Rare 2030

Foresight in Rare Disease Policy

RARE 2030
A Foresight Study on Rare Disease Policy

2019 EMM Workshop
Bucharest, Romania

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PART | 1

WHAT IS RARE 2030?

We cannot predict the future but what we can prepare for it.

PREPAREDNESS FOR AN OPTIMAL FUTURE

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- **Foresight** is a tool that provides us with different future scenarios and sets out road maps for how we can reach them through changes in policies and strategies so to better shape our tomorrow for people living with a rare disease in Europe.

- Rare 2030 is a EURORDIS-led 2-year foresight study using a participatory approach to:

1. Identify the most relevant drivers of change in the field of rare diseases
2. Anticipate their influence over the next decades: policy scenarios through 2040
3. Propose policy recommendations through 2030 that lead us to a better future for people living with rare diseases
4. Forge a consensus on better policies so that as the RD landscape and the world changes we are prepared

PART | 2

WHY RARE 2030?

The health of 30 million people living with rare diseases in Europe should not be left to luck or chance.

A PIVOTAL MOMENT IN RARE DISEASE HISTORY

- We have made major progress since the 2008/2009 soft RD legislations but much work remains
- End of major RD policy platforms – EUCERD and RD Action
- New European Parliament/Commission
- Rapidly changing landscape – political, economic, cultural, technological ethical/legal, environmental
- Europe has the opportunity to continue setting an example for other world regions and contribute to global effort to address needs for our community
- Without vigilance and constant effort, progress can easily be reversed in the coming decades

PART | 3

HOW WILL IT WORK?

The health of 30 million people living with rare diseases in Europe should not be left to luck or chance.

4 MAIN STEPS

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1

Establish a knowledge base by critically review existing knowledge and emerging issues through a literature review.

2

Identify the major **TRENDS and DRIVERS** that may shape the future of rare disease policy.

3

Build a number of **FUTURE SCENARIOS** in which certain trends are more or less prioritized

4

Translate the preferred scenarios into **POLICY RECOMMENDATIONS** that will lead to a better future for people living with a rare disease.

SHAPE THE FUTURE CONSIDERING EXISTING TOOLS

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1

KNOWLEDGE BASE

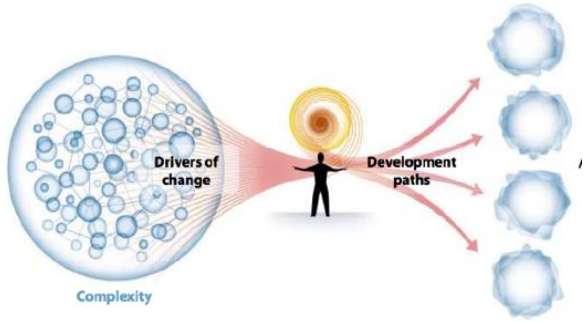
orphaNews

RD ACTION
DATA AND POLICIES FOR RARE DISEASES

LITERATURE REVIEW

2 3

HORIZON SCANNING AND SCENARIO BUILDING



ALL STAKEHOLDERS AND YOUNG CITIZENS

3

BACK-CASTING AND REFINING SCENARIOS

European Reference Networks

EU Health Policy Platform

EXISTING RESOURCES AND REALITIES

4

POLICY RECOMMENDATIONS

IMPACT

PART | 4

WHO IS INVOLVED?

Stakeholders are consulted at every step.

EURORDIS MEMBERS

INTERACTIVE WORKHSOP

SATURDAY 18 MAY 9.30 – 12.30

- Identify past, current and future trends that have an impact on your life and your advocacy work
- Help improve the process

Anna Kole,
Public Health Policy Advisor
EURORDIS

Giovanna Giuffrè,
ISINNOVA

PANEL OF EXPERTS

> Currently 125 (up to 250 members) of the rare disease community including:

- Patients and advocates
- National and regional authorities
- EU Policy makers
- Organisations involved in former EUCERD Joint Actions
- Healthcare professionals and managers
- Learned Societies
- European research networks and infrastructures
- Young advocates
- Researchers
- Pharmaceutical and other health-related industries
- Regulators (EMA committees)
- HTA bodies, reimbursement authorities
- International initiatives (RDI, NGO Committee for RD at UN, ICORD, IAPO)
- Other relevant NGOs

YOUNG CITIZENS

> Build capacity

- Online training
- Attendance to events

> Citizen conference

PARTNERS: KEY OPINION LEADERS

- The project brings together some of the most dedicated and influential actors, each representing a valuable stake in advancing the field of rare diseases.

EURORDIS RARE DISEASES EUROPE — Non-profit alliance of 826 rare disease patient organisations from 70 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe.

orphanet Inserm — Information portal for rare diseases and orphan drugs

Newcastle University — John Walton Muscular Dystrophy Research Center – translational research to bring diagnosis, care and therapy to people with neuromuscular disease

FONDAZIONE Telethon — Non-profit organization fostering research that leads to cures for rare genetic diseases

Innovation for sustainability ISINNOVA — Research institute supporting international, national and local public bodies for the analysis, design, implementation and the evaluation of sustainable policies

HELIOS Dr. Horst Schmidt Kliniken Wiesbaden — European reference network for Hereditary Metabolic Disorders — European Reference Network MetabERN European Reference Network for Hereditary Metabolic Disorders

SERVIZIO SANITARIO REGIONALE EMILIA - ROMAGNA — European reference network on Rare Bone Disorders — European Reference Network Bone disorders (ERN BOND)

Imperial College London — Centre of excellence within the Institute of Global Health Innovation

RESEARCH ADVISORY BOARD

- High level opinion leaders in foresight, innovative industries and health policy

Ruediger Krech
WHO

Ruxandra Draghia-Akli
Merck
(foremerly DG RTD)

Robert Madeline
FIPRA
(foremerly DG SANTE, CONNECT)

Milan Macek
Orphanet, Eurogentest, RD Connect

Cécile Wendling, AXA Group Public Affairs and Corporate Responsibility

Didier Schmitt, former advisor to EC on Foresight

Simon Kos
CMO Microsoft

Natacha Assopardi-Muscat
President EPHA

Kate Bushby
Former RD Action leader

Philine Wanke
Fraunhofer Institute

PART | 4

WHEN ARE THE RARE 2030 EVENTS?

Face to face opportunities for all stakeholders at different stages of the process.

KEY EVENTS

- 2019 EURORDIS MEMBERSHIP MEETING 15-18 MAY 2019 Bucharest
- Panel of Experts Workshop/EURORDIS CNA and CEF 7 Nov 2019 Brussels
- European Conference on Rare Diseases (ECRD) 15-16 May 2020 Stockholm
- Young Citizen Conference Summer 2020
- Regional Workshops aligned with upcoming EU presidencies May-Nov 2020
  - 2020 – Croatia, Germany
  - 2021 – Portugal, Slovenia
  - 2022 – France, Czech Republic
  - 2023 – Sweden, Spain
- Policy Conference @Parliament December 2020

PART | 5

HOW RARE 2030 WILL IMPACT POLICY

HOW TO RE-ENGAGE WITH THE EU?

Environment

- New EU Institutions (EP, EC)
- New EU budget (MFF)
- Ongoing legislative files
  - HTA joint assessment
  - Other access to therapies
- Assessment and possible revisions
  - Cross border Healthcare
  - Revision of key pharmaceutical legislation (Orphan Drugs, Paediatrics, more ?)

EURORDIS actions

- Stakeholder Network for Rare Diseases
- New Parliamentary Advocates for Rare Diseases
- **Foresight study Rare 2030 and recommendations end 2020**
- Towards a new Recommendation on Rare Diseases?

THE IMPACT OF RARE 2030

- Project supported by Parliament; Recommendations presented to Parliament and Commission in December 2020
- Support from Member States and key advocates at the national level can help lead to a potential new future legislation for RDs in Europe
- Europe has the opportunity to continue setting an example for other world regions and contribute to global efforts, but without vigilance and constant effort, progress can easily be reversed in the coming decades
- The health of 25-30 million people living with rare diseases in Europe should not be left to luck or chance.

PART | 6

MORE INFO ON RARE 2030

FOR MORE INFO

- Leaflet in meeting bags
- Current EURORDIS newsletter
- www.rare2030.eu
- Twitter: @rare2030
- Facebook: @rare2030

PART | 7

NEXT STEPS

AFTER ATTENDING THIS WORKSHOP PLEASE…

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- Give us your feedback via the evaluation from you will be emailed.
- Give your inputs on the factsheets once they are distributed.
- Vote on the prioritization of Trends in fall 2019
- Sign up for Rare Barometer Voices to vote on future scenarios.
- Come to the ECRD to help refine the scenarios from your point of view.

The health of 30 million people living with rare diseases in Europe should not be left to luck or chance.

Help shape and secure the healthy future of people living with rare diseases by supporting the Rare 2030 Foresight Study

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For additional information:

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