level

10. Elaborate policies based on common values (equity, solidarity and Social Justice) that have an impact on the lives of rare disease patients.

Survey:

- In order to best support policy shaping aimed at promoting RDs at international level, advocacy activities by an international movement of RD patients is seen as the best way forward by 84% of the respondents, based on common values of equity, solidarity and social justice
- 97.6% respondents expressed interest in joining a Rare Diseases International initiative to be developed closely with IAPO