

# Advancement of Treatments FOR RARE DISEASES



## Advancement of Treatments for Rare Diseases

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| Tuesday, June 16, 2026



# Advancement of Treatments FOR RARE DISEASES

## The Téléthon :

since 1987, an extraordinary nationwide movement, with no equivalent



**4M**

→ ~4 million participants.



**260k**

→ ~260,000 volunteers mobilized and more than 22,000 events.



**13k**

→ More than 13,000 committed municipalities.



**59k**

→ ~59,000 local associations involved.



**100**

→ + 100 partners companies, professional, sport and leisure federations ...



**30h**

→ 30 hours TV program general interest partnership since 1987 with **France Télévisions**.



Organisers:



Funded by  
the European Union

The conference is funded by the  
European Union (project no 101297283)

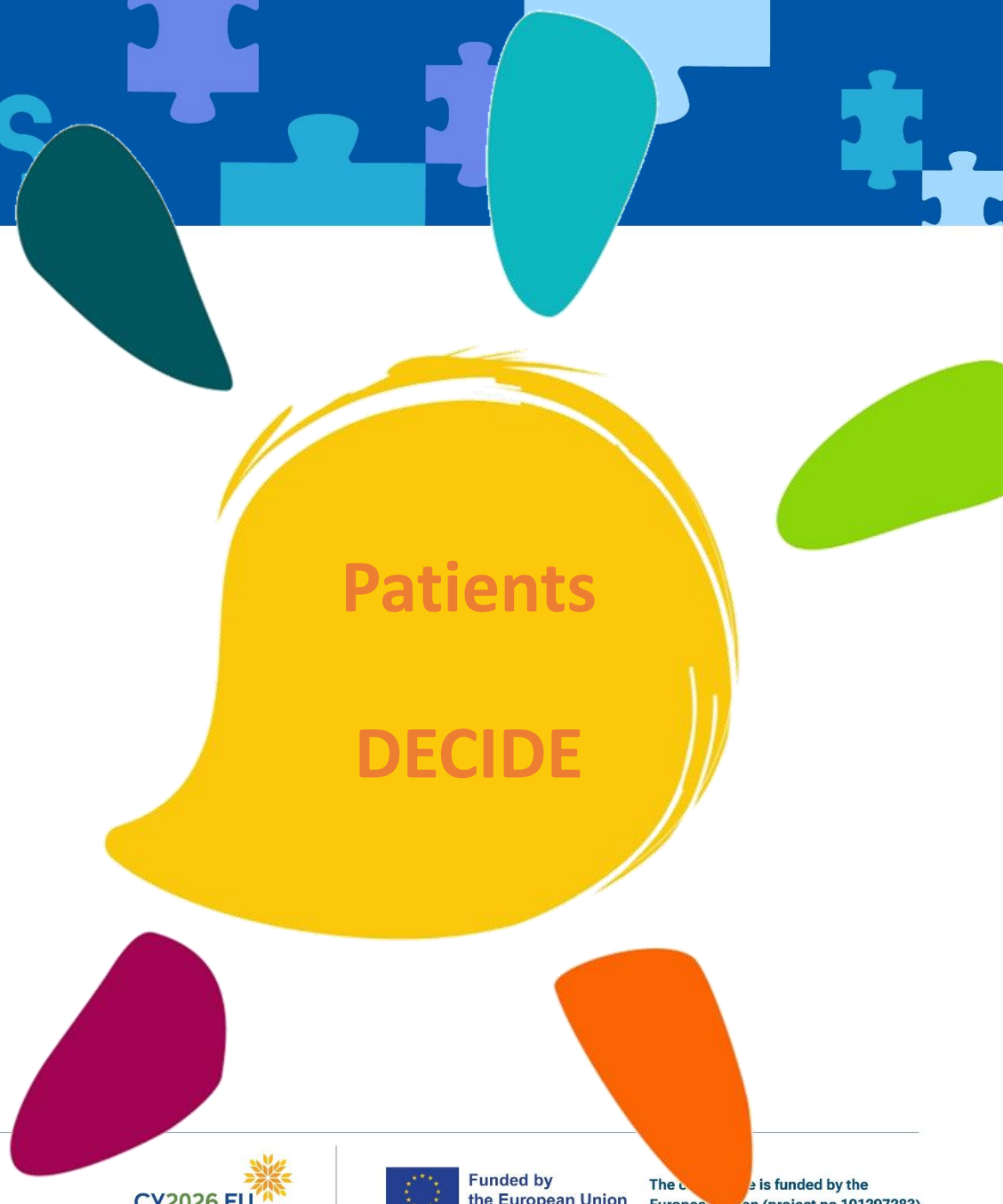
# Advancement of Treatments FOR RARE DISEASES

A strategy driven by  
**emergency**  
and  
**general interest**

CAre

Cure

Communicate



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## Care

 **10**

→ Expert Patient groups

 **270**

→ Professional Facilitators  
→ Meet families at home  
→ Empowerment

 **80**

→ Local representations

 **10k**

→ Patient follow-up  
→ Life project

 **1** resource center

→ Hotline  
→ Psychotherapy  
→ Technical Help  
→ Documentation  
→ Psychology  
→ Ergotherapy  
→ Emergency



# Advancement of Treatments FOR RARE DISEASES

Drug development from fundamental research **to the patient**

## Call for proposals

- PhD / postdoc fellowship
- Research project



## Strategic projects

## Strategic poles

→ 50% of the funds go abroad



Organisers:

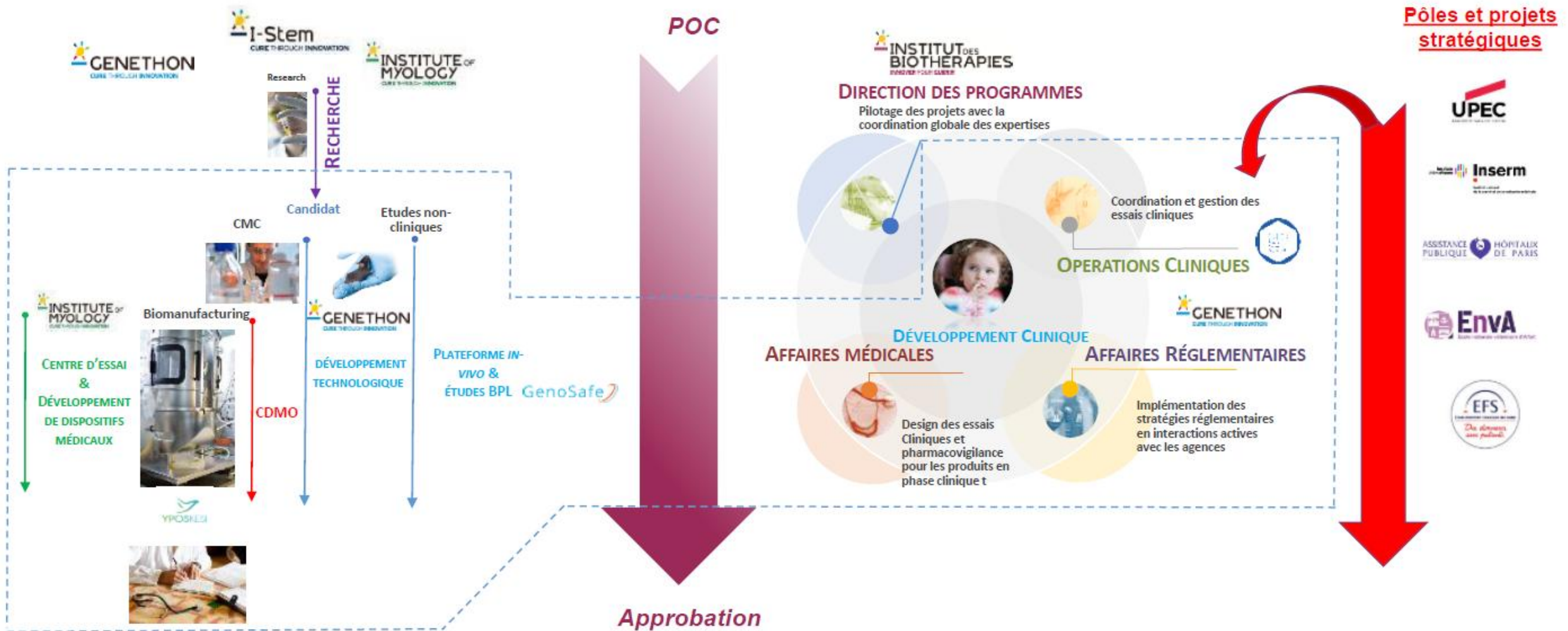


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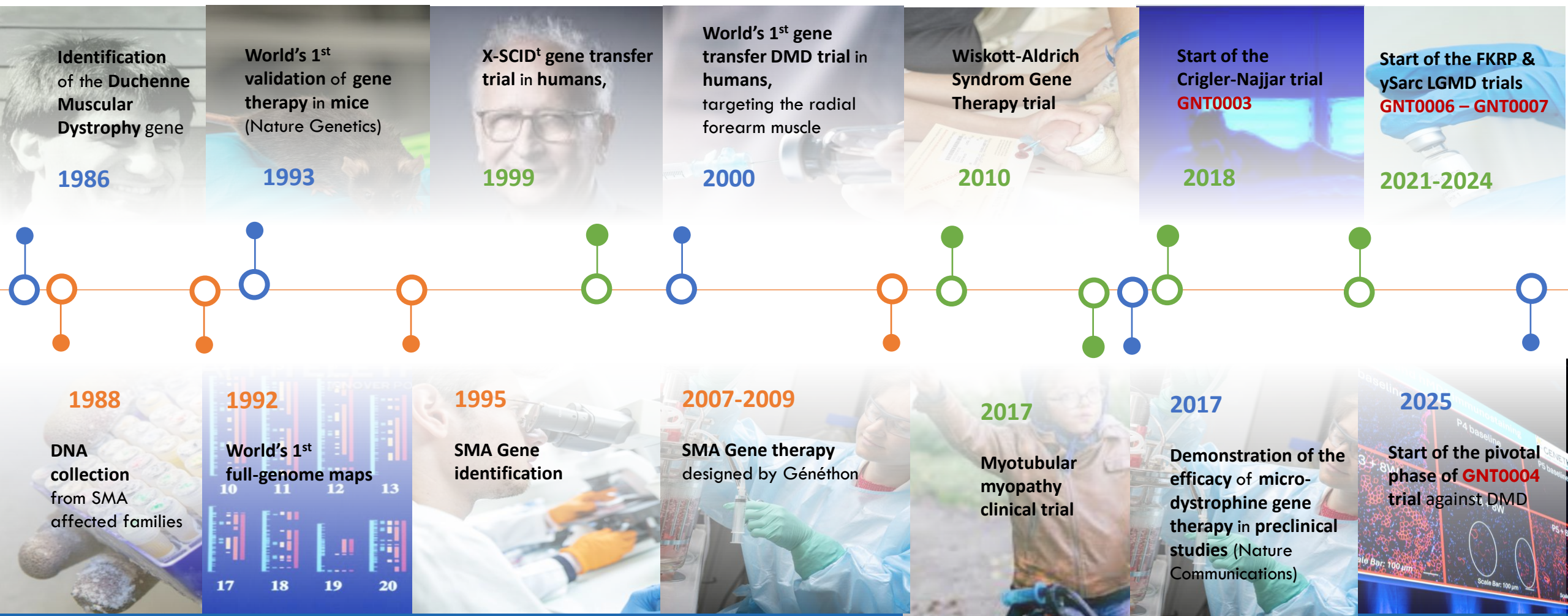
# Advancement of Treatments FOR RARE DISEASES

## Drug development from fundamental research to the patient



# Advancement of Treatments FOR RARE DISEASES

## 40 years of world-class outcomes paving the way to victory



# Advancement of Treatments FOR RARE DISEASES

## 40 clinical trials supported in more than 30 Rare Diseases

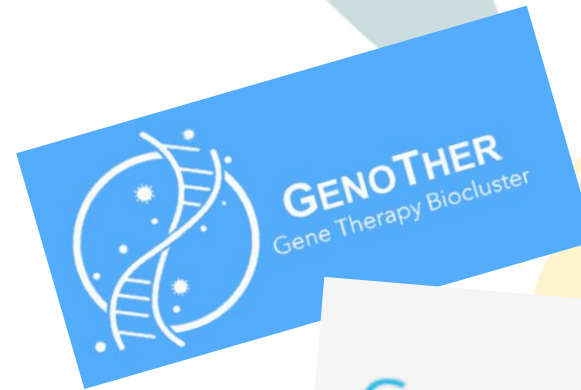
### Last kilometer challenges

Market access

Ultra Rare Diseases

Innovative drugs price

Health independence



Organisers:

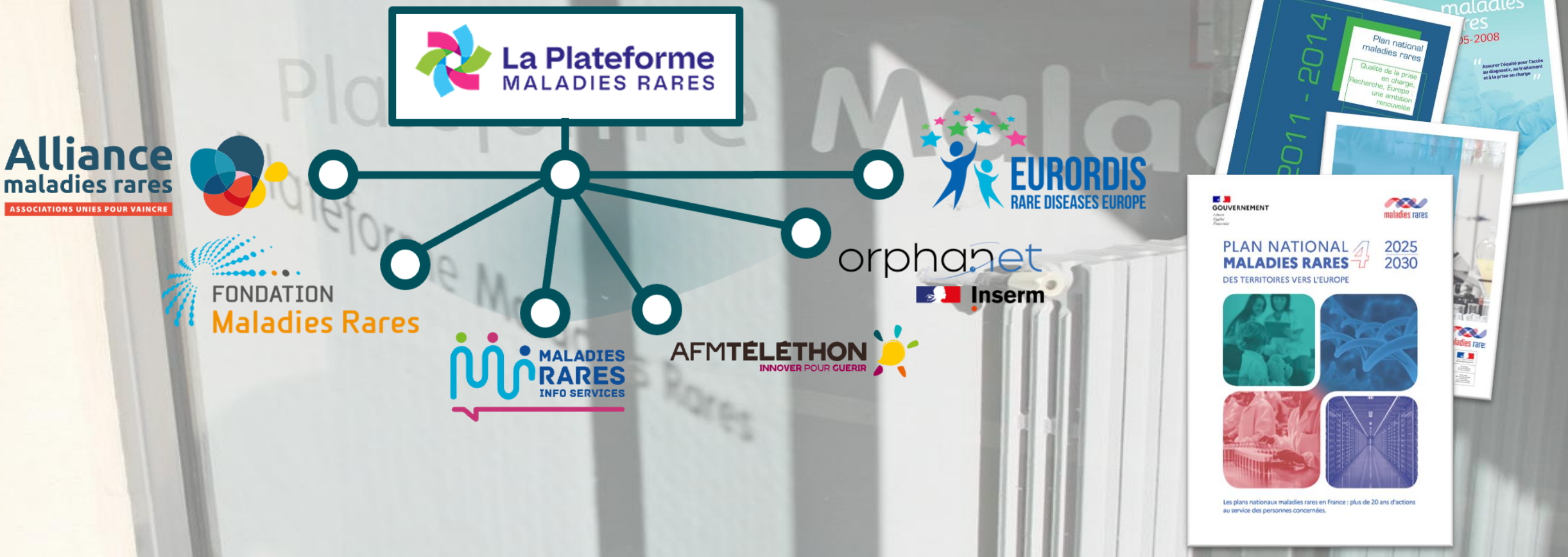


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## Decisive Rare Disease ecosystem support



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# Advancement of Treatments FOR RARE DISEASES

## DIAGNOSTIC



## RESEARCH



## CLINICAL CARE



## REGULATORY FRAMEWORK



What about?

IndustriAlization

Ultra rare diseaseS PATHWAY

EARLY ACCESS PROGRAMS

Innovative drugs FAIR PRICE

Social CARE

Patients place in governance

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# Advancement of Treatments FOR RARE DISEASES

**Patients Organizations must be equal partners  
European Union Rare Diseases Action Plan  
governance & operational execution**

**EU RD Plan must consider RD  
patients care in all its dimension**

**EU RD Plan must consider drug  
patient access in all its dimension**

Organisers:



Placing Patients At The Centre  
Of A Future European Framework

23 April | 9AM CEST

CY2026.EU  
CYPRUS PRESIDENCY  
OF THE COUNCIL OF THE EU



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