Rare2030

Foresight in Rare Disease Policy

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National Plans and Strategies in Europe

The status quo in 2021

EMM 13.5.21

TODAY'S PRESENTATION:

- The concept of a national plan or strategy
- Source of information I am presenting
- Our status quo in 2021
- How does this link to our Rare 2030 recommendations?

Recommendation 1

Long-term, integrated European and National Plans and Strategies

ORIGINS OF EUROPE'S NATIONAL PLANS/STRATEGIES FOR RARE DISEASES

- Council Recommendation on an action in th rare diseases 2009 – made Recommendatio Member States
- MS asked to "elaborate and adopt a plan o soon as possible, preferably by the end of latest, aimed at guiding and structuring rele in the field of rare diseases **within the fra their health and social systems**"
- Link : [Council Recommendation]()

Then vs. now

- In 2009, only 5 EU MS had adopted a NP/NS for RD: France, Bulgaria, Spain, Greece and Portugal

24 of our 27 EU Member States have now adopted a NP or NS for RD at one time or another

Resource on the State of the Art of RD activities in Europe

orphanet

Inserm

2014 REPORT ON THE STATE OF THE ART OF RARE DISEASE ACTIVITIES IN EUROPE

PART I: OVERVIEW OF RARE DISEASE ACTIVITIES IN EUROPE

EUCERD Joint Action

Newcastle University

RD ACTION
DATA AND POLICIES FOR RARE DISEASES

Rare2030
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COUNTRY DATA COLLECTION

- We established Data Contributing Committees (DCCs) for each EU MS. Intended to provide a Multistakeholder perspective on each country's activities in RD
- Composed (in most cases) of the following:
  - official representative in the EUCERD/CEGRD
  - Orphanet national team representative
  - Patient Alliance representative
- Duties are to try to provide data in a semi-structured format on a wide range of RD-related issues

WHAT DO WE ASK COUNTRIES TO PROVIDE?

- Data sought on many topics deemed important to gauge national approaches to diagnosis, treatment, care (broadly defined) of RD
- http://www.rd-action.eu/wp-content/uploads/2015/10/Final-State-of-the-Art-question-bank/pdf

1. Introduction

2. Question Mapping

A. National Plans and Strategies for Rare Disease
B. Rare Disease Registration
C. Definition of a Rare Disease
D. Neonatal Screening
E. Genetic Testing
F. Centres of Expertise
G. European Reference Networks (ERNs)
H. National Alliances of Patient Organisations and Patient Representations
I. National Helplines for Rare Disease
J. Activities of Orphanet/Alternative RD-specific information systems
K. Official Information Centres for Rare Diseases
L. Clinical Practice Guidelines (CPGs)
M. Training and Education
N. Rare Disease Events
O. Existence of Rare Disease research programmes/projects in your country
P. Participation in E-Rare and International Research Initiatives.
Q. Orphan Medicinal Products (OMPs)
R. Social Services and Social Integration
S. Other

WHY WE

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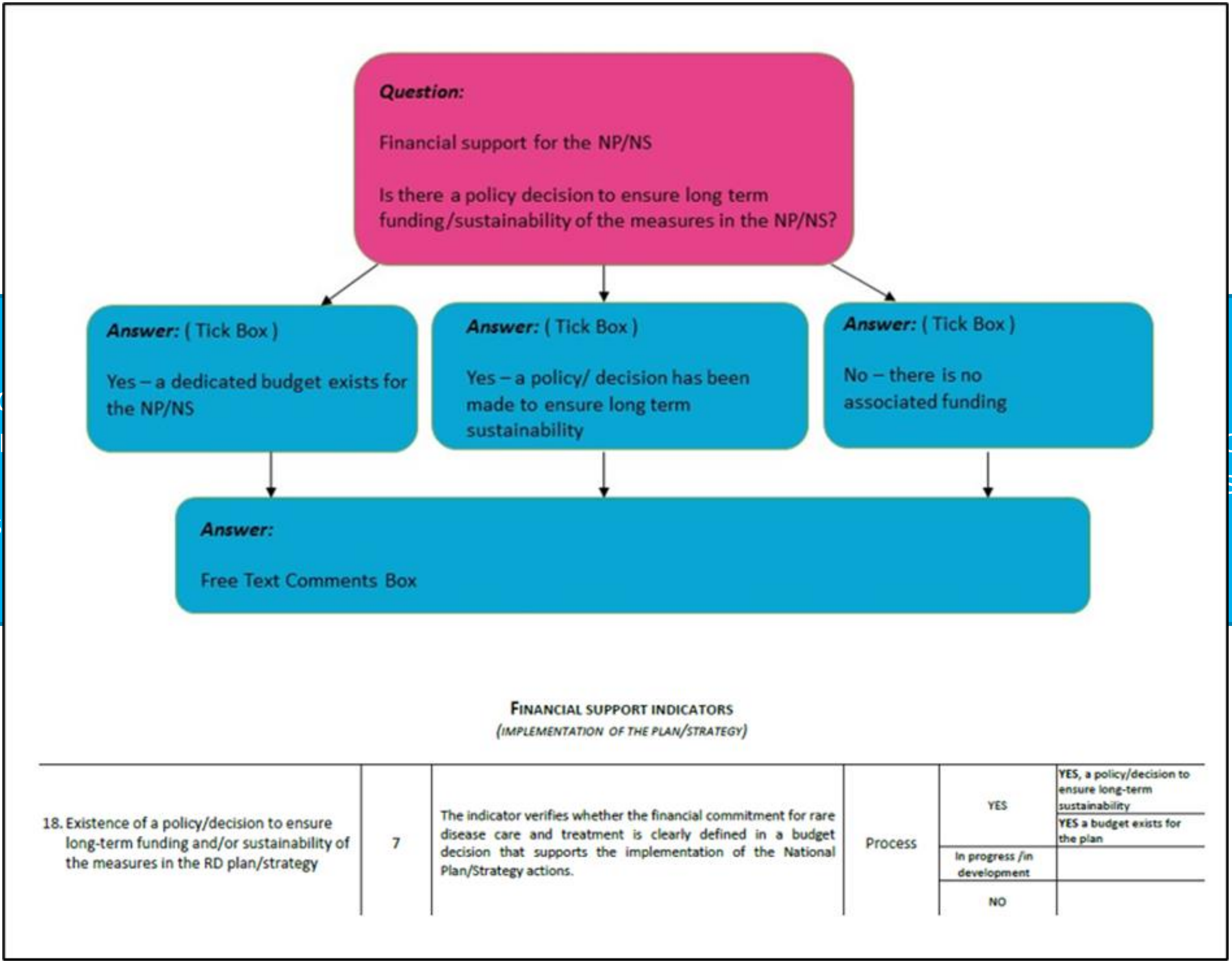


Question:

Financial support for the NP/NS

Is there a policy decision to ensure long term funding/sustainability of the measures in the NP/NS?

Answer: (Tick Box)

Yes – a dedicated budget exists for the NP/NS

Answer: (Tick Box)

Yes – a policy/ decision has been made to ensure long term sustainability

Answer: (Tick Box)

No – there is no associated funding

Answer:

Free Text Comments Box

FINANCIAL SUPPORT INDICATORS

(IMPLEMENTATION OF THE PLAN/STRATEGY)

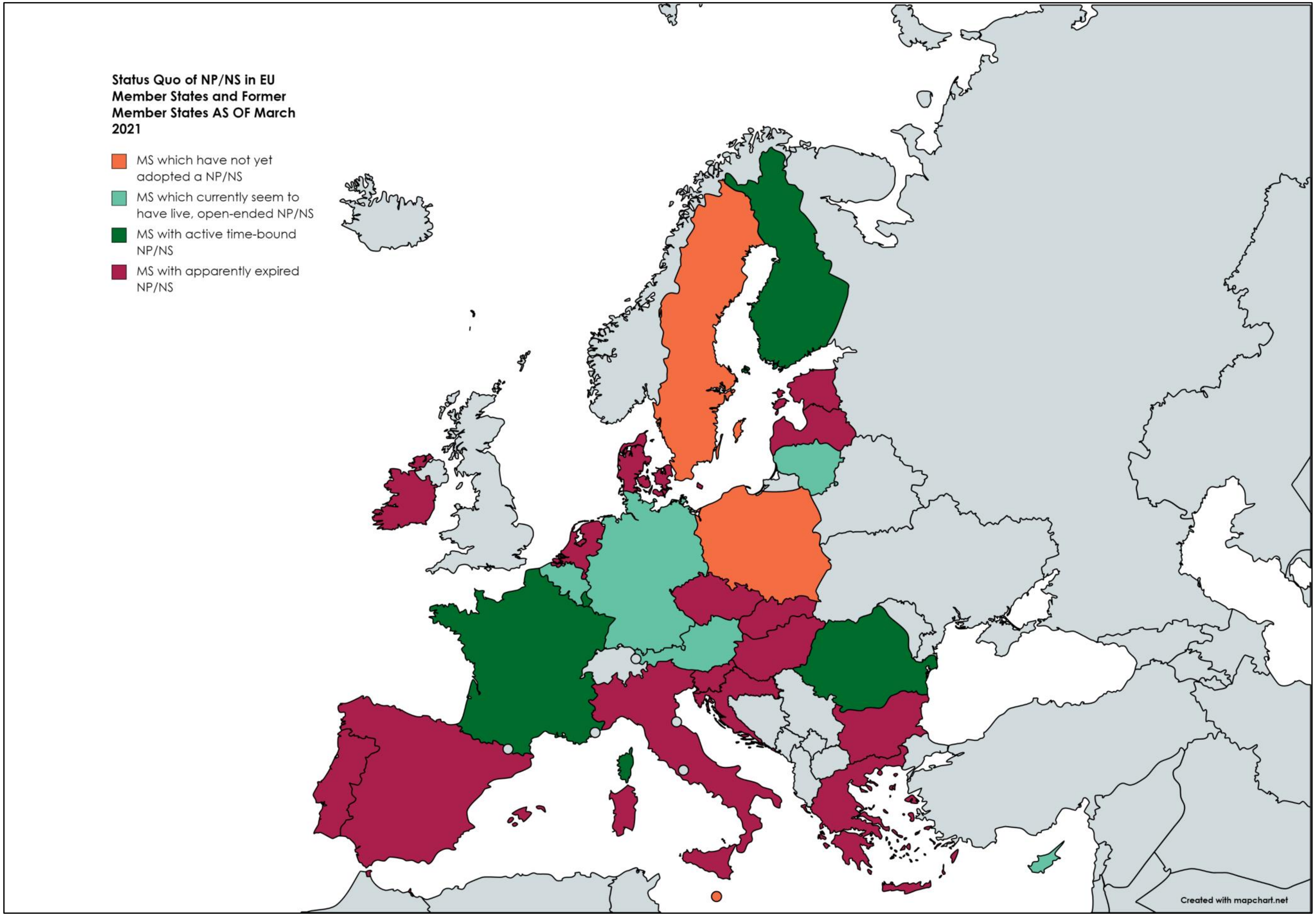
18. Existence of a policy/decision to ensure long-term funding and/or sustainability of the measures in the RD plan/strategy	7	The indicator verifies whether the financial commitment for rare disease care and treatment is clearly defined in a budget decision that supports the implementation of the National Plan/Strategy actions.	Process	YES	YES, a policy/decision to ensure long-term sustainability
					YES a budget exists for the plan
				In progress /in development	
				NO	

the EUCERD
ommendation
s on Core
ndicators

CAVEATS!

- Survey wording can be open to interpretation – not good! Based on the EUCERD Recs
- Sometimes difficult to tell who really has input and to address that
- The data is everything – this is our basis, and if not quite accurate, difficult to know unless you tell me ☺
- Data request on NP/NS made in Oct 2020. https://link.springer.com/article/10.1007/s12687-021-00525-4
- A full review requested in February and March 2021, asking for status quo as of March 1st
- NB only EU MS at present: plan under the new State of the Art Resource is to open this up significantly
- A few countries have yet to respond to latest update, so some info may change a little
  - Do not yet have a complete response from Poland or Greece, and not complete for Estonia:

! The fact that 24 of the 27 EU MS adopted a NP/NS at some point does not mean all have active NP/NS today!



Status Quo of NP/NS in EU Member States and Former Member States AS OF March 2021

- 🟧 MS which have not yet adopted a NP/NS
- 🟩 MS which currently seem to have live, open-ended NP/NS
- 🟩 MS with active time-bound NP/NS
- 🟥 MS with apparently expired NP/NS

Status of National Plans/ National Strategies	Country	More details
Ongoing NP/NS (i.e. not time-bound) = 5	**Austria, Belgium, Cyprus, Lithuania** and **Germany**	Austria was time-bound then moved to open-ended with timebound *actions* Germany on 3rd term of 1st plan
Apparently expired Plans/Strategies = 15	**Bulgaria** (2013), **Denmark** (apparently: 2019), **Estonia** (2017), **Greece** (2012), **Ireland** (2018), **Italy** (2016), **Netherlands** (2018): *At end of 2020*: **Croatia**; **Czech Republic; Hungary; Latvia; Portugal; Slovak Republic; Slovenia; Spain (8)**	• Czech Rep. had NS 2010-20, with 3 action plans therein: new NS 2021-30 in prep. • Italy's 2nd NP has been in prep for a while, COVID impact • Hungary - New NP expected soon • Netherlands – New policy in discussion • Latvia: PH Guidelines expected soon for 2021-2027 include some actions to further improve RD care (not accepted yet but in final stage). Discussions are still ongoing on the need for a new NPRD • Slovak Rep - NP 2020-2030 ready and approval expected • Slovenia – 2012-2020 to be replaced by 2021-2030 expected adoption soon

Time-bound Standalone NP/NS which are active as of March 1st 2021 = 4	**Finland** **France** **Luxembourg** **Romania**	Onto 2nd NP: 2019-2023 Onto 3rd NP 2018-2022 2018-2022 Adopted the NPRD and included it in the National Public Health Strategy 2014 –2020. New National PH Strategy 2021-2027 maintains RD as a health priority and the new national plan for rare diseases is a strategic document providing a comprehensive policy framework and includes specific measures and actions in the context of pandemic periods.
MS which have not yet adopted an NP/NS =3	**Malta** **Poland** **Sweden**	• An internal action plan serves as a basis for a number of specific actions taken by the Ministry -e.g setting up of the Rare Disease Register • Draft resolution of the Council of Ministers on the adoption of the document Plan for Rare Diseases out for pre-consultation in March • Swedish healthcare system is decentralized and currently has a national structure operated in cooperation between all the regions. Is centered around 25 National Program Areas (NPAs), one for RD.

WHAT DOES ALL THIS SUGGEST? WHAT QUESTIONS DOES THIS STATUS QUO RAISE?

- Is a NP better than a NS?
- Is it ‘better’ to be ongoing or time-bound? If specific actions within an ongoing policy are time-defined, is this really the key thing?
- What is the ideal time for these? Is 10 years too long?
- What do we think about NP/NS somehow nested within broader policies? E.g. NP for RD part of the national health development plan 2009-2020. Had an action plan within that, covering 2015-17. RO has new NP nested within the Nat. PH Strategy.
- Many countries are ‘preparing a new one’ or have 1 drafted, ready for approval. How long will this take? Esp with COVID?

WHY WE NEED THE RARE 2030 RECOMMENDATIONS

- In 2009, only 5 EU MS had adopted a NP/NS for RD: France, Bulgaria, Spain, Greece and Portugal

24 of our 27 EU Member States have now adopted a NP or NS for RD at one time or another

Only 9 of the 24 MS seem to have active NP/NS as of March 2021

DIGGING DEEPER: HOW FAR ARE THESE FUNCTIONING??

Of the 18 still 'active' in October 2020 (5 ongoing, 13 T.B still active):

- 10 reported that a 'dedicated advisory body/expert advisory group' of some sort was in place to oversee the implementation or evaluation of the Plan, and that this body was Multistakeholder **&** fully functioning (meeting regularly)
- 6 Reported that such an advisory body existed, was Multistakeholder, and was *functioning* (but doesn't have meetings)
- 2 Reported a body which was 'partially functioning but does not include all stakeholders'

- 14 of the MS reported **no dedicated funding behind the NP/NS**…

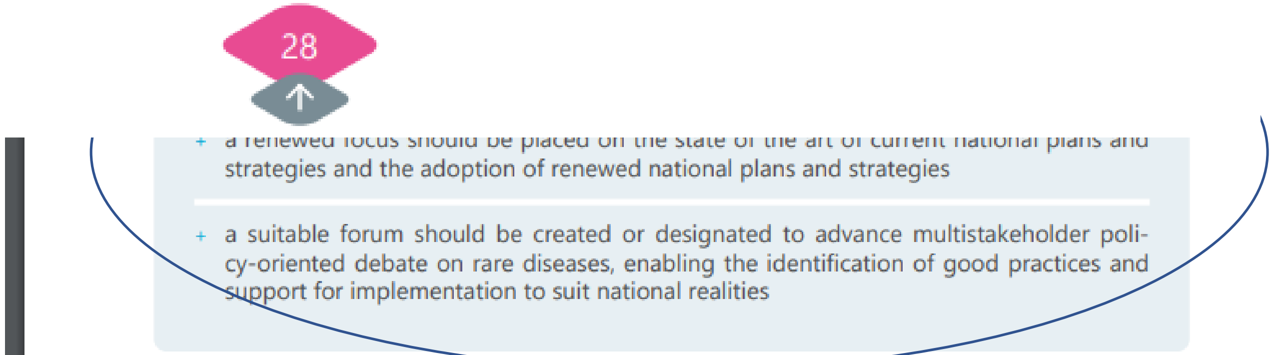
KEY RARE 2030 RECOMMENDATIONS:

RARE 2030
RECOMMENDATION 1

LONG-TERM, INTEGRATED EUROPEAN AND NATIONAL PLANS AND STRATEGIES

A European policy framework for rare diseases defined by societal responsibility, equity and driven by

- European and national plans and strategies should be sustained on a long-term basis, with adequate funding, and should be monitored by the appropriate authorities and key opinion leaders in the field
- a renewed focus should be placed on the state of the art of current national plans and strategies and the adoption of renewed national plans and strategies
- a suitable forum should be created or designated to advance multistakeholder policy-oriented debate on rare diseases, enabling the identification of good practices and support for implementation to suit national realities



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AT THE NATIONAL LEVEL:

The elaboration, implementation, evaluation and renewal of robust and effective national plans and strategies for rare diseases must once again be embraced as a key policy priority.

- The European Union shall consider an updated request to Member States in connection with national plans and strategies for rare diseases, structured within the frameworks of the health and social systems
- The aforementioned EU-level multistakeholder group tasked with overseeing policy challenges and opportunities for the full breadth of rare disease/rare cancer issues should ensure a key focus on revitalising the national plans and strategies agenda
- Support should be provided from the European level in terms of updated KPIs for national plans/strategies and the identification and dissemination of good practices and solutions to shared challenges
- National plans and strategies should be robustly evaluated and – in the case of time-bound policies – renewed or replaced by national authorities in a timely and transparent manner. National authorities should ensure intersectoral collaboration in the elaboration, evaluation and implementation of national frameworks for rare diseases/rare cancers, encompassing also social and holistic actions alongside the medical and research angles
- National authorities should dedicate designated funding to implement the national plans and their constituent activities (which should include SMART objectives, wherever possible)
- The integration of rare cancers (both in adults and paediatric cancers) in national cancer control plans should be fostered, with relevant synergies with national rare disease plans
- National authorities should avoid subsuming ‘rare diseases’ into broader health strategies which reduce addressing their specificities and their strategic prioritisation and; however, where relevant strategies exist (for instance for genomics or cancer) appropriate links to the rare disease field should be ensured
- National authorities should consider the applicability of rare diseases to the UN Sustainable Development Goals and Universal Health Coverage debates and incorporate this to their strategic agendas
- Countries should create a Mirror Group on rare disease research, to interact with the European Joint Programme on Rare Diseases Policy Board on research matters, and integrate this to their national plans and strategies for rare diseases/rare cancers
- Each renewed national cancer control plan should include relevant and specific measures for both paediatric cancers and rare cancers in adults, addressing the issues of research and access to adequate care, in synergy with national plans or strategies for rare diseases where relevant.
- By 2025, all countries should have a ‘live’ national plan or strategy for rare diseases, with a dedicated multistakeholder oversight body and an annual budget separate from the wider health and social system