Advocacy WORKSHOP

ENGAGING WITH POLICY MAKERS

EMM 2025 – Riga 23-24 May 2025

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EXPECTATIONS

Introduction to the session

What is advocacy

- What for ?
- What types?
- Where?

Analyse the issue

- What's the issue
- Prioritise !
- Resources

Design the solution – the 4 Ps

- Goal and objectives
- The four "Ps"
- EU and national

Jump into action

- Build your message
- Power of data
- Power of stories
- Meeting policymakers
- Oral and written communication
- Leveraging media

"Healing," Happloinsufficiency
MEF2C, Poland

I. What is advocacy?

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Advocacy: What is it ?

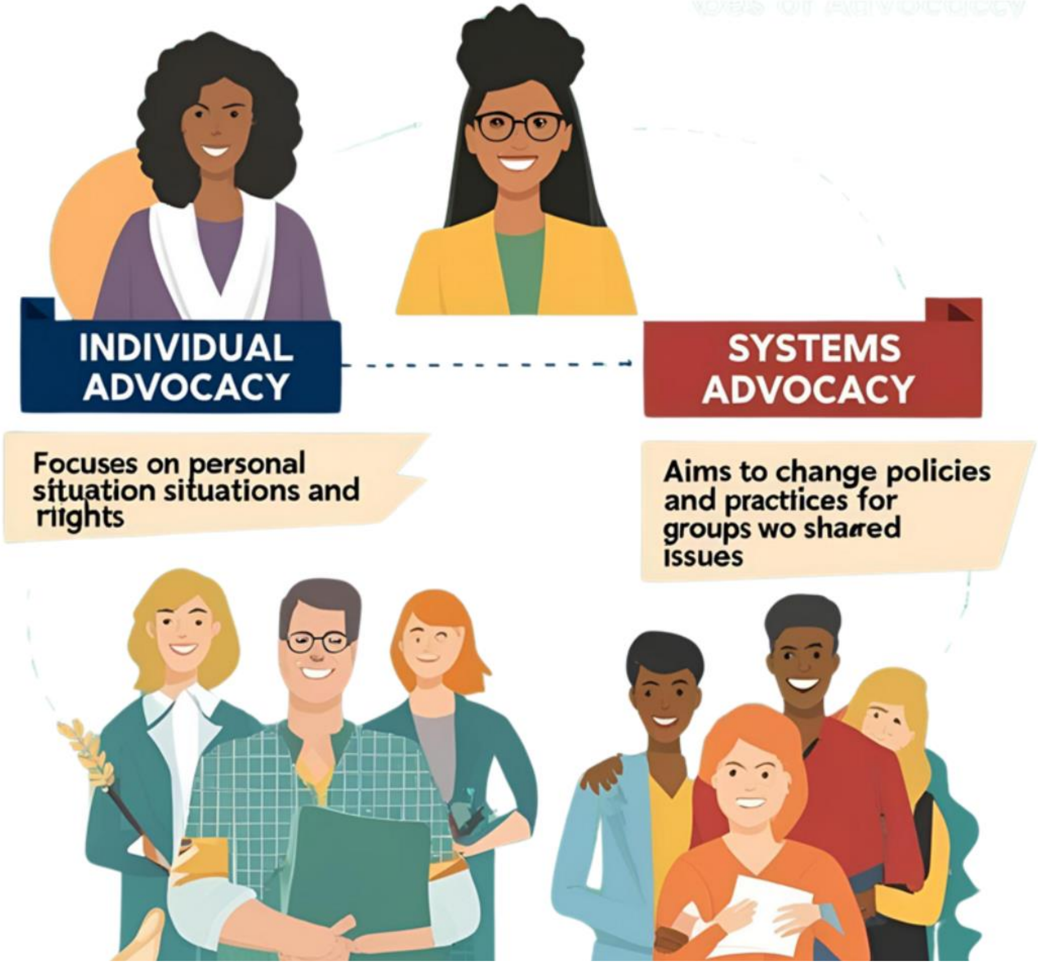
Ensuring everyone in society can:

- Have their voices heard & is listened
- Have their opinions considered in decision-making processes
- Defend their rights …

Influencing an action :

- Policy, legislation, administrative acts…

Bringing about institutional change



Advocacy is a planned, deliberate, and sustained effort to advance an agenda for change

What does it mean to be influential in health policy ?

A universally accepted definition for a patient organisation does not exist. The European Medicines Agency (EMA) has developed a definition which is widely used:

> *"Patients' organisations are defined as not-for profit organisations which are patient focused, and whereby patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies"*

Being influential is different from simply being an influencer!

- Influence is key for public health leaders to drive change and improve community health.
- Influential organizations are seen as credible and can impact policy and stakeholder relationships.
- Developing influence takes time but is a valuable investment.
- What types of influential actions can we define?
- Organising events is *not* always the best solution.

Advocacy: Where?

What level of governance, what constituency

Local, regional

National

EU-level

International

"Healing," Happloinsufficiency
MEF2C, Poland

II. Analyse the issue

Analyse your issue

Identify and analyse the **issues** you want to address

Which **rights** are you aiming to guarantee? Is there a violation of your rights that need to be anticipated, stopped, reversed?

> People living with rare diseases face great barriers to having their disabilities adequately assessed and recognised.
>
> National disability assessment processes must be improved to ensure that their human rights are fully respected, protected and fulfilled.

Raquel Castro,
EURORDIS Social Policy and Initiatives Director

https://www.dcaf.ch/sites/default/files/imce/Women's%20Guide%20Curriculum/InclusiveSecurity_Curriculum_Series_SSR_MOD10.pdf

Examples of key advocacy issues

Research Policy and Activities

Development and Access to Therapies

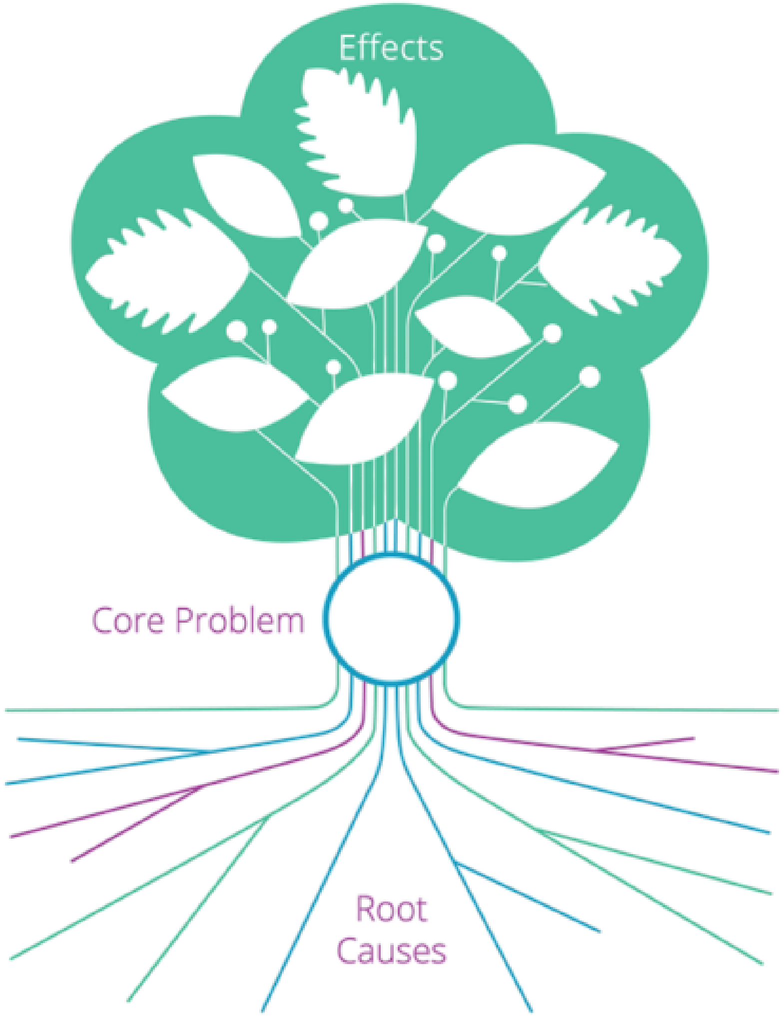
Data and Digital Health

Development and Access to Diagnosis

Healthcare Policy and Services

Social Policy and Services

The problem tree



Prioritise!

Once you’ve delved into a problem’s causes and effects, determine:

1. **The priorities within that issue**
2. **What creates value and what destroys value”** – the core of the analysis
3. **What opportunities exist for change**

What **creates value** for the rare disease community you represent?

What could **destroy value** that is already there ? Or **jeopardise rights** that are already there?

Tips to prioritise

Impact

Added value

- Relevance
- Empowerment
- Creation of value ?

Coherence

- Consistency with your vision, mission, strategy?
- Fits with your values?

Capacity

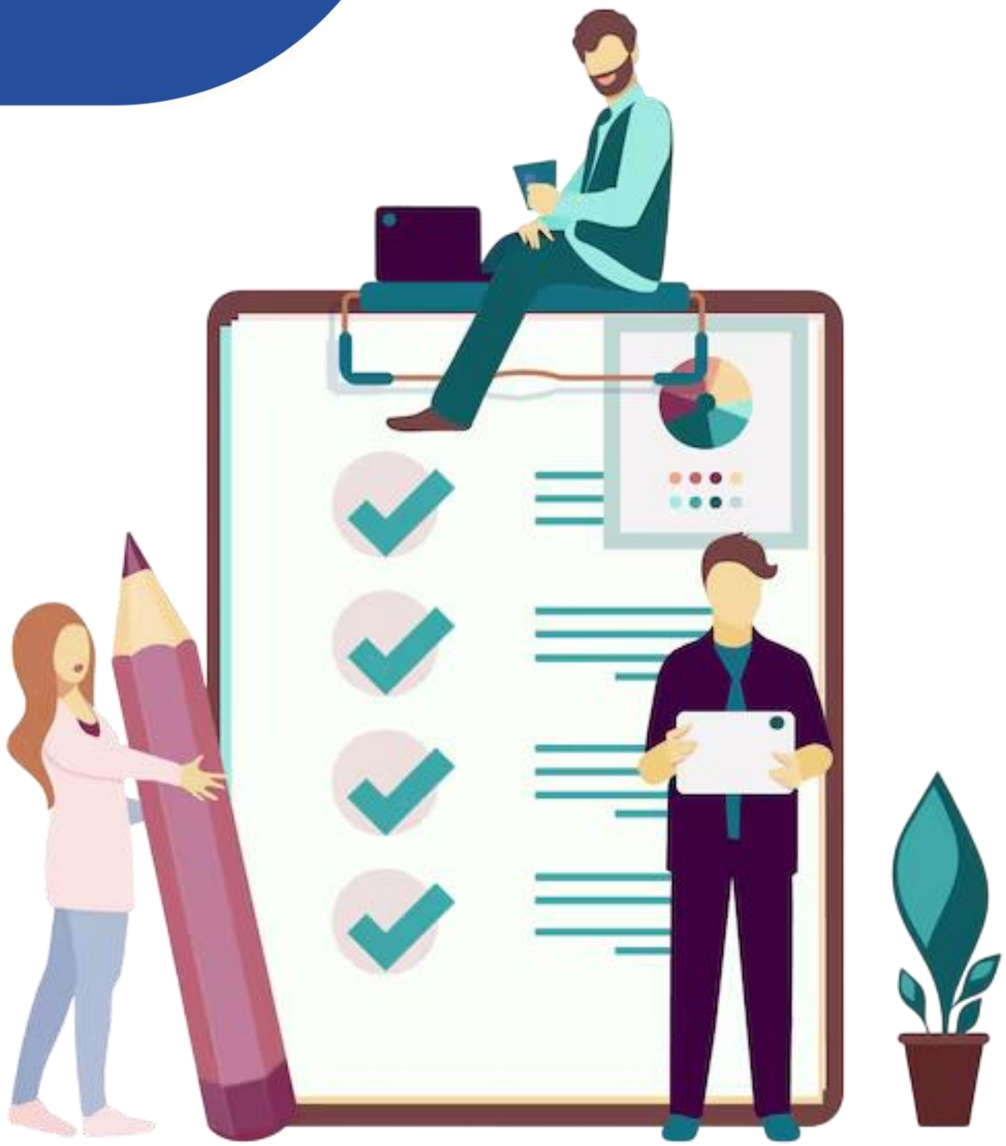
Effectiveness

- Window of opportunity?
- Do you have something to contribute?
- Someone else could it better?

Efficacy

- In-house expertise
- Capacity
- Resources to deliver?
- Environmental scan?

Resources!



ASSESS YOUR RESOURCES AS A PATIENT ORGANISATION!

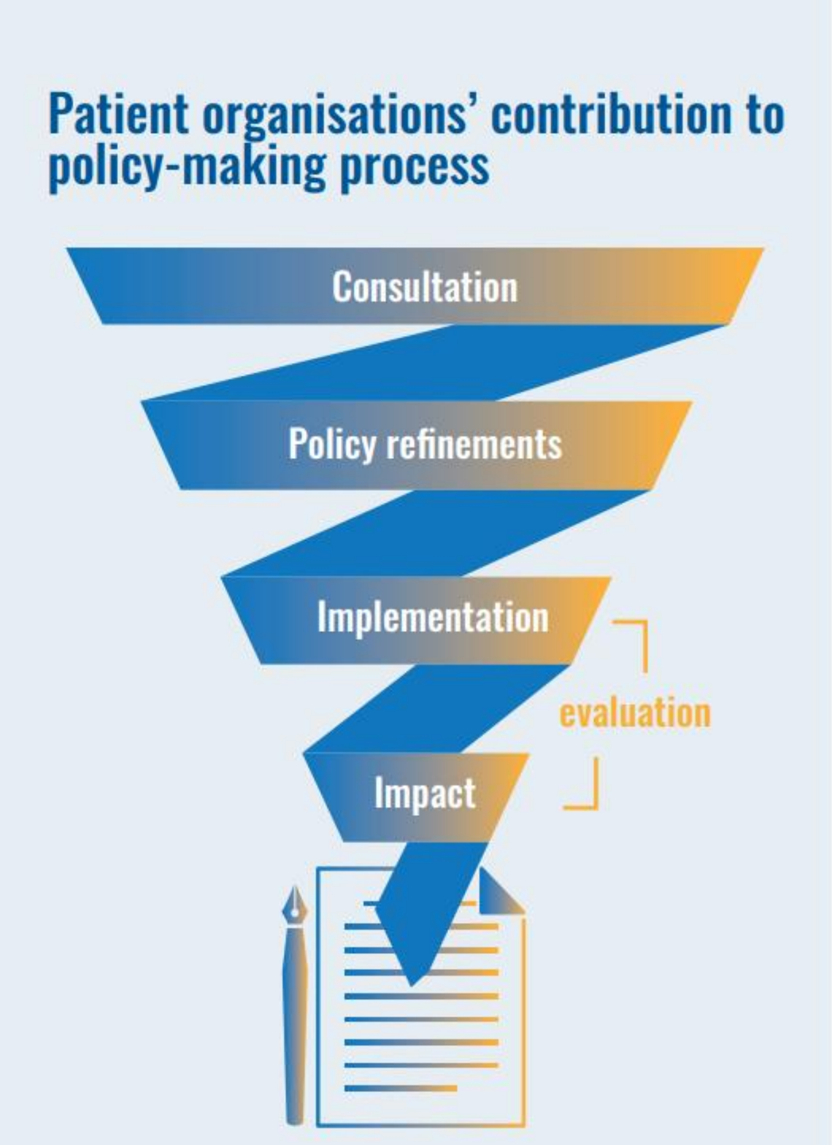
Measure your resources as patient's organisations

Pan-European disease-specific organisations (in our case NAs and EFs) are another type of umbrella organisations, gathering national patient organisations active in the same disease-area.

- They enable the patient community to compare situations between countries, to have a better overview of the treatments and care available across Europe, and to transfer good practices whenever possible.
- They are also in a better place to monitor legislative developments at EU level.

> *"The people have the right and duty to participate individually and collectively in the planning and implementation of their healthcare."*

Alma Ata Declaration, Principle IV (1978, WHO)



Measure your resources as patient's organisations (POs)

To assess our level of
CREDIBILITY / RECOGNITION

- **What is our message?**
- **Who will deliver our message?**
- **Who needs to hear it?**

- Patient organisations often **lack formal recognition** as credible stakeholders in health and non-health policy debates.
- Patients' experiences are frequently **dismissed** as biased or too narrowly focused.
- Declining public health and research funding pushes patient organisations to **seek private funding**.
- Awareness of the importance of **transparency and ethical conduct** has grown within the patient community.
- Patient organisations have become more **professional** over time.

III. Designing the solution

"Healing," Happloinsufficiency MEF2C, Poland

What do you want to change?

What is the **overall change?**

Equitable and timely access to treatments for all people living with a rare disease across the EU.

What are the **pre-conditions?**

- **Political and regulatory prioritisation** of rare diseases and orphan drugs at EU level within the Pharmaceutical Strategy for Europe.
- **Incentive frameworks** that balance innovation with affordability and access, including predictable pathways for orphan drug approval and reimbursement.
- **Cross-border collaboration** on diagnostics, clinical trials, registries, and access (especially via ERNs – European Reference Networks).
- **Robust data infrastructure**, including rare disease registries and real-world evidence collection.
- **Active involvement of patients** in policy design, in regulatory pathways, HTA (Health Technology Assessment), and research funding decisions.

What is **your contribution?**

- **Monitor and influence EU legislation, including revisions to the Orphan Drug Regulation**
- Aggregate and amplify the patient voice through **consultations, testimony, public campaigns, media outreach**…
- Build **alliances** …

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https://www.bond.org.uk/resources/theory-of-change-for-advocacy-and-campaigns/

Set goal & objectives

Once you've determined the change you want to make & how you can contribute to it

What is your **GOAL ?**

= what impact you intend to have?

- Increased number of authorised orphan drugs with pan-EU availability and access.

What are your **OBJECTIVES**

= what outcomes do you expect?

- Revised pharma Regulation includes improved access incentives and clearer market rules for Orphan Medicinal Products (OMP).
- EU-level guidance on pricing, reimbursement, and joint procurement of orphan drugs.

What **ACTIVITIES** do you need to put in place?

What **INDICATORS** to measure success?

The four “Ps”

Engaging with Policymaking: The 4 Ps

POLITICS
The political environment

POLICY
The substance of policies

PROCESS
The decision-making process

PEOPLE
The key stakeholders

The four “Ps”

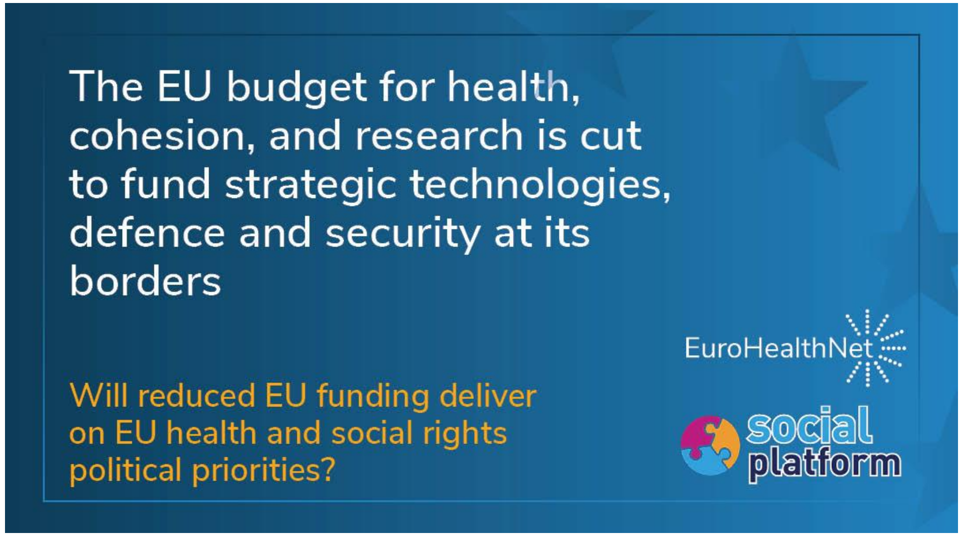
POLITICS

The political environment: the power dynamics, the geopolitical developments, the current political climate.

Understand the context: agenda, timing, sensitivities. Identify threats and opportunities


CNBC TV18

'WILL ANNOUNCE MAJOR TARIFF ON PHARMA SOON'


The EU budget for health, cohesion, and research is cut to fund strategic technologies, defence and security at its borders

Will reduced EU funding deliver on EU health and social rights political priorities?

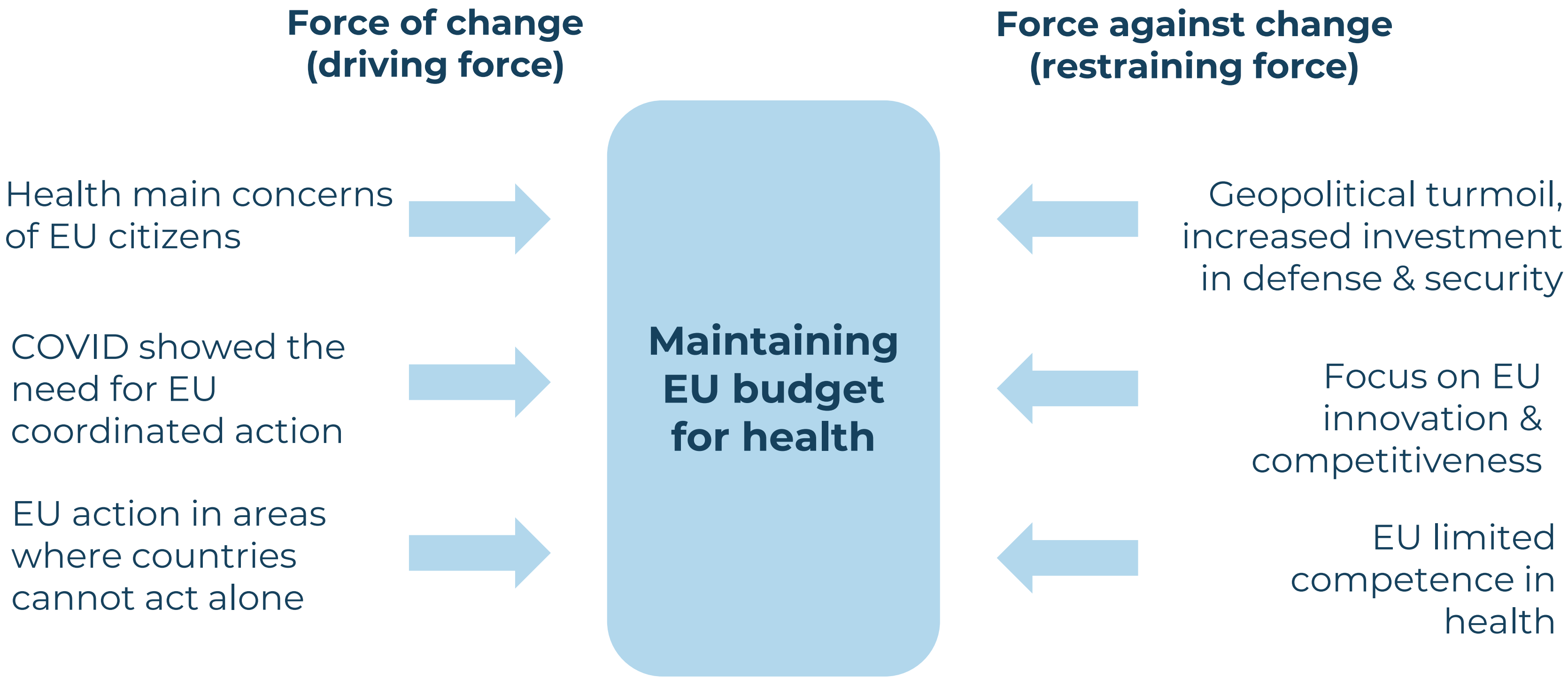
EuroHealthNet

social platform

The four “Ps”

POLITICS

“Forcefield analysis”



Force of change (driving force)

Health main concerns of EU citizens

COVID showed the need for EU coordinated action

EU action in areas where countries cannot act alone

Maintaining EU budget for health

Force against change (restraining force)

Geopolitical turmoil, increased investment in defense & security

Focus on EU innovation & competitiveness

EU limited competence in health

The four "Ps"

POLICY

The content of the laws, regulations, and strategies being developed.

Know the **substance** – what's being proposed, debated, or revised – and how it impacts your interests.

Which **key policy is affecting you** / your family/ your community?

What new policy and legislation is coming up?
Or there is **no policy** and **I need one**?

Revision of the EU pharmaceutical framework to ensure equitable access to rare disease therapies

Date: April 13, 2021

→

KEY PRINCIPLES FOR NEWBORN SCREENING

A EURORDIS Position paper

January 2021

eurordis.org/newbornscreening

EURORDIS.ORG

The four “Ps”

PROCESS

The formal and informal steps by which policy is made. The **decision-making process**.

Engage at the right time. Identify consultation periods, committee reviews, decision points …

Knowing the decision-making process is like having a **map** and a **clock.**

It shows :
→ **where to go**
→ **who to speak to**
→ **when**

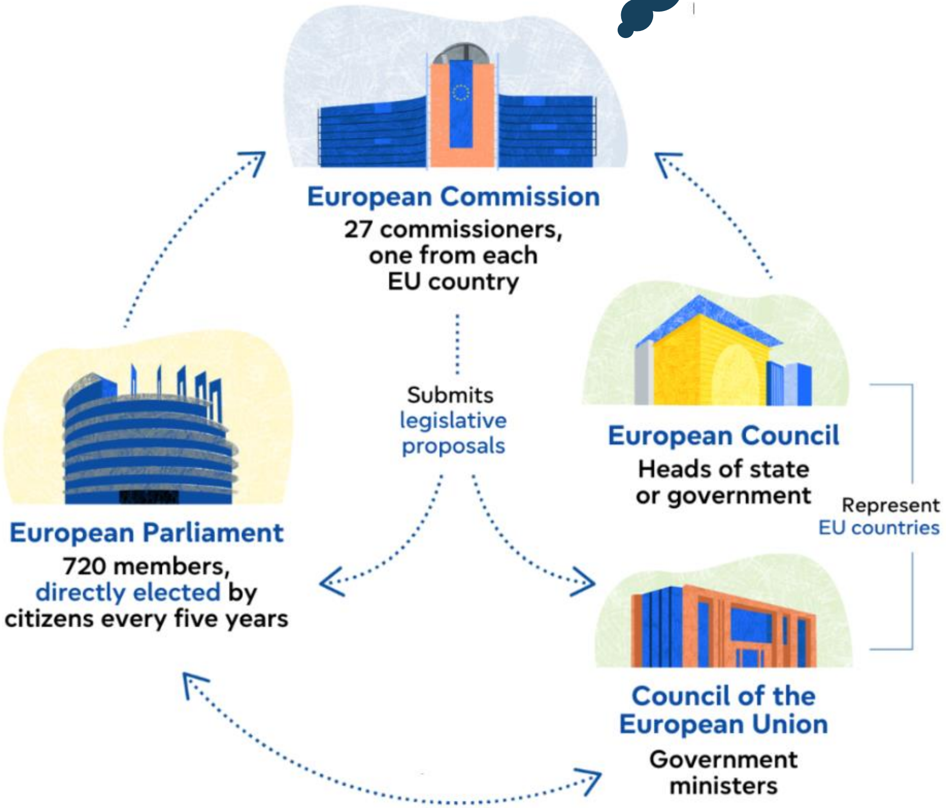
Without it, even the most passionate and well-reasoned advocacy risks missing its mark!

The four “Ps”

What process does affect your policy ?

PROCESS

- Formal or informal inputs - Public consultations?
- Space for patients voice ? Patient seating at the table? E.g. National plan committee?
- Committee review vs plenary
- Public vs closed debate
- Don’t miss the deadline to table amendments!



European Commission
27 commissioners, one from each EU country

Submits legislative proposals

European Parliament
720 members, directly elected by citizens every five years

European Council
Heads of state or government

Represent EU countries

Council of the European Union
Government ministers

The four “Ps”

PROCESS

Knowing the **decision-making process** is critical to:

- **target efforts effectively**
- **time interventions appropriately**, and
- **influence the right people** at the right moments.

EXAMPLE

If you meet with the European Commission while they are preparing a proposal, it will be much easier to get your points in that if you meet with policy makers once the proposal is approved within the EC and being discussed in the so-called Trialogues with the other EU institutions.

The four “Ps”

PEOPLE

The **key influencers**: politicians, civil servants, advisors, stakeholders …

Build relationships. Know who decides, who influences, and who can be your …ally or advocate.

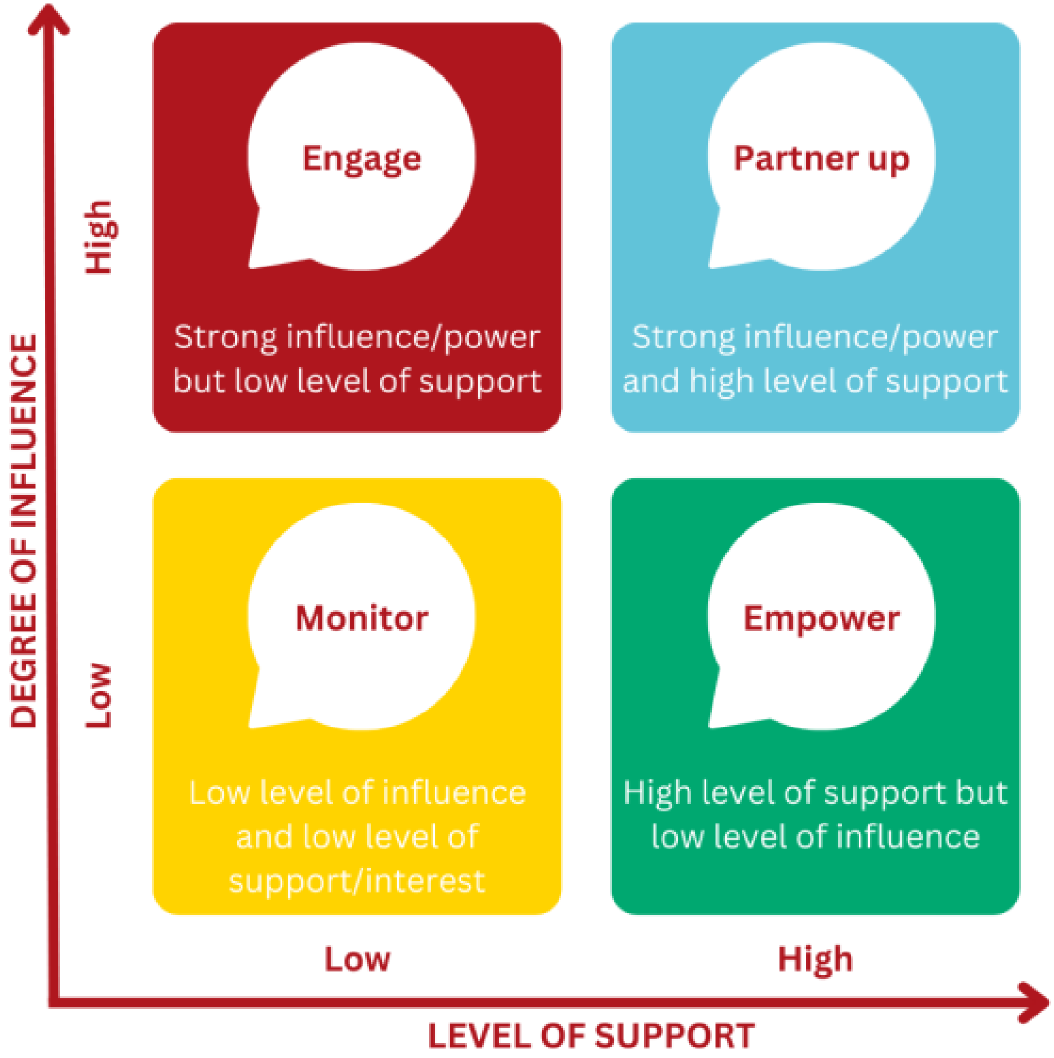
Advocacy hinges on **influencing actors** outside your organisation to take action.

- **Who Are the Key Policymakers? Who are the actual decision makers?**
  - European Level
  - National Level
- **Are all MEPs and European Commission representatives equally important?**

https://oeil.secure.europarl.europa.eu/oeil/en/procedure-file?reference=2023/0132(COD)#section2

The four “Ps” – stakeholder mapping

PEOPLE



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[18] Also called Venn Diagram, Systems Mapping or Chapatti Diagram

[19] Tang, A. (n.d.). Power Mapping and Analysis. The Commons: Social Change library. Retrieved from: https://commonslibrary.org/guide-power-mapping-and-analysis/

The four Ps – allies & opponents

PEOPLE

Who Are Your Allies?

Who Are Your Opponents or Challenges?

The four Ps – in practice

EXAMPLE

Campaign on the patients' voting rights

Objective: block the majority position in Council concerning patients' voting rights removal in EMA committees before it goes to Trilogue, hence asking to keep the EC proposal.

Solution: A plan derived from that involved:

- **forcefield analysis** (see next slide)
- **consider the process** (revision of the EU pharma legislation, EU ordinary legislative procedure)
- **consider the timing** (vote in Council Working Party few days after);
- **identify decision makers** (Member States, specifically MoH/Med agencies + health attaches);
- **create the coalition EPF + National Alliances etc.**
- **develop the message and recommendations** (asks): keep the EC proposal wordings in Articles xxx of the Regulation;

Engaging at EU & national level

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What is European Added Value for RDs ?

- '[the value resulting from an EU intervention which is additional to the value that would have been otherwise created by Member State action alone.']

- The specificities of rare diseases - limited number of patients and scarcity of relevant knowledge and expertise - single them out as a distinctive domain of very high European added-value.

- European cooperation can help to ensure that scarce **knowledge can be shared** and **resources combined** as efficiently as possible, in order to tackle rare diseases effectively across the EU as a whole

(Communication COM(2008) 679 final of the European Commission).

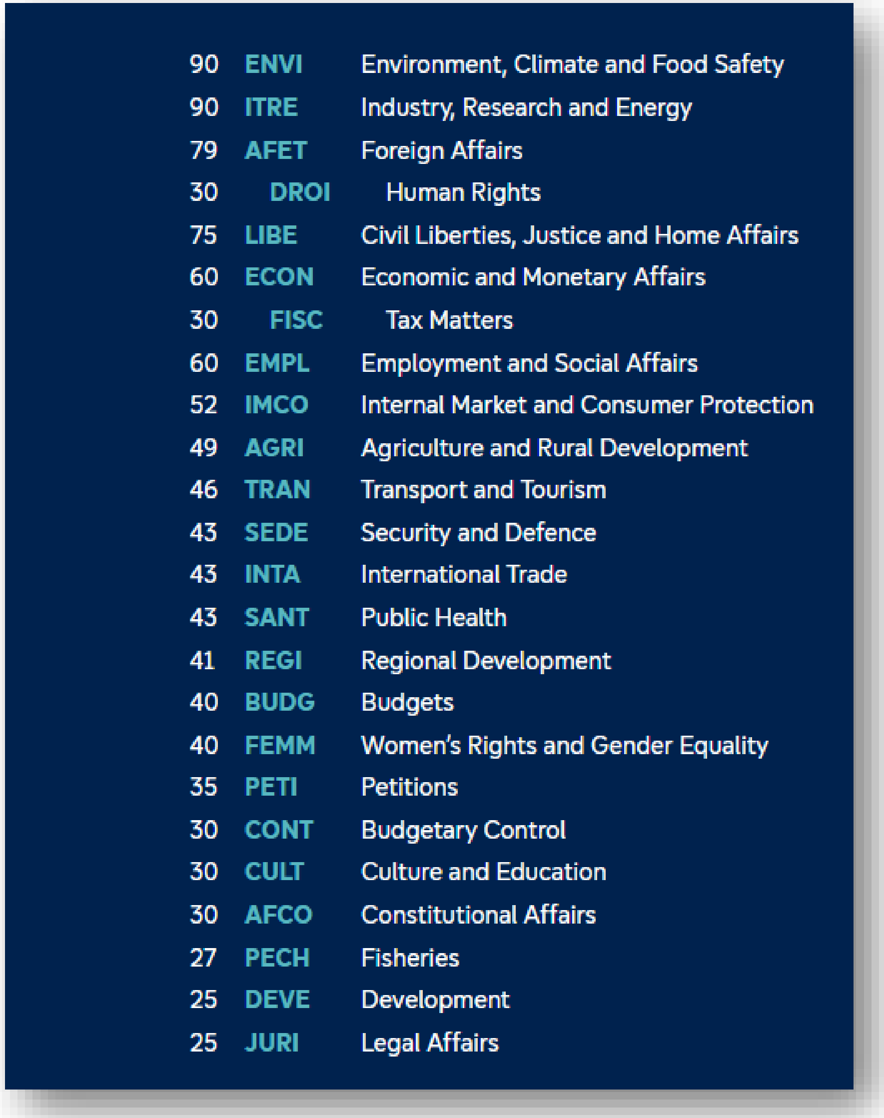
Advocating at EU level – engaging with the European Parliament

The European Parliament has 3 main roles:

- **Legislative**
- **Supervisory**
- **Budgetary**

How is the work of the European Parliament organised?

- **20 Committees** – prepare legislation
- **Plenary** – pass legislation



90	ENVI	Environment, Climate and Food Safety
90	ITRE	Industry, Research and Energy
79	AFET	Foreign Affairs
30	DROI	Human Rights
75	LIBE	Civil Liberties, Justice and Home Affairs
60	ECON	Economic and Monetary Affairs
30	FISC	Tax Matters
60	EMPL	Employment and Social Affairs
52	IMCO	Internal Market and Consumer Protection
49	AGRI	Agriculture and Rural Development
46	TRAN	Transport and Tourism
43	SEDE	Security and Defence
43	INTA	International Trade
43	SANT	Public Health
41	REGI	Regional Development
40	BUDG	Budgets
40	FEMM	Women's Rights and Gender Equality
35	PETI	Petitions
30	CONT	Budgetary Control
30	CULT	Culture and Education
30	AFCO	Constitutional Affairs
27	PECH	Fisheries
25	DEVE	Development
25	JURI	Legal Affairs

Advocating at EU level – engaging with the European Parliament

May I ask an MEP to present **new legislation**?

What may I ask to my MEP to do for supporting PLWRDs ?

Can I ask to my MEP to present an **initiative** on rare diseases in the SANT committee ?

Own Initiative Reports

Parliamentary Questions: scrutiny power !

- **Written questions**
- **Oral questions with debate in plenary**
- **Qs for Question Time**

Advocating at EU level – engaging with the European Parliament

May I influence my MEP to take into account Rare Diseases in the next **EU budget**?

Yes, and it is very important to do so !

- Negotiations on the MFF, the EU Multiannual Financial Programme, start this year, budget to cover 2028-2035.
- In the MFF 2021-2027 we had, for the first time, the **EU4Health Programme** with a budget of €5,3 billion (then reduced to €4,4).
- Influence your MEPs for the renewing of the EU4Health Programme in the future MFF.

LEGAL TOOLKIT FOR PATIENT ADVOCATES

Report on the patients' participation in decision-making and law-making processes

- A comprehensive document on the possibility of patients' participation in decision-making and law-making processes in selected countries of CEE prepared by The Healthcare Education Institute in association with Boehringer Ingelheim.
- Countries covered in the project: Austria, Czech Republic, Hungary, Poland, Romania, and Slovakia.
- Research focused on three main areas:
    1. Specific legal framework
    2. General administrative procedures
    3. Alternative ways
- Our primary objective is **to inspire patient organizations in various countries by showcasing best practices and concrete examples from others**.

LEGAL TOOLKIT
FOR PATIENT ADVOCATES

Report on the patients' participation in decision-making and law-making processes

Patient organizations can often operate effectively within the existing system even before dedicated solutions are introduced.

SPECIFIC LEGAL FRAMEWORK:

Different types of councils and committees composed of patient organization representatives

1. **Councils created by the Minister of Health**:

Permanent advisory bodies to the Minister of Health, composed of representatives of patient organizations.

Responsible, in particular, for giving opinions on draft legal acts and other government documents, initiating changes in legal regulations in the field of health care.

- In the Czech Republic: the Patients' Council (*Pacientská rada*)
- In Hungary: Hungarian Patient Forum (*Nemzeti Betegfórum*)
- In Poland: the Patient Organization Council (*Rada Organizacji Pacjentów przy Ministrze Zdrowia*)

An important forum for dialogue, though **their role remains limited to consultation**.

SPECIFIC LEGAL FRAMEWORK:

Different types of councils and committees composed of patient organization representatives

The activity of such rare disease councils may influence the creation of health policy regarding rare diseases, financing issues, details, e.g. regarding newborn screening or patient registry.

2. **Bodies related to the National Plans for Rare Diseases:**

In Austria: the Rare Diseases Advisory Council (*Beirat für seltene Erkrankungen*) –> Members of **PRO RARE AUSTRIA**, are delegated to this Council.

In Hungary: the Expert Committee on Rare Diseases (*Ritka Betegségek Szakértői Bizottság*) –> The National Organization for Rare Disorders (*Ritka és Veleszületett Rendellenességgel élők Országos Szövetsége*) was mentioned by name in the National Plan.

In Romania: the National Committee for Rare Diseases (*Comitetul Național de Boli Rare*) –> The Committee has 20 members including **two patient representatives** as well.

In Slovakia: the Commission of the Ministry of Health of the Slovak Republic for Rare Diseases (*Komisia Ministerstva zdravotníctva Slovenskej republiky pre zriedkavé choroby*) –> One representative from **the Slovak Alliance for Rare Diseases** is delegated to this Commission.

GENERAL ADMINISTRATIVE PROCEDURES

Tools available to all citizens in a given country

In many countries, patient organizations can use tools that are accessible to all citizens for their advocacy efforts.

1. **The citizens' initiative (*Bürgerinitiative*)** in Austria:

   The opportunity to submit an interpellation – like question to the National Council.

   500 signatures from citizens.

   Citizens are guaranteed to receive an answer to their question on paper.

   Available to almost all patient organizations.

2. **The people's legislative initiative**:

   I.e. Austria, Poland, Romania – **100,000 signatures.**

   Requires good organization, easier for large POs, in media cases.

   ➢In Poland: a citizens' draft amendment to the act on social pensions increasing this benefit was partially adopted.

GENERAL ADMINISTRATIVE PROCEDURES

Tools available to all citizens in a given country

3. **Public consultations**:
   - ➢In Hungary:
     1. General consultations - if the legislator rejects the comments, he must publish **a collective justification**.
     2. Direct consultations – the minister can make a **strategic partnership agreements** with organizations that represent broad social interests .
   - ➢In Slovakia:
     1. Mass comment – 500 signatures under the comment + representative – if the legislator doesn't agree with these comments, he must conduct **adversarial proceedings**.
     2. During the second reading of the bill, committee meetings are public, and public participation is allowed until the seats in the room are filled.

In many countries, patient organizations can use tools that are accessible to all citizens for their advocacy efforts.

GENERAL ADMINISTRATIVE PROCEDURES

Tools available to all citizens in a given country

4. **Access to public information**:
   It allows to obtain hard, official data, numbers, statistics.
5. **Participation of NGOs in administrative proceedings** on the rights of the party in the medicine reimbursement proceedings:
   - Case study from Poland: a patient with an ultra-rare disease.
6. **Other tools:** the Ombudsman, the right to petition, etc.

In many countries, patient organizations can use tools that are accessible to all citizens for their advocacy efforts.

ALTERNATIVE WAYS OF PATIENT ADVOCACY

Creative approach to influencing decisions

1. **Cooperation agreement between PO and the Ministry:**
   - ➢In Romania: between the Ministry of Health and the Romanian National Alliance for Rare Diseases.
   - ➢In Hungary: between the Secretariat of State for Health of the Ministry of the Interior and the Hungarian Alliance of Patient Organizations.
2. **The Medical Association**:
   - ➢In Austria: the Austrian Medical Association has voting rights in the Committee for the Evaluation of Therapeutic Products (*Heilmittel-Evaluierungs-Kommission, HEK*); Representative of the Austrian Chamber of Pharmacists (*Österreichische Apothekerkammer*), is also present in the HEK and the Federal Health Commission (*Bundesgesundheits-kommission, BGK*).

Alternative advocacy methods typically involve collaborating with other entities that have a stronger position.

ALTERNATIVE WAYS OF PATIENT ADVOCACY

Creative approach to influencing decisions

3. **Trade unions and employers organizations:**

➢In Austria: Members of the Austrian Chamber of Commerce and the Chamber of Employees are legally authorized members of both the inpatient (LKF) and outpatient (DVSV) medical procedure evaluation committees.

!Medical associations, trade unions, and employer organizations mainly represent their members' interests. However, if their goals align with those of patient organizations, it may be worth collaborating while keeping activities fully transparent.

Alternative advocacy methods typically involve collaborating with other entities that have a stronger position.

EURORDIS
RARE DISEASES EUROPE

Advocating at national level – National Plans & National Strategies

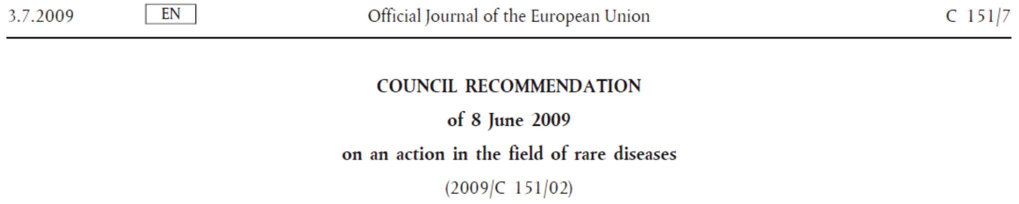
Background and principles of RD plans in the EU

The Communication from the European Commission in 2008, followed by the EU Council Recommendation on ‘an action in the field of rare diseases’ adopted in 2009, have provided strong political support and guidance to address the challenges faced by the patients, and to foster research.

European Commission

Project EUROPLAN (2008-2011)
- Recommendations
- Indicators

- Elaborate and **adopt a plan or strategy** as soon as possible, preferably **by the end of 2013 at the latest**
- Take action to integrate current and future initiatives at **local, regional and national levels** into their plans or strategies for a **comprehensive approach**
- Framework of their **health and social systems**
- Objectives and **follow-up mechanisms**


3.7.2009 EN Official Journal of the European Union C 151/7

COUNCIL RECOMMENDATION
of 8 June 2009
on an action in the field of rare diseases
(2009/C 151/02)

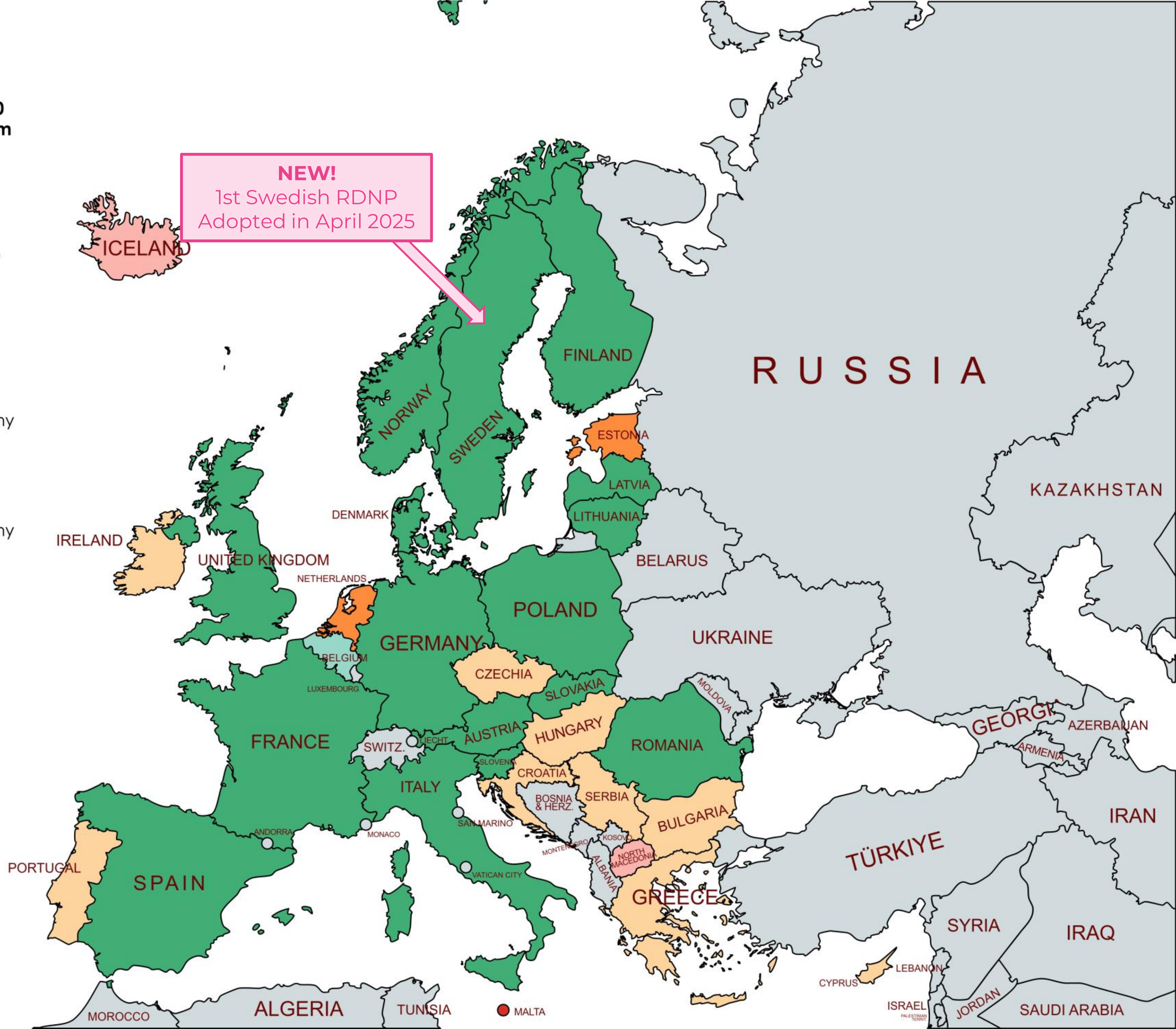
EURORDIS.ORG

32 European countries surveyed:

27 EU MS
+
Iceland
Norway
UK
Serbia
North Macedonia

STATUS QUO OF RDNP/RDNS AS OF 20 MAY 2025 - Data from National Alliances

- 🟩 Adopted
- 🟩 No expiry date. Implementation is no longer in place
- 🟧 Terminated. Not renewed
- 🟨 Terminated. New Plan/Strategy is under development
- 🟥 Never adopted any but a 1st Plan/Strategy is under development
- 🟥 Never adopted any



NEW!
1st Swedish RDNP
Adopted in April 2025

Created with mapchart.net

Why is a rare disease national plan needed?

1 **Recognise and address the specificities of rare diseases in a comprehensive manner** by setting up a political and legal framework, involving all stakeholders and coordinating all relevant actions at the national and regional level.

2 **Implement adequate care pathways for rare disease patients:** map out the medical expertise on rare diseases across the country; adopt an official process to designate multidisciplinary Centres of Expertise; facilitate the participation of these centres in European Reference Networks for rare diseases.

3 **Identify or establish social services and programmes relevant to rare diseases** (resource centres, disability programmes, specialised trainings for professionals…); bridge the gaps between different services through case management.

4 **Support basic, translational, clinical and social research.**

5 **Facilitate the integration of European legislations and policy recommendations** relevant to rare diseases into national healthcare and social systems.

6 **Acknowledge National Alliances and rare disease patients' organisations as key and equal partners;** involve them in the governance of the plan and decision-making committees of programmes dedicated to healthcare, social care and research.

A classification of RD National Plans

- **France** as a single example of an **integrated plan** covering all the fields (care, cure, research, centres of expertise, patient's recognition, etc.) and receiving an **adequate funding** and having a **continuity**. New French Plan 2025-2030 as the 4th Plan with 223 millions of euros.

- Countries were the plan covers some aspects of EUROPLAN and EURORDIS recommendations with *discontinuity or modest financing*.

- Countries using National Plan to resolve national/regional *coordination of actions*.

- Countries where the National Plans are a "*love letter*" to patients.

The influence of the size of the country.

See description of Plans:
https://www.eurordis.org/rare-disease-policy/national-policy/

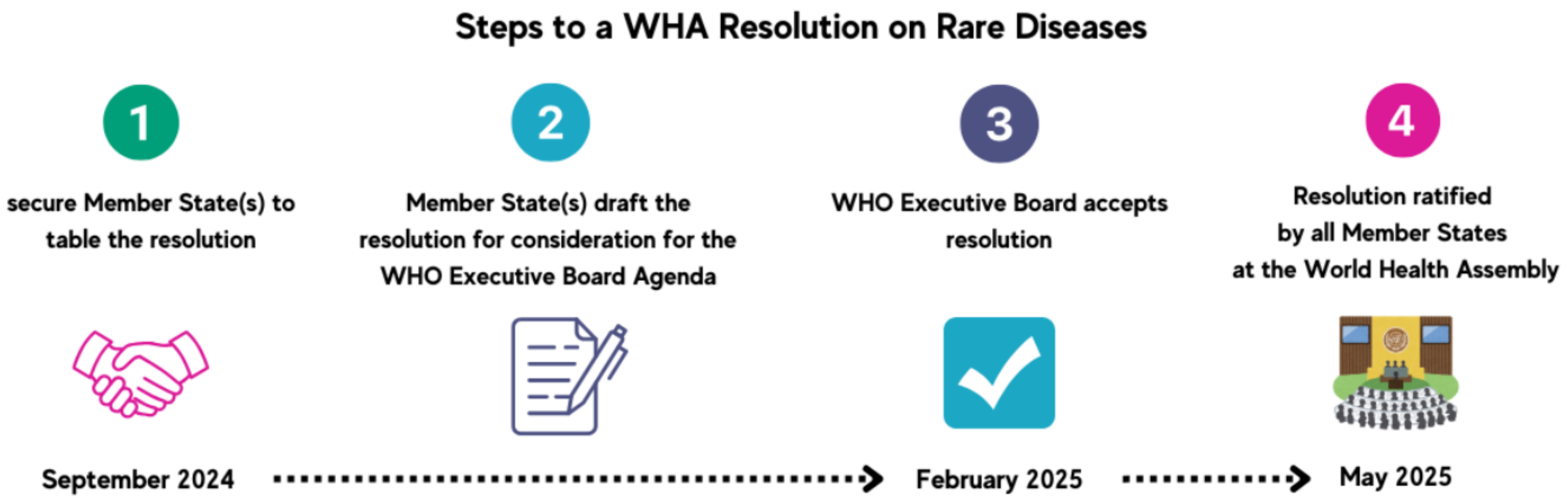
Advocating at national level – National Plans & National Strategies

We need common goals and a roadmap to arrive there together!

National Plans of Rare Diseases: are they efficient without a European Rare Diseases Action Plan?

- → Your **actions at national or regional level** adopting National or Subnational Plans are absolutely necessary
- → Raise **awareness** with national authorities in favour of an expanded EU action
- → Sensibilisation of rotating EU Council **Presidencies**
- → Adoption and debates in the European Parliament & adoption of resolutions of other European bodies supporting the European Action Plan
- → Direct lobbying action to the European Commission

… and the international level?



Steps to a WHA Resolution on Rare Diseases

1. secure Member State(s) to table the resolution
2. Member State(s) draft the resolution for consideration for the WHO Executive Board Agenda
3. WHO Executive Board accepts resolution
4. Resolution ratified by all Member States at the World Health Assembly

September 2024 → February 2025 → May 2025

The WHA Resolution on Rare Diseases, which will be decided upon this Friday 23rd May 2025 is a step toward visibility, inclusion, and real change.

Your turn to apply the knowledge!

Group exercise

"An injection a day, keeps my flare away,"
RELA haploinsufficiency, Netherlands

Break time

"Healing," Happloinsufficiency
MEF2C, Poland

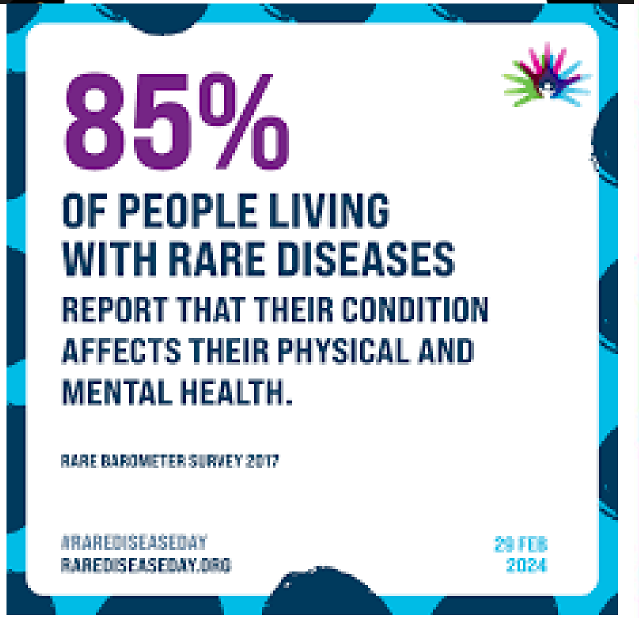
IV. Jump into action!

Build your message

Your key messages should :

- Identify the **main issues** faced by your community
- Be communicated in a **concise**, understandable and **impactful** way
- **Adapted** depending on your audience!
- Convey **robustness** (*power of data*)
- Convey **urgency** & a “**human**”, anecdotal element (*power of stories*)

The power of data



85%

OF PEOPLE LIVING WITH RARE DISEASES REPORT THAT THEIR CONDITION AFFECTS THEIR PHYSICAL AND MENTAL HEALTH.

RARE BAROMETER SURVEY 2017

#RAREDISEASEDAY
RAREDISEASEDAY.ORG

29 FEB 2024

72%

OF ALL RARE DISEASES ARE GENETIC

WHILST OTHERS ARE THE RESULT OF INFECTIONS (BACTERIAL OR VIRAL), ALLERGIES AND ENVIRONMENTAL CAUSES OR ARE RARE CANCERS

#RAREDISEASEDAY
RAREDISEASEDAY.ORG

29 FEB 2024

70%

OF GENETIC RARE DISEASES START IN CHILDHOOD!

#RAREDISEASEDAY
RAREDISEASEDAY.ORG

29 FEB 2024

1 IN 5 CANCERS IS RARE

THE INCIDENCE FOR RARE CANCERS IS LESS THAN 6 INDIVIDUALS PER 100,000 A YEAR.

#RAREDISEASEDAY
RAREDISEASEDAY.ORG

29 FEB 2024

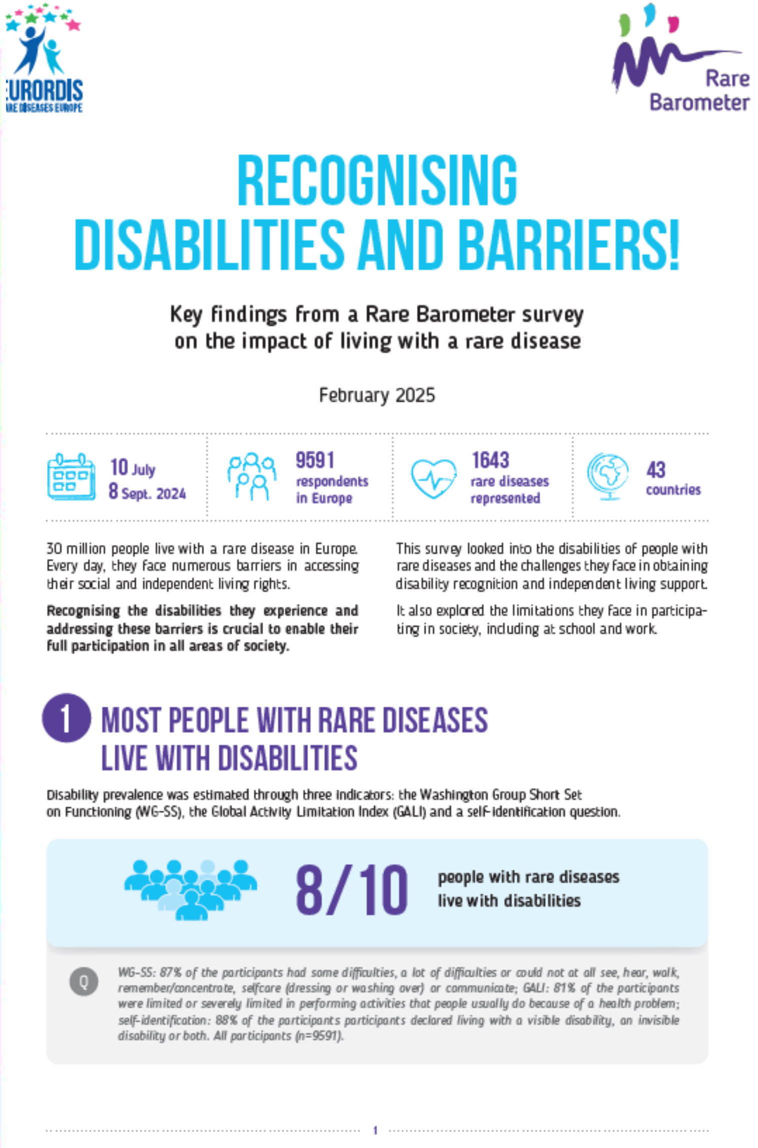
THE POPULATION OF PEOPLE WITH RARE DISEASES EQUALS THAT OF

THE WORLD'S 3RD LARGEST COUNTRY

300 MILLION PEOPLE WORLDWIDE ARE LIVING WITH A RARE DISEASE

#RAREDISEASEDAY
RAREDISEASEDAY.ORG

29 FEB 2024



EURORDIS RARE DISEASES EUROPE

Rare Barometer

RECOGNISING DISABILITIES AND BARRIERS!

Key findings from a Rare Barometer survey on the impact of living with a rare disease

February 2025

10 July
8 Sept. 2024

9591 respondents in Europe

1643 rare diseases represented

43 countries

30 million people live with a rare disease in Europe. Every day, they face numerous barriers in accessing their social and independent living rights.

Recognising the disabilities they experience and addressing these barriers is crucial to enable their full participation in all areas of society.

This survey looked into the disabilities of people with rare diseases and the challenges they face in obtaining disability recognition and independent living support.

It also explored the limitations they face in participating in society, including at school and work.

1 MOST PEOPLE WITH RARE DISEASES LIVE WITH DISABILITIES

Disability prevalence was estimated through three indicators: the Washington Group Short Set on Functioning (WG-SS), the Global Activity Limitation Index (GALI) and a self-identification question.

8/10 people with rare diseases live with disabilities

Q *WG-SS: 87% of the participants had some difficulties, a lot of difficulties or could not at all see, hear, walk, remember/concentrate, selfcare (dressing or washing over) or communicate; GALI: 81% of the participants were limited or severely limited in performing activities that people usually do because of a health problem; self-identification: 88% of the participants participants declared living with a visible disability, an invisible disability or both. All participants (n=9591).*

1

When we turn lived experience into data, we turn stories into systems change. That’s the power of Rare Barometer.

JESSIE DUBIEF,
SOCIAL RESEARCH DIRECTOR,
RARE BAROMETER PROGRAMME LEAD

The power of data

Sources for research that support your position can be found via:

- EURORDIS Rare Barometer surveys: https://www.eurordis.org/publications/make-your-voice-heard-rare-barometer-guide/
- Academic research (Google Scholar, etc)
- Your own research: Open Academy Course > https://openacademy.eurordis.org/courses/survey-design-rare-disease-patient-organisations/
- Publications in your specific disease area

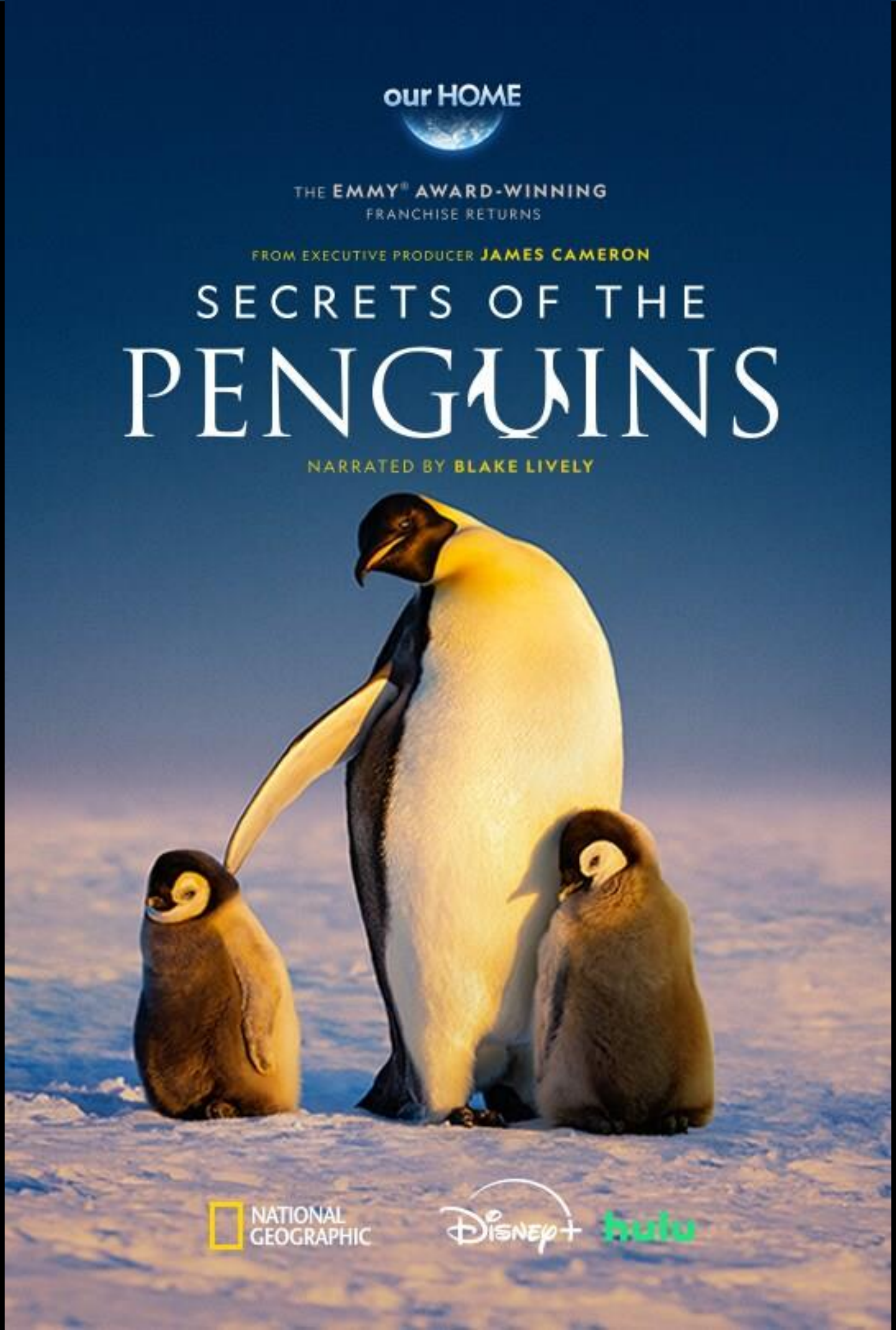
The power of stories

CERTIFICATE OF ADOPTION

Zé

Penguin Number:	AP031
Date Admitted:	05-May-2021
Area found:	Bird Island
Age:	Chick
Reason for Admission:	Abandoned
Adopter:	Raquel Castro
Date:	2021-07-09

www.sanccob.co.za


our HOME
THE EMMY® AWARD-WINNING
FRANCHISE RETURNS
FROM EXECUTIVE PRODUCER JAMES CAMERON
SECRETS OF THE
PENGUINS
NARRATED BY BLAKE LIVELY
NATIONAL GEOGRAPHIC
Disney+
hulu

The power of stories

Make complex issues simple, relatable, memorable.

Help connect emotionally and encourage action.

Evidence isn't enough.
Facts alone don’t move people to act.

You don’t need to be dramatic or poetic.
Telling stories is part of being human.

The power of stories

Use storytelling to:

1. Make your audience care about your "penguin".
2. Show the challenges your "penguin" faces.

Mix stories with evidence:

- ✓ To humanise statistics and help build empathy.
- ✓ To illustrate and reinforce the impact of evidence.
- ✓ To strengthen credibility.

The power of stories

Your story is who you are to a policymaker.
Make it strategic. Make it authentic.

Collect your stories & choose stories intentionally

- What story reflects your cause’s urgency, humanity, and hope?
- Which stories build empathy?
- Which create suspense? Which make people laugh?
- Remember to keep them concise

Tell positive stories too:
About your impact, about the future you're trying to achieve.

But, but, but…

- ☑ Informative
- ☑ Relatable
- ☑ Relevant
- ☑ Emotional
- ☑ Inspirational
- ☑ Engaging
- ☑ Entertaining

To turn a story into a powerful advocacy tool…

You need an ask:
a clear, specific call to action
that your policy maker can do

Use storytelling to:

1. Make your audience care about your "penguin".
2. Show the challenges your "penguin" faces.
3. Articulate how your recommendations can address them.
4. Ask the policy maker to act and be part of the change.

CERTIFICATE OF ADOPTION

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Adopter:	Raquel Castro
Date:	2021-07-09

www.sanccob.co.za

CERTIFICATE OF ADOPTION

String

Date Admitted:	03-July-2011
Area Found:	Blouberg
Reason for Admission:	Weak and tame. Neurological deficit, compromised sight in one eye
Age on Admission:	Blue
Adopted by:	Raquel Castro
Adoption Date:	17-May-2025

Meeting policymakers

Before the meeting

- Research the policymaker’s background, position on healthcare previous work on rare diseases.
- Develop a clear agenda for the meeting, outlining the key asks (e.g., increased funding for rare disease research, amendments to orphan drug laws).
- Be concise and clear about your issue and why it should matter to them.

During the meeting

- Share your story and make it personal.
- Provide supporting facts and data to back up your position.
- Focus on the potential impact of policy changes on both patients and society.
- Ask for specific actions (e.g., signing a petition, advocating for legislative changes,...).

After the meeting

- Send a follow-up email thanking them for their time, reiterating key points, and providing additional resources or evidence if necessary.

Meeting policymakers: the good the bad & the ugly

Watch :

https://youtu.be/XxqvbeqrR8s
THE UGLY

https://youtu.be/vCfnOt_56ok
THE BAD

https://youtu.be/Hzk5fduCuKk
THE GOOD

Top tips…

→ Identify the individual policymakers who have the most influence over issues that affect your community i.e. healthcare funding, orphan drug regulation, rare disease research etc.

→ Consider their stance and their history with rare disease advocacy.

→ Tailor your messaging to align with their interests, demonstrate the impact of rare diseases on their constituency or Europe at large.

Remember: a policy maker:

- Has spent time building social and political capital. Safeguards public image and reputation.
- Operates in a political hierarchy.
- Personal story and interests.
- Short on time, limited attention for details.
- Constantly assesses trade-offs: media appeal, popular support, feasibility, cost.
- Interest may depend on election cycle.

Time for a role play exercise

There is more than meetings…

BASICS OF MEDIA RELATIONS

How to start and maintain relations with journalists

1. **Build a database**
    - Look for journalists covering healthcare, include new media like podcasts, videos, social channels
    - **Creat a simple division of the data (regional/national)**
    - If something happens, you can reach out to the right part of the database
    - You can also send press releases related to your diseases' days, weeks or months
2. **Compose the right message**
    - AI can help you with a press release or summary for journalists
    - Keep it consise, make a bullet points summary at the beginning, include max. 2 „speakers"
    - Offer something for the journalists, like a patient to interview

Always suplement you messages with rich media ready to be used – videos or photos. Keep your communication short.

WRAP-UP

A toolkit

For extra material:

EDU

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Thank you!