

# Rare Disease Week 2024

Webinar #4: Preparing for the training  
week !



# Welcome!

We will be starting shortly...



**Use your real name**



**Mute yourself when not speaking**



**Turn your camera on, if comfortable**



**Use the chat box for questions**



**Or raise your hand to use your microphone**

*Please note that this training will be recorded. The recording will be available for participants only.*

# Agenda

- **Practical information and resources for Brussels – Rachel**
- **What the EU has done for RD – Vale**
- **MEP meetings explained – Julie**

# Practical information for Brussels

# In-person agenda

Monday 18th November 2024

Time	Session
2-2.30pm	Introduction & Welcome - <a href="#">Silversquare Europe</a> - EURORDIS CEO Virginie Bros-Facer
2.30-4pm	Presentation from European Commission - <a href="#">Silversquare Europe</a> - Commission give more details on their role in the EU and legislative process
4-4.30pm	Comfort break
4.30-6pm	Workshop: Preparation for MEP meetings - <a href="#">Silversquare Europe</a> - Workshop with advocacy team and Toni Montserrat (ALAN) to prep for MEP meetings
7pm	Welcome drink - <a href="#">Moxy Brussels City Center</a> (optional)

# In-person agenda

Tuesday 19th November 2024

Time	Session	
11-12:30pm	<b>Tour of parliament - <a href="#">EP Visitor Area Henri Spaak</a></b> - Guided tour by staffer of parliament and hemicycle	<b>MEP meetings in parallel</b>  - You will receive a personalised agenda of MEP meetings organised for you during RDW24
12.30-2pm	<b>Drop-in/drop-out lunch - <a href="#">Silversquare Europe</a></b> - Catch-up between MEP meetings and activities	
3-4.30pm	<b>Parlamentarium visit - <a href="#">Parlamentarium</a></b> - Learn about the history of the EU	
<b>Free evening - time for you to explore Brussels!</b>		

# In-person agenda

Wednesday 20th November 2024

Time	Session	
10-11.30am	<b>Meeting the European Economic and Social Committee - <a href="#">EESC office</a></b> <ul style="list-style-type: none"> <li>- Presentation and discussion from Beatriz Porres, Head of Unit, and Ágnes Cser, rapporteur, EESC</li> </ul>	<b>MEP meetings in parallel</b> <ul style="list-style-type: none"> <li>- You will receive a personalised agenda of MEP meetings organised for you during RDW24</li> </ul>
12-1.30pm	<b>Networking lunch with MEPs - <a href="#">European Parliament</a></b> <ul style="list-style-type: none"> <li>- MEPs present their work, future health issues &amp; how they engage in the EP</li> </ul>	
3-4.30pm	<b>Roleplay game - <a href="#">Parliamentarium</a></b> <ul style="list-style-type: none"> <li>- Learn how decisions are taken in the EP</li> </ul>	
6.30pm	<b>Closing dinner - <a href="#">Moxy Brussels City Center</a></b>	

# Resources to help prepare for BXL

- [MEP Briefings](#)
- [RDW24\\_key documents](#)
  - **Practicalities document** – information on logistics and accessibility
  - **EP Access Map** – Map of buildings of the European Parliament
  - **RDW24 Agenda** – programme of the in-person activities in Brussels
  - **EURORDIS Policy Priorities** – concrete and practical asks that we are proposing to MEPs to help improve the lives of people living with rare diseases

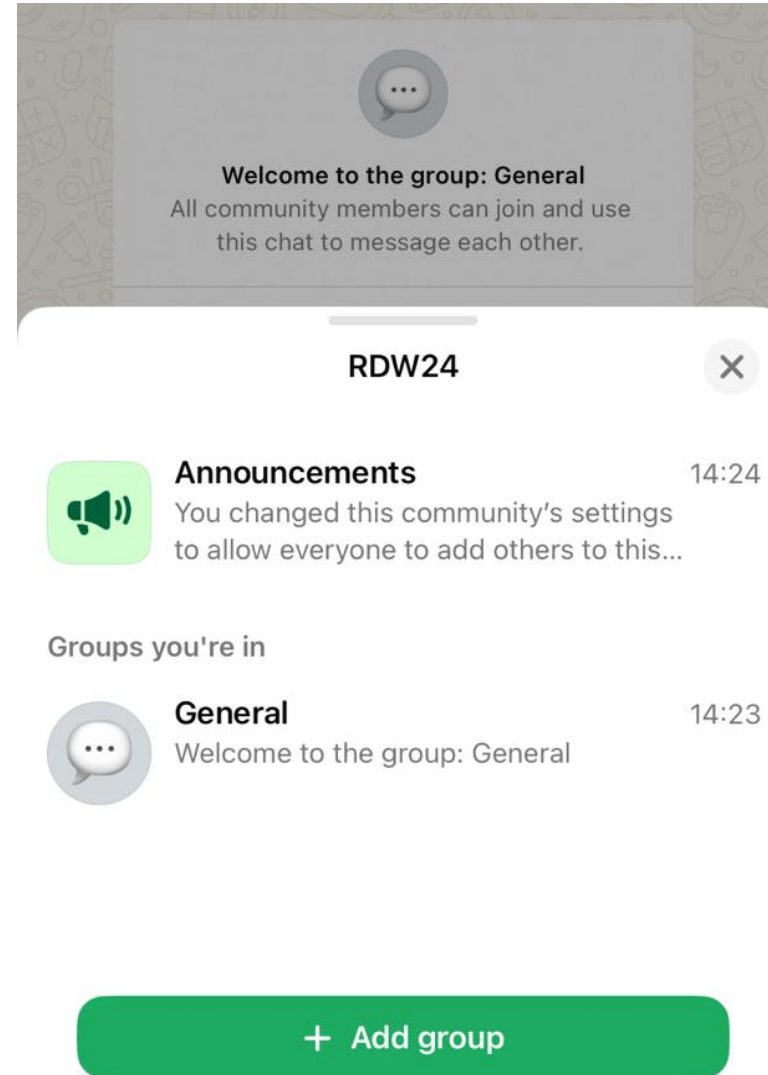




# Resources to help prepare for BXL

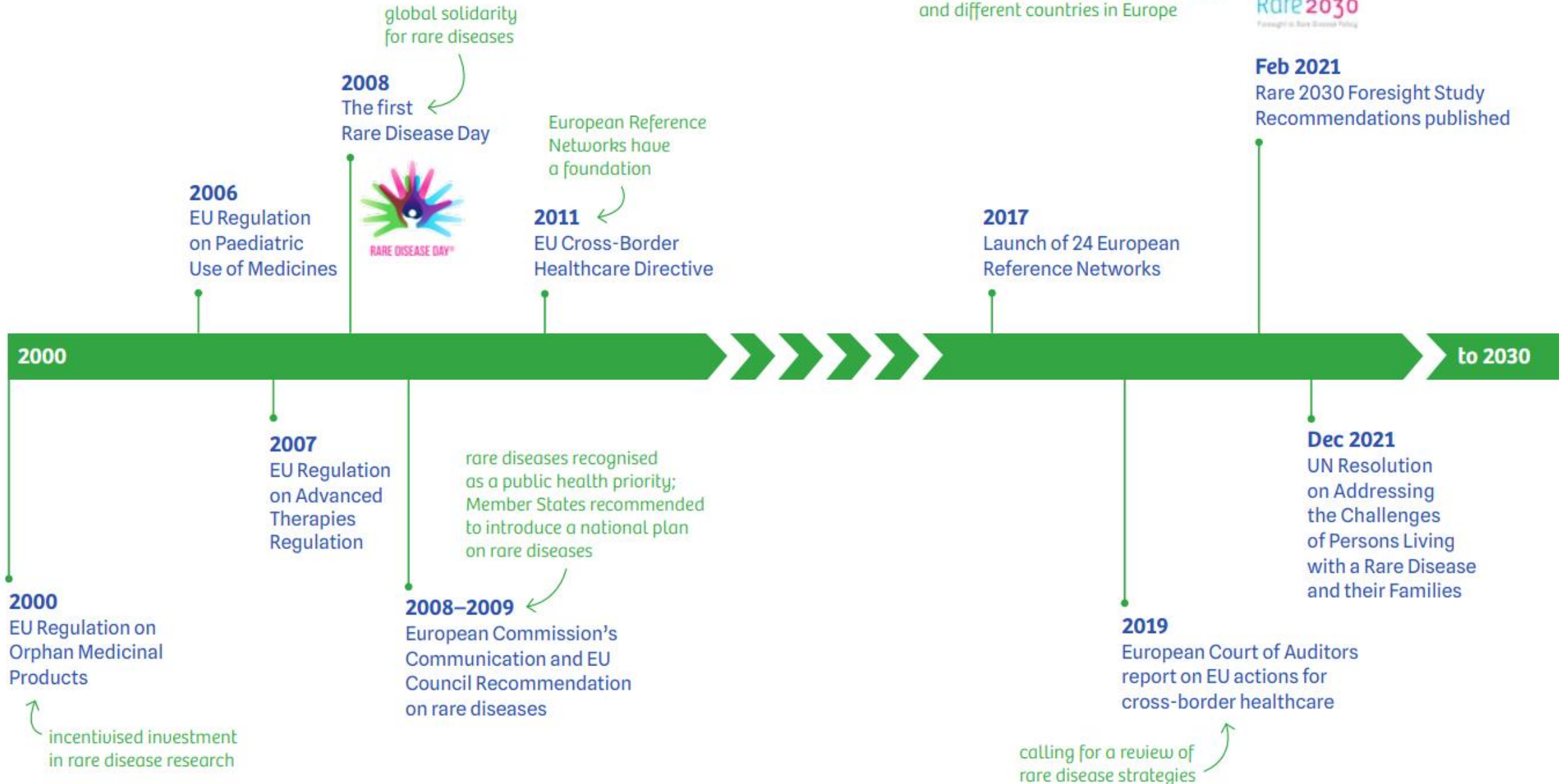
- **WhatsApp Community**
  - Join our WhatsApp Community to receive information, create groups with other participants and keep in touch with us during the in-person days
- Join via the QR code or via the link >

LINK



# What has the EU done for RD ?

# The rare disease story in Europe so far



# First milestone

Together with its members, EURORDIS has contributed to establishing a centralised procedure for the designation of orphan medicinal products and putting in place incentives for their research and development.

## year 2000

EU Regulation on orphan medicinal products



# Milestones

- EU Regulation on medicinal products for paediatric use (2006)
- EU Regulation on advanced therapy medicinal products (2007)
- EU Directive on Patients' Right to Cross-Border Healthcare (2011)
  - ↓
- European Reference Networks are established (2017)
- EU Regulation on Health Technology Assessment (2021)
- EU Regulation on European Health DataSpace (2024)

**EURORDIS went on to play an instrumental role in advocacy efforts that led to the adoption of numerous EU legislations that have a positive impact on people living with a rare disease in Europe**

# Milestones

## EU Strategic policy frameworks:

- **Commission Communication Rare Diseases: Europe's Challenges (2008)**
- ↓
- **EU Council Recommendation on a European action in the field of rare diseases (2009)**

**Advocating for funding and for policy developments (non-legislative) is equally essential!**

## EU funding programmes:

As from 1998 Rare Disease in EU funding programmes:

- **EU Research Framework Programmes**
- **Public Health Programmes**

# What was the outcome of these legislative and non legislative actions?

These milestone policies and legislations, adopted in the first decade of the XXI century, paved the way for an integrated EU strategy on issues including diagnosis, treatment, and care, and encouraged national plans to support the 30 million Europeans living with a rare disease.



The past 25 years of taking forward the patient voice to EU Institutions have given us the progress that we would not have had otherwise.

But the work is not yet finished, so we will put our best foot forward to work toward establishing a **European Plan for rare diseases**, a new overarching strategy that works for all.





Do you want to know more?

Have a look at the 12 Labours comics strip:  
<https://www.eurordis.org/publications/12-labours-of-ecrd/>

LABOUR 1

## The Beginning: The Orphan Medicinal Products Regulation

Moving forward, we'll delve into the twelve labours that mirror the historical and ongoing efforts to address rare diseases. We begin in 2001 with the implementation of the EU Orphan Medicinal Products Regulation, a pivotal moment that catalysed the development of treatments for rare diseases.

For too long, rare diseases have lingered in the shadows, their mysteries locked away without hope for cures.



But change whispers on the horizon, carried by the voices of the few who dare to dream.



With unity and perseverance, the rare disease community comes to the fore, marking a pivotal moment.



From the shadows emerge hope and healing, as 250 new paths to treatment are forged, promising brighter days ahead.

# Next and ongoing challenges

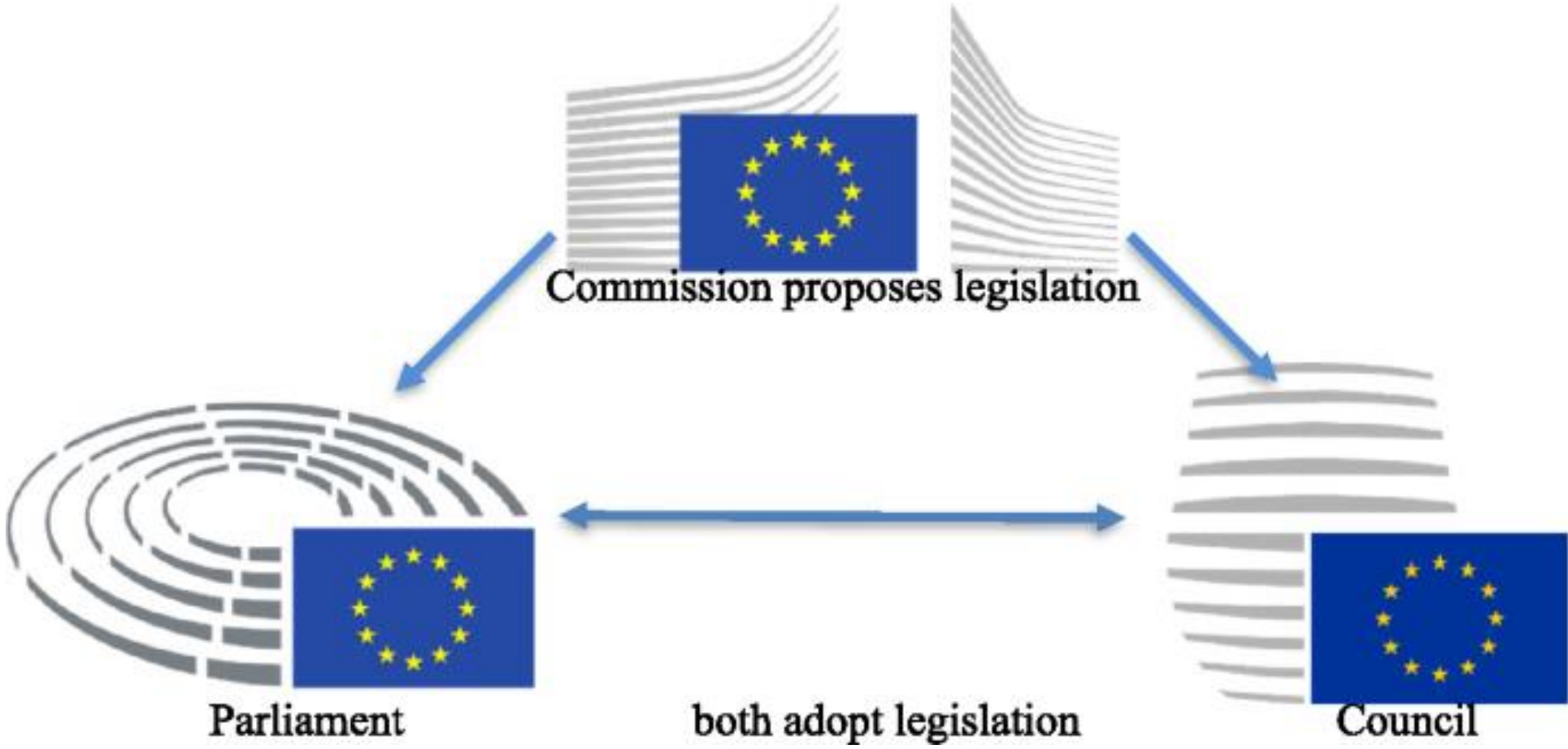
## LEGISLATIVE FILES:

- **A European Action Plan for Rare Diseases**
- **Revision of General Pharmaceutical Legislation-->**  
including review of the orphan and paediatric medicines legislation
- **Long-term budget of the EU 2027-2035 -->**  
including Health and Research funding programme

**It is still crucial to make the voice of people living with rare disease heard and continue the advocacy efforts leading to the adoption of EU legislations and policies that have a positive impact on their lives**

**What decision  
makers are going  
to meet this week**

# The "game of three"



# The European Commission



The **European Commission** represents the **EU general interest**

**Propose** new laws,  
enforcing legislation,  
set budget priorities  
and supervise budget

**27 Members:** College  
of Commissioners, 1  
from each EU country

**President:** Ursula von  
de Leyen

**Location:** Brussels  
(Belgium)



# The European Parliament



**Members of the European Parliament** are directly elected by the citizens of Europe, and they **represent the interests of EU citizens** at European level.

**Role:** Directly-elected EU body with **legislative, supervisory, and budgetary** responsibilities

**Members:** 705 MEPs (Members of the European Parliament)

**President:** Roberta Metsola

**Location:** Strasbourg (France), Brussels (Belgium), Luxembourg



# The Council of the EU



The **Council of the EU** represents **Member States governments** at European level

**Role:** Adopts EU laws and coordinates EU policies, adopts EU budget

**Members:** Government ministers from each EU country, according to the policy area to be discussed

**President:** Each EU country holds the presidency on a 6-month rotating basis – currently Czech

**Location:** Brussels (Belgium)

# The European Economic and Social C (EESC)



The **EESC** is an **advisory body** of the European Union representing the voice of organised civil society organisations

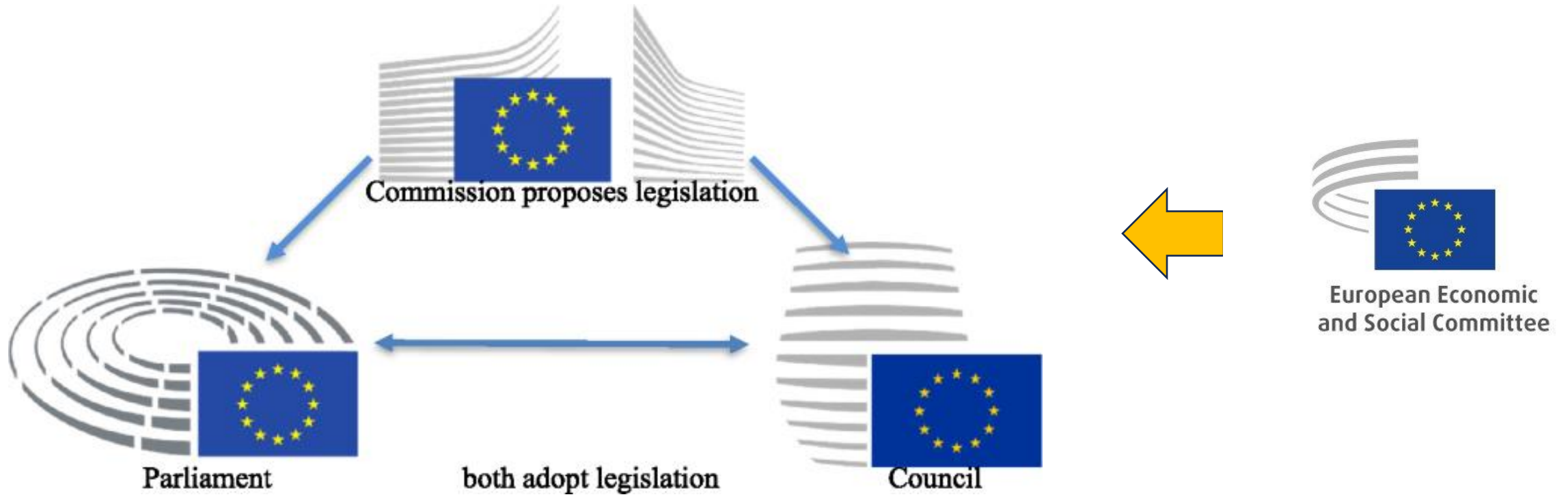
**Role:** Ensures that EU policies tie in with economic, social and civic circumstances on the ground

**Members:**  
329 representing employers, civil society org. Nominated by their governments for 5 years

**President:** elected by the Committee for 2,5 years

**Location:** Brussels (Belgium)





# EESC Opinion "Ensuring strong European solidarity for rare diseases" (October 2022)

“Having heard evidence from people right across the patient community and civil society, we learned that there is huge, unrealised potential for EU policy to make sure scientific, technological, clinical and social advances reach every European citizen living with a rare disease”  
Alain Coheur, EESC Rapporteur.



## What the EESC called for:

- A European Action for RDs based on measurable goals
- Optimising the use of health data
- Speeding up diagnoses
- Support for – and beyond – the European Reference Networks
- Improving access to orphan drugs
- Greater support for informal carers

# EESC – Opinion on “EU commitment to tackling rare diseases” (23 October 2024)



SOC/806

A European commitment to fight rare diseases

## PRELIMINARY DRAFT OPINION

Section for Employment, Social Affairs and Citizenship

**We leave no one behind: a European commitment to fight rare diseases**

(exploratory opinion requested by the Hungarian presidency)

Rapporteur: **Ms Ágnes CSER**

# A new European Parliament and a new European context for health



# Current challenges

- **Far-right is now a big player:** not convinced by stronger European collaboration
- **De-prioritisation of health** by the EU institutions (in policy and funding)
- **Competition of other health initiatives** (cardiovascular diseases, diabetes)

# Political opportunities for this mandate (tbc)

- SANT own-initiative report on rare diseases
- Legislation on medicine shortages
- Initiative on women's health
- Mental health of vulnerable groups
- EP intergroup on Cancer and Rare diseases
- EU action plan on rare disease (tbc)

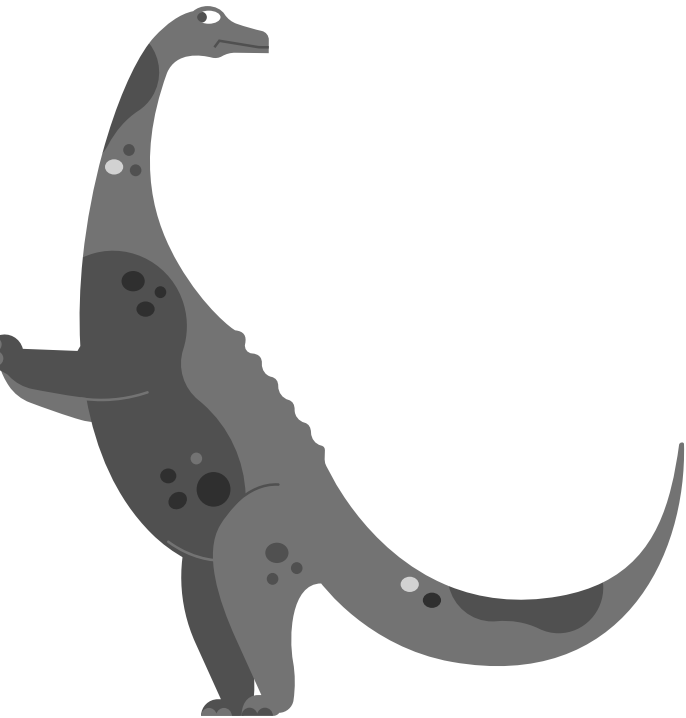
# MEP meetings explained

# HOW TO MANAGE AN MEP (MEETING)?





# Meetings with MEPs



- **29 meetings** on Tuesday 19 and Wednesday 20 November
- With **various groups**: EPP, RE, S&D, the Greens, the Left, PfE, ECR (far-right included)
- **From the following committees** : **SANT** (public health), **EMPL** (Employment and social affairs), **BUDG** (Budget)
- Meetings with **key players** : Chair, Vice-Chairs, group coordinators

# Like all human beings, some MEPs are nicer than others...



**Some are genuinely interested in what patients have to say...**



**Some are more interested in the nice picture they will take !**

**Some know already a lot about rare diseases, some don't have a clue... !**



# Things to keep in mind



- MEPs generally **don't have much time** : Meetings will last 20/30 min
- MEPs want **you** to talk ! They generally like to listen
- MEPs have **different interests** depending on the committees on which they sit
- MEPs like to hear **what they can do to help**
- MEPs act at **European level**, not at national level

# Different committees, different interests

- **SANT/ENVI (Public health)** : diagnosis, access to treatments, care pathways, cross-border healthcare etc.
- **EMPL (Employment and social affairs)** : impact of RD on access to employment, social care, participation to social life, economic situation, discriminations, disability rights etc.
- **BUDG (Budget)** : funding for rare diseases in EU programmes (healthcare, research), in a context of de-prioritisation of health by the EU institutions
- **FEMM (women's rights and non-discrimination)**: impact on RD on women (accessing a diagnosis, healthcare, the situation of carers etc.)

# Some tips



- Go **straight to the point** (after introducing yourself !)
- Keep it short so that all patient advocates can speak and keep it **European**
- Choose the **best discussion angle** depending on the MEP's committee & interest
- Invite them to support the **EP Intergroup on Cancer and Rare Diseases**
- Hand out the **policy priorities prepared by EURORDIS**

# The meeting in detail





# Key general messages to share with MEPs

- For rare diseases, **European collaboration is KEY** ! (message for MEPs belonging to far-right groups)
- Acknowledge the **specific high unmet needs of PLWRD** in various areas (healthcare & socio-economic)
- Keep **health as a top EU policy and funding priority**
- Create an ambitious and comprehensive **EU action plan/ European Strategy on Rare diseases** to address the high unmet needs of PLWRD
- **Increase cross-country collaboration** on newborn screening, access to specialised care and treatments, holistic care and mental health support
- **Address the situation of carers** (often family and women), the social isolation of PLWRD and the discrimination that can occur in the field of employment, housing, services

# What you will get to prepare the meetings



- A summary of the above points : the **Cheat sheet**
- A **short brief** for each MEP
- **EURORDIS policy priorities**



# Thank you

Any questions?