EURORDIS Vision of rare disease research in the next 15 years from the Rare 2030 perspective

Yann Le Cam (EURORDIS)

EURORDIS VISION OF RARE DISEASE RESEARCH IN THE NEXT 15 YEARS FROM THE RARE 2030 PERSPECTIVE

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The Rare 2030 Foresight Study

WHY

2008 Commission Communication & 2009 Council Recommendations

- *Now outdated: emergence of new contexts, trends, technologies; Build on first results*
- *Need for a new, updated framework; Recommended by the European Court of Auditors*

HOW

Foresight Study – 1st ever on rare diseases

- *Initiated by the European Parliament; Funded by the European Commission; Open Call*
- *Participatory and iterative methodology; large range of stakeholders (patients, doctors, KOLs).*

WHAT

Two years project, Eurordis & academic partners, ended March 2021

- *8 policy recommendations*

Rare2030
Foresight in Rare Disease Policy

Rare 2030 Partners

Rare 2030 brought together some of the most dedicated and influential actors, each representing a valuable stake in advancing the field of rare diseases.

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.

Information portal for rare diseases and orphan drugs

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

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

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

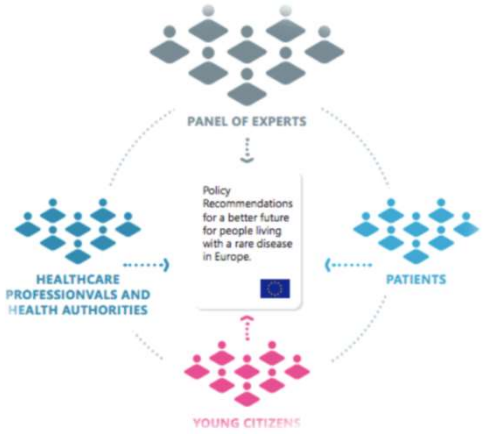
European reference network for Hereditary Metabolic Disorders

European reference network on Rare Bone Disorders

Centre of excellence within the Institute of Global Health Innovation

Rare 2030 Methodology

- 2-year extensive consultation
- Based on a Foresight methodology
- Iterative and inclusive process
  - *12 members of the Research Advisory Board*
  - *200+ members of a Panel of Experts*
  - *75 representatives of the European Reference Network (ERN) ecosystem*
  - *Consultation of patient groups and wide European survey to patients*
  - *Consensus Conference of Young Citizen – next Generation*



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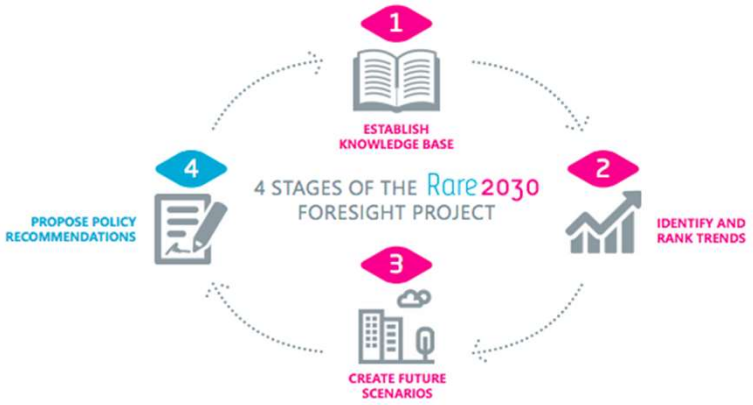
Rare 2030 Panel of Experts

200+ members coming from 38 different countries

Knowledge and expertise across different aspects of the field

- Policy makers (national, EU)
- Patient advocates
- ERN coordinators, hospital managers
- Health/social care representatives and providers
- Social care and social innovation experts
- Pharmaceutical industry representatives
- Regulatory experts
- Individual experts
- Researchers
- HTA bodies/reimbursement authoriti
- Health economists
- International initiatives representativ
- Journalists



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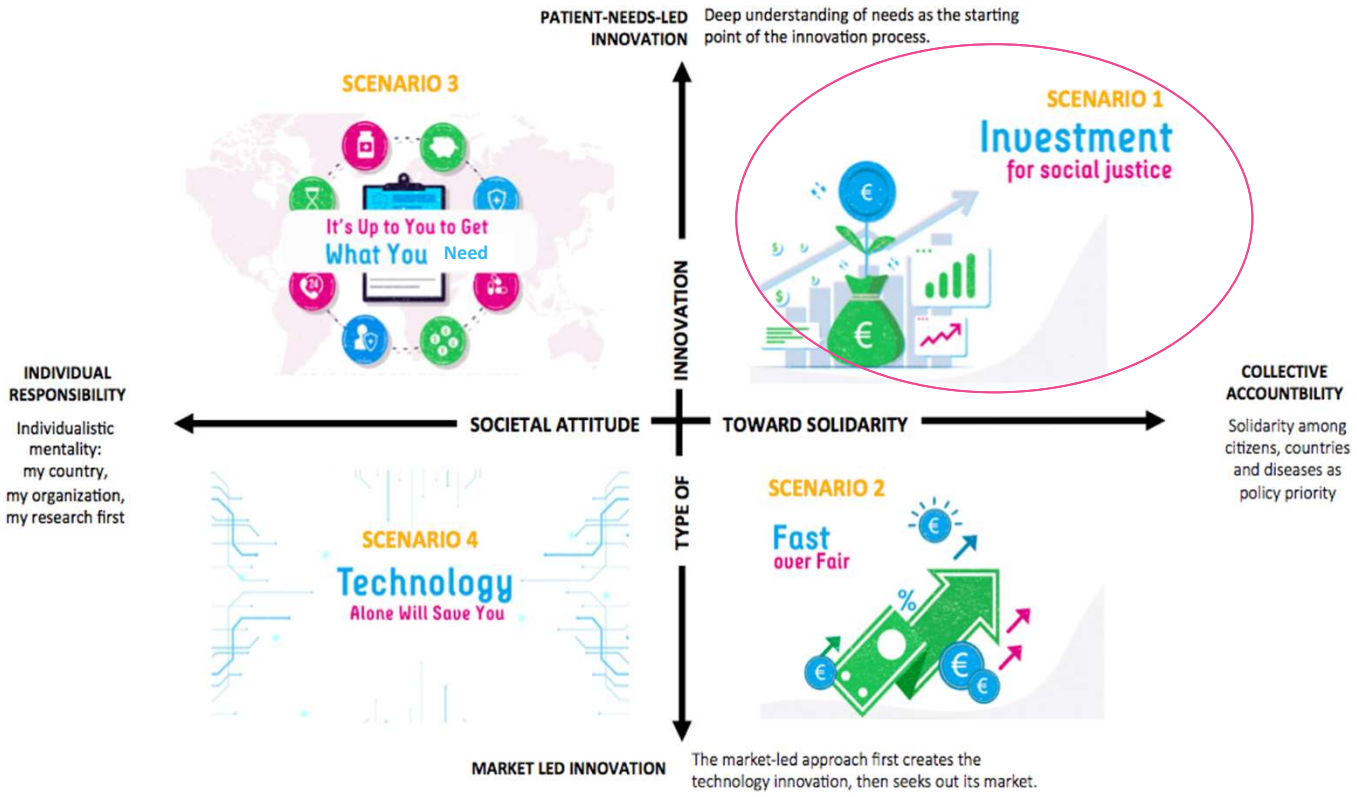
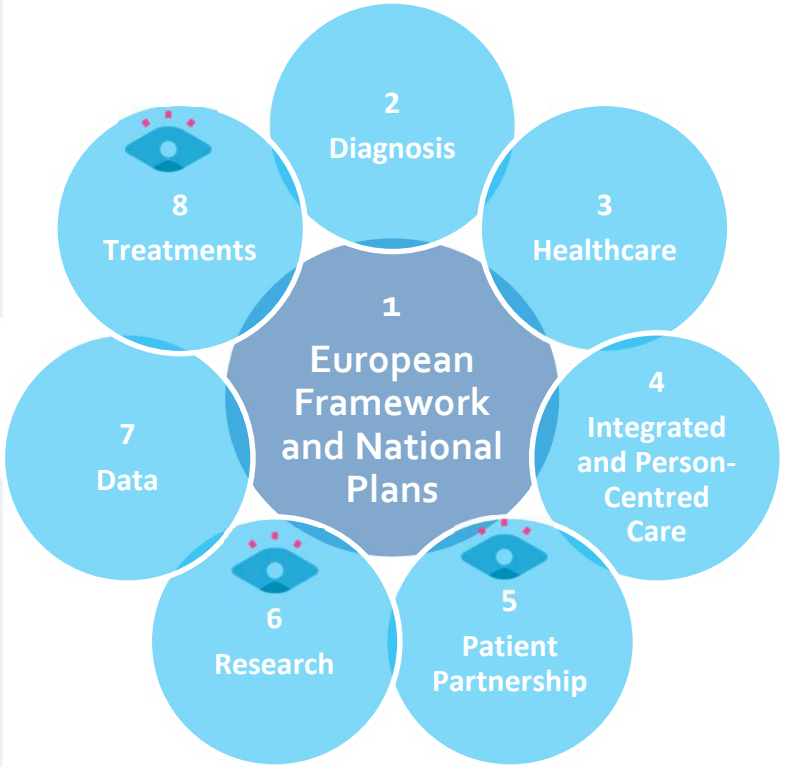
Outcomes – Rare 2030 Scenarios



Outcomes – Rare 2030 Policy Recommendations



- Propose updates, adaptations and replacements of the current strategy
- Provide **a roadmap to the preferred scenario**
- Fit for purpose to address unmet needs of the new generation
- With the support of Members States and European countries
- **Outcomes-based and goal-oriented**
- Contribute to the **UN Sustainable Development Goals**
- Aspire to **leave no one behind**

Recommendation 5 - Partnerships with Patients

Patient partnership can be defined as a mutual relationship between all stakeholders including patients where input from persons living with a rare disease or carers routinely and formally informs policy reflections and decisions.

Recommendation 5 - Partnerships with Patients

THE ISSUE

Patients are experts

GOAL AND VISION

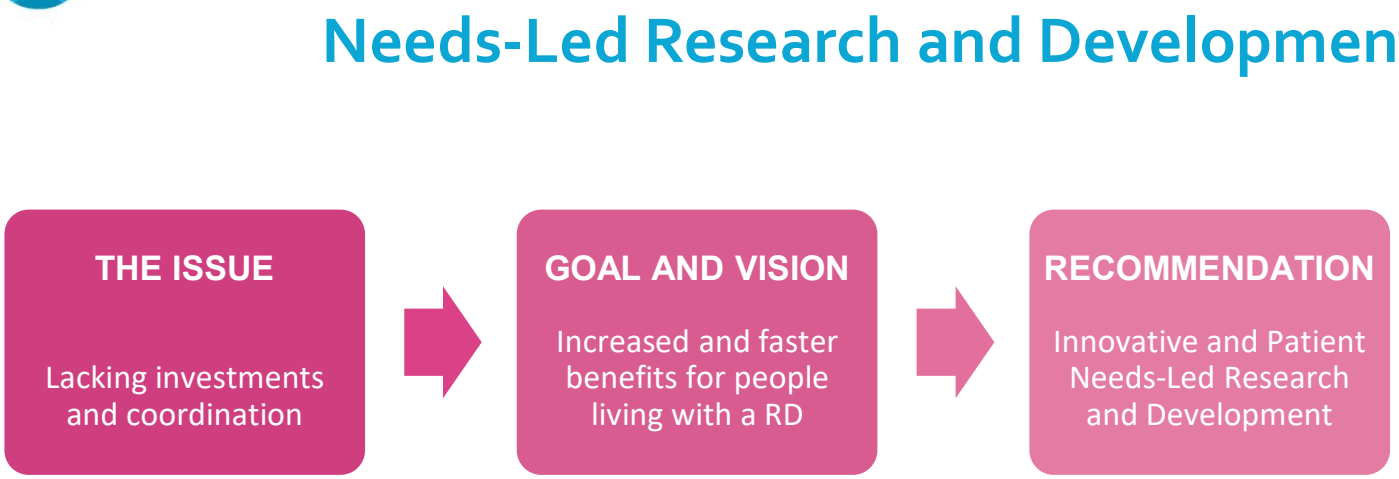
All patients and representatives can find their opportunity to provide expertise

RECOMMENDATION

Culture fostering meaningful patient partnerships

An overall culture encouraging **meaningful participation, engagement, involvement and leadership** of people living with a rare disease in both the public and private sectors.

Recommendation 6 – Innovative and Patient Needs-Led Research and Development



Basic, clinical, social and translational research maintained as a priority by **increasing the funds** for, **establishing greater incentives** in more **neglected areas**, and **supporting infrastructures** required to **expedite discovery and knowledge acquisition**

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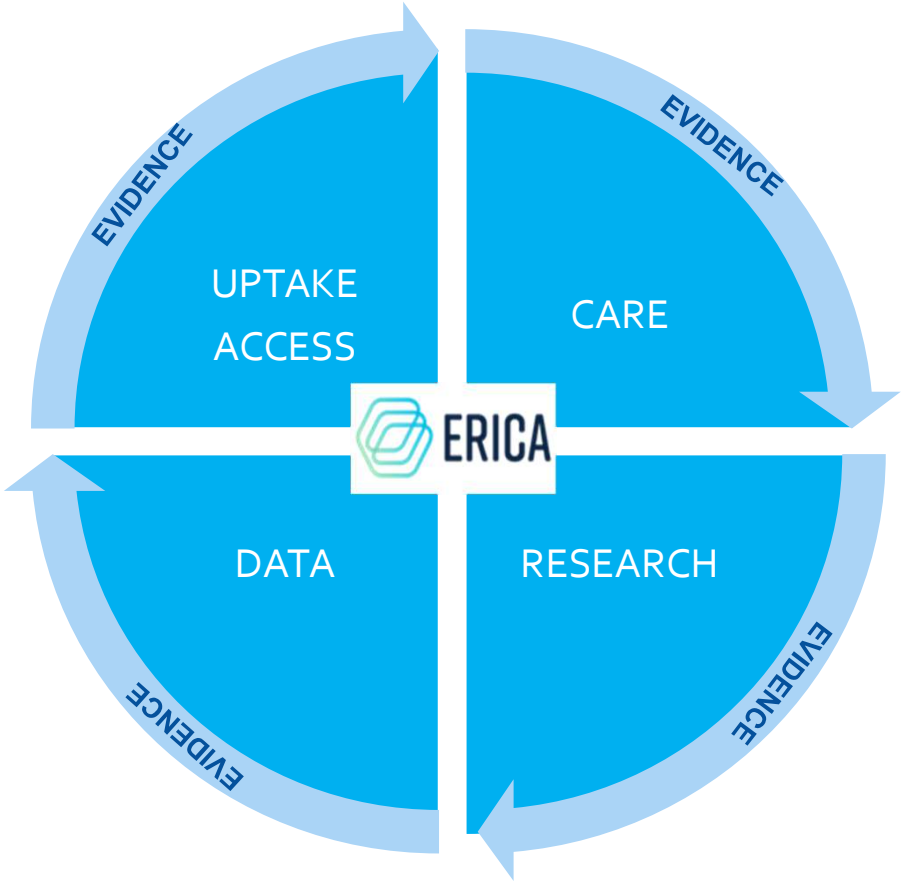
Recommendation 8 – Available, Accessible and Affordable Treatment

Encouraging a **continuum of evidence generation** and a **European ecosystem that attracts investments, fosters innovation** and addresses challenges of sustainability.

EU has the assets for a cohesive strategy (research, digital, health) to deliver transformational change: a holistic approach to research and care powered by data, generating innovation in EU of high added value

ERICA has the potential to reinforce the RD ecosystem between research, care and data



ERICA

ERICA should be the first step towards having Clinical Research Networks covering all RDs, embedded in the ERNs, conducting collaborative research for RD that complies with the standards required by regulatory and HTA bodies while enabling systematic data collection, especially for neglected areas

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RARE DISEASES CLINICAL RESEARCH NETWORK

Data Management and Coordinating Center (DMCC) comprised of 4 Cores, that provide different services to Network participants:

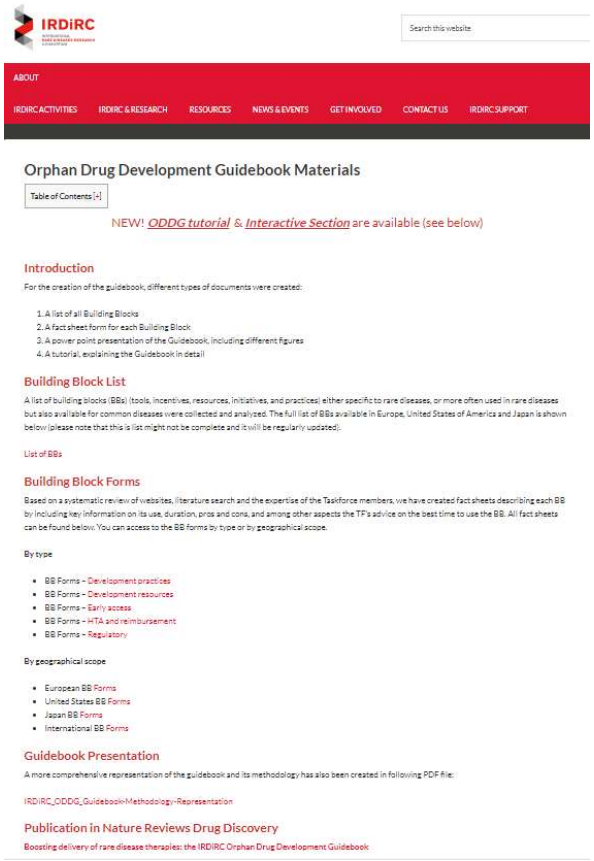
1. Data Management Core
2. Clinical Research Core
3. Engagement and Dissemination Core
4. Administrative Core

ERICA

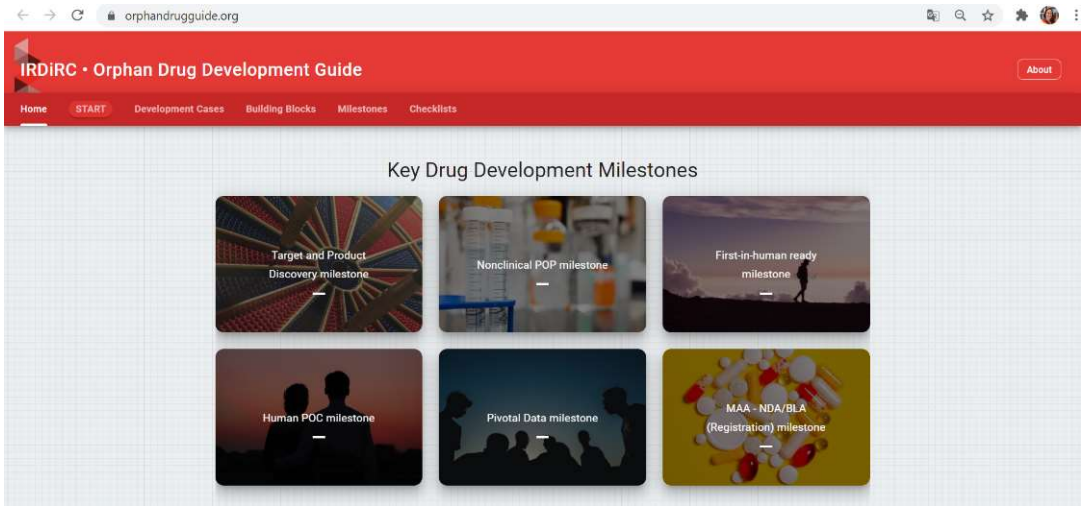
should support the creation of a common research infrastructure that would provide services in 4 domains

1. Data Management, including tools to share information within and outside the ERNs.
2. Clinical Research, including support and guidance to identify relevant patient-centered outcome measures, navigating the regulatory process or having a framework for PE (re-using PARADIGM PE toolbox)
3. Engagement and dissemination
4. Administrative support to oversee and coordinate the common research infrastructure

Tool already available
IRDiRC Orphan Drug Development Guidebook



Freely available on the IRDiRC website Including a video tutorial & an interactive website



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EURORDIS RARE DISEASES EUROPE

EU Clinical Research Networks for RDs supported by a strong infrastructure can position Europe as a world leader for research & innovation

People living with a rare disease no matter where they live can reach a network of expertise to access appropriate diagnosis, care and treatment

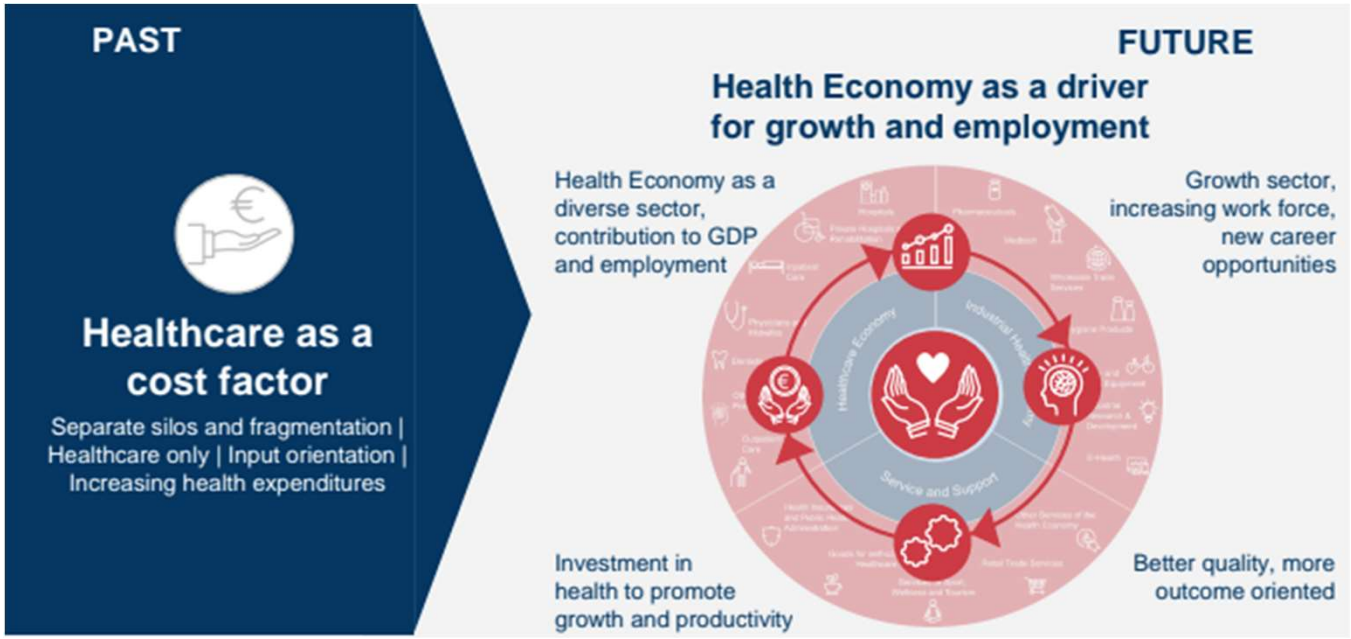
SDG Agenda 2030

UHC Implementation

GCN4RD

1. Accelerate and provide accurate diagnosis
2. Break down barriers accessing care
3. Strengthen healthcare systems

Making EU a world leader in rare disease research will help to attract investment, support innovation of high added value, create jobs locally, support trade balance



Credit: Pr. Dr. Dennis A. Ostwald, WiFOR Intitute"

Look ahead, think of the next 20 years and leverage on ERICA's initial funding to attract additional public and private investments to consolidate the EU CRNs

- Horizon Europe + HE partnership for RD + IHI
- EU Digital Strategy, EHDS, EU4Health
- Other PPP and industry investment, once ERNs have a framework for collaboration with industry