Advocating for change at national level: considering Rare 2030 in the Italian national plan

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The first National Plan: an advocacy act

- With the launch of EUROPLAN the Italian Federation started a process of co-building of the National Plan for Rare Diseases: Institutions and Associations together to build the Plan. 6 working group, one for each chapter.
- The National Plan was approved by the Ministry of Health at the end of 2013, for three years (it should have been renewed in 2016).
- From 2015 UNIAMO decided to monitoring the improvement of the acts inside the plan, publishing MONITORARE, the report of the condition of people living with a rare disease in Italy.

Question time!

1. Have you, as patient representative, been involved in the design and/or monitoring of your national plan?
2. In the next 3 years, do you expect to be involved in the design of a new national plan which could incorporate the RARE203 recommendations?

The Italian national plan – where we are

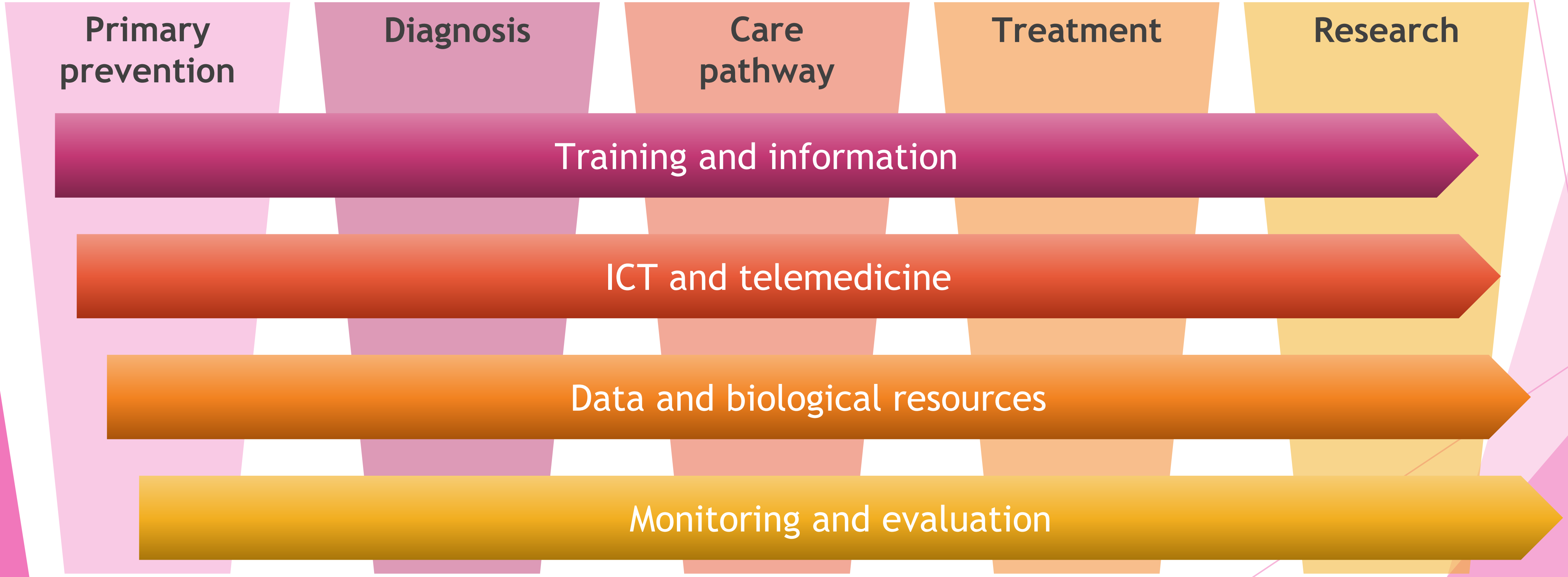
- Renewal of the plan expired in 2016
- Working group established since 2019 with strong delays due to COVID
- Working group led by Ministry of Health involving
  - ERNs
  - Patients (UNIAMO)
  - Policy makers (Ministry of Health, Regions)
  - Regulatory and public authorities (AIFA, AGENAS, Istituto Superiore di Sanità)
  - ORPHANET Italy
  - Fondazione Telethon
- A first, incomplete, **draft of the plan was discarded by the group in May 2021** and the process just restart

How and why was the draft rejected?

- Because of the pressure of the alliance of Patient representatives (UNIAMO), ERNs and Research funder (F. Telethon)
- To align it with the priorities of the National Recovery Plan
- To take into considerations the lessons learned during the COVID-19 crisis
- To incorporate a Vision!

The (new) structure of the plan

As recently agreed (May 2021), the plan should be structured in a matrix



Primary prevention

Diagnosis

Care pathway

Treatment

Research

Training and information

ICT and telemedicine

Data and biological resources

Monitoring and evaluation

Where does Rare 2030 recommendations fit?

Everywhere!

The backbone of the plan: a vision for the next years aligned with Rare 2030

- **Innovation driven by patient’s unmet needs** rather than financial gain

- Outcome-based goals:
    - **earlier faster and more accurate diagnosis;**
    - **an integrated and person-centred approach to care;**
    - **improved availability, accessibility, affordability and sustainability of medicinal products**

The example of research

How RARE 2030 Recommendations will feed the plan

From celebrating to planning

First Draft

- 5 pages on the «state of the art», presenting all the results achieved and some of the challenges
- 8 specific objectives (1 and 1/2 page)
- Indicators to monitor the achievement of the objectives

New structure of the chapter

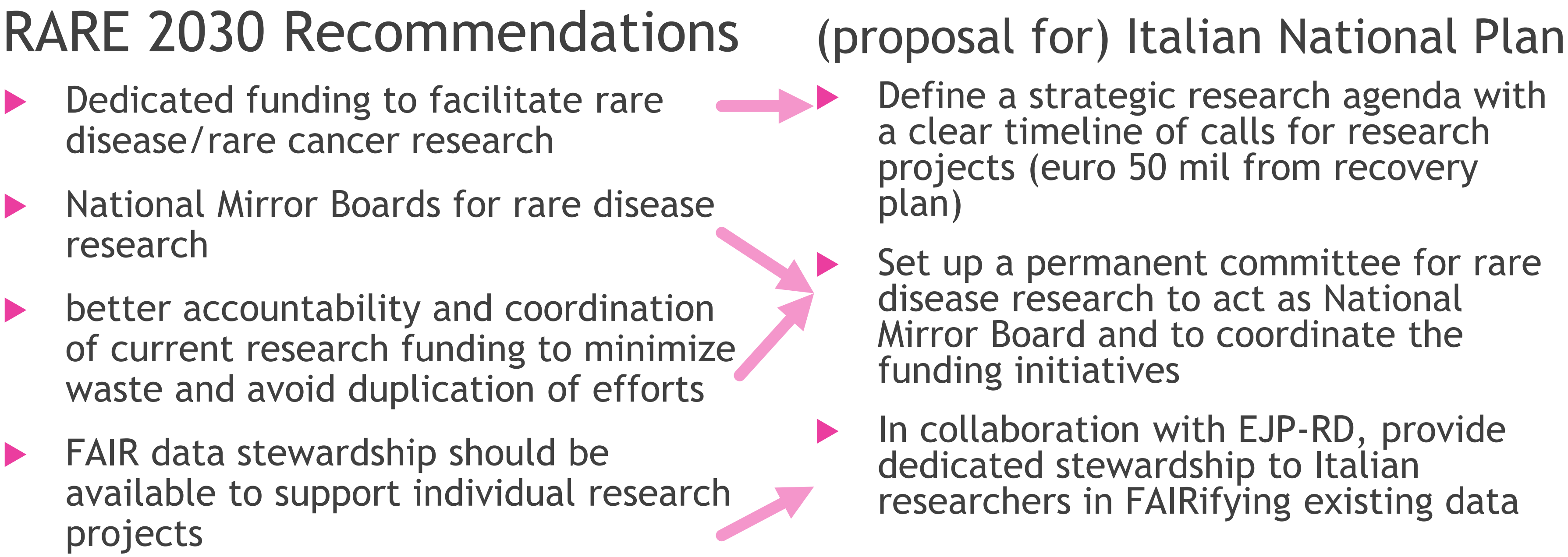
1. Title
2. Specific Objectives
3. Actions
4. Tools and methods
5. Responsible organization
6. Products/deliverables/expected results
7. Resources needed
8. Roadblocks / recommendations for political reforms
9. Timetable
10. Indicators

... Ideally all in maximum 5 pages!

From Rare 2030 Recommendations…

- To **optimize the use of limited resources** through strategic investments
- To **incentivize the sharing of data** and other research assets
- To **ensure greater incentives** for all stages and areas of research
- To **invest in pre-competitive infrastructures** to advance needs-led research
- To **enhance long-term multinational public-private research partnerships**
- To **promote and sustain international research collaboration**

…to Italian National Plan



RARE 2030 Recommendations

- Dedicated funding to facilitate rare disease/rare cancer research
- National Mirror Boards for rare disease research
- better accountability and coordination of current research funding to minimize waste and avoid duplication of efforts
- FAIR data stewardship should be available to support individual research projects

(proposal for) Italian National Plan

- Define a strategic research agenda with a clear timeline of calls for research projects (euro 50 mil from recovery plan)
- Set up a permanent committee for rare disease research to act as National Mirror Board and to coordinate the funding initiatives
- In collaboration with EJP-RD, provide dedicated stewardship to Italian researchers in FAIRifying existing data

What’s next

- The national working group will prepare a new draft by end of June
- The new plan should be launched in parallel with the National Recovery Plan and will leverage some of the resources of that plan

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FONDAZIONE
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Thank you

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