

# It is time to move mountains

## Third Multi-Stakeholder Symposium on Improving Patient Access to Therapies for Rare Diseases

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Chief Executive Officer

Brussels, 13 February 2019

**EURODIS.ORG**

In the next 15 minutes



# The ambition of EURORDIS and its members

## Breaking the Access Deadlock to Leave No One Behind

A Position Paper by EURORDIS and its Members

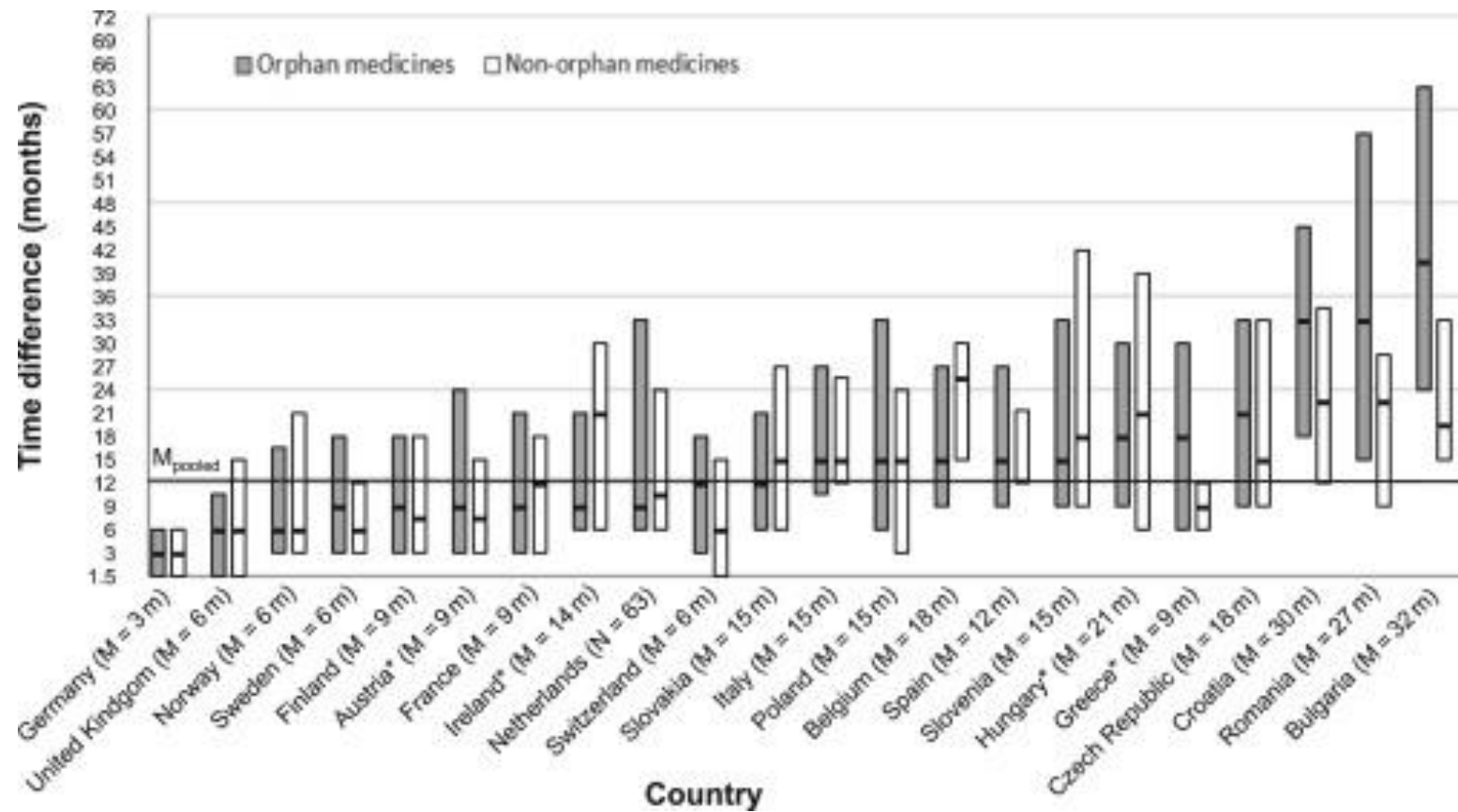
*“ The ambition of **EURORDIS** is to have  
3 to 5 times more new rare disease therapies approved per year,  
3 to 5 times cheaper than today by 2025 ”*

# In line with international ambitions



- 1. All patients coming to medical attention with a suspected rare disease will be diagnosed within one year if their disorder is known in the medical literature; all currently undiagnosable individuals will enter a globally coordinated diagnostic and research pipeline**
- 2. 1000 new therapies for rare diseases will be approved, the majority of which will focus on diseases without approved options.**
- 3. Methodologies will be developed to assess the impact of diagnoses and therapies on rare disease patients**

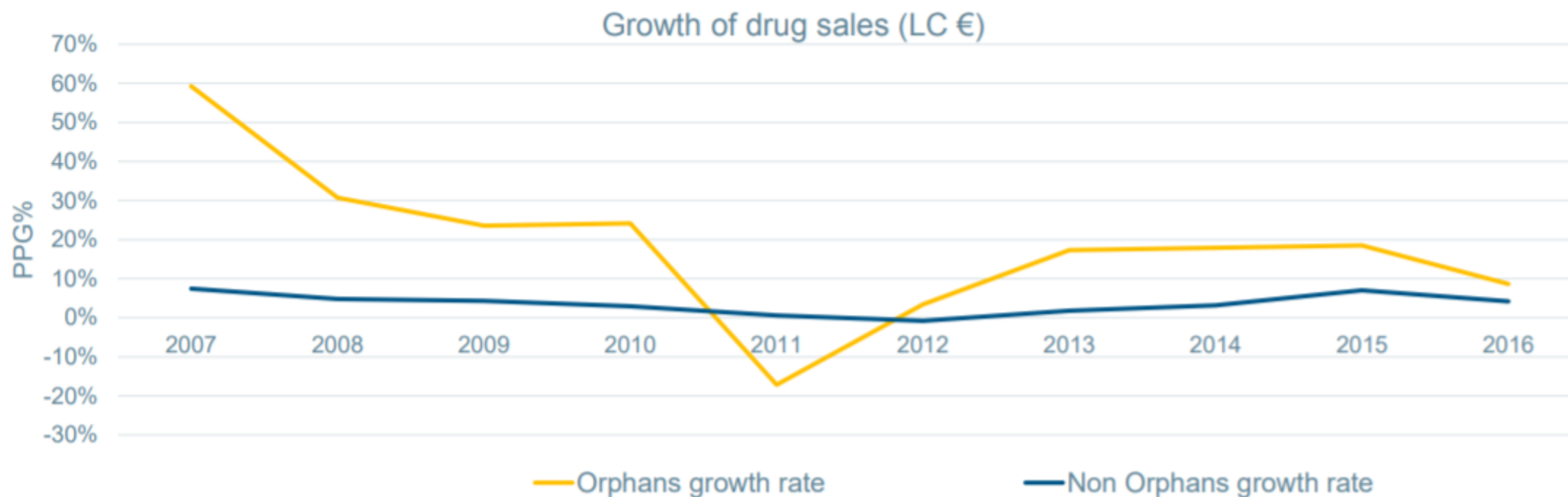
# Access remains a challenge



Value in Health 2018 21, 553-560 DOI: (10.1016/j.jval.2018.01.007)



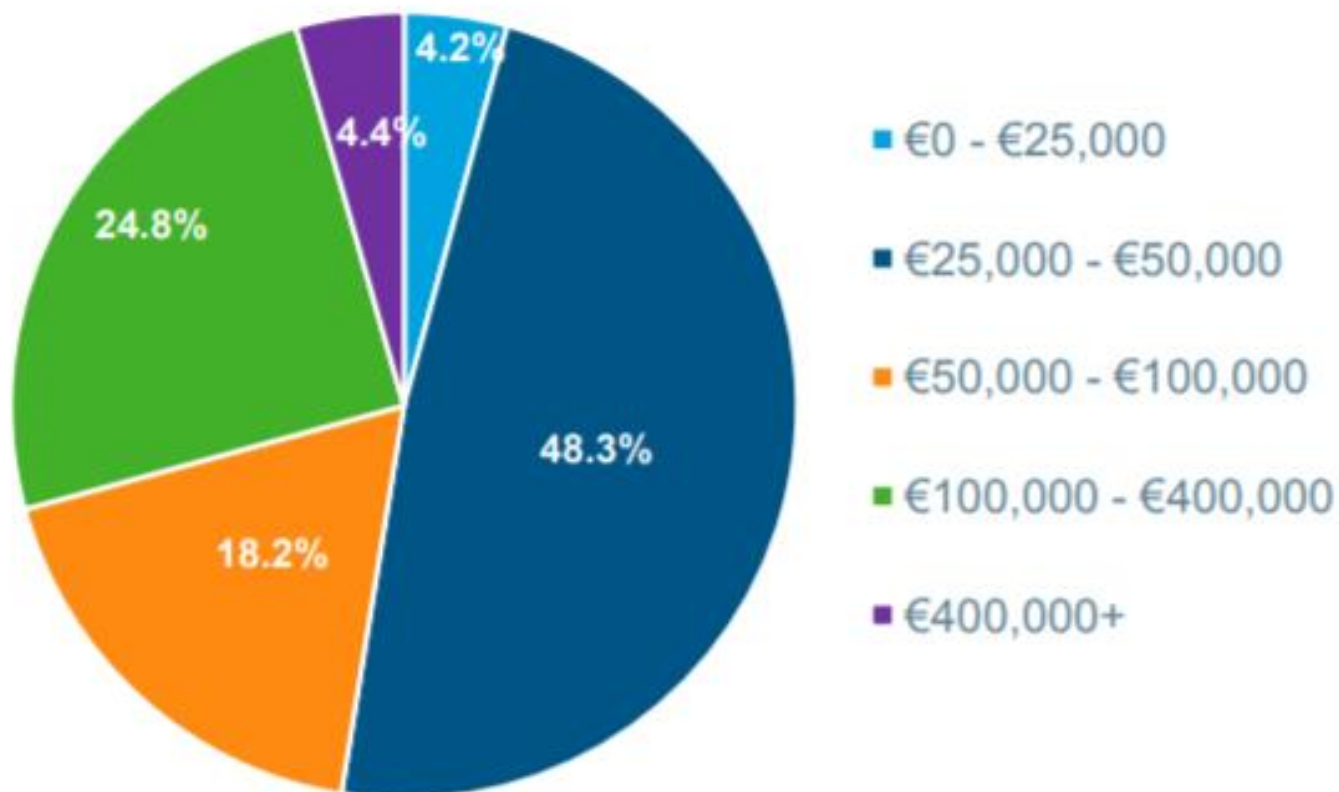
# Looking beyond the headlines: growth of OMPs spending in Europe has declined



Share of Drug Sales (€)	1.5%	1.9%	2.2%	2.7%	2.2%	2.3%	2.6%	3.0%	3.3%	3.5%
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Source: IQVIA (2017)

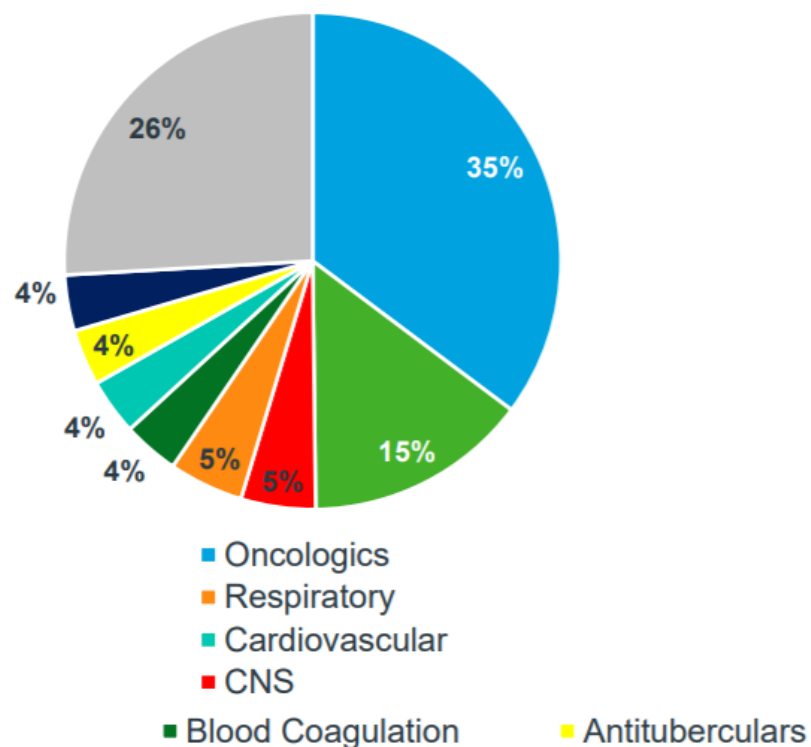
# High price medicines represents a fraction of OMPs



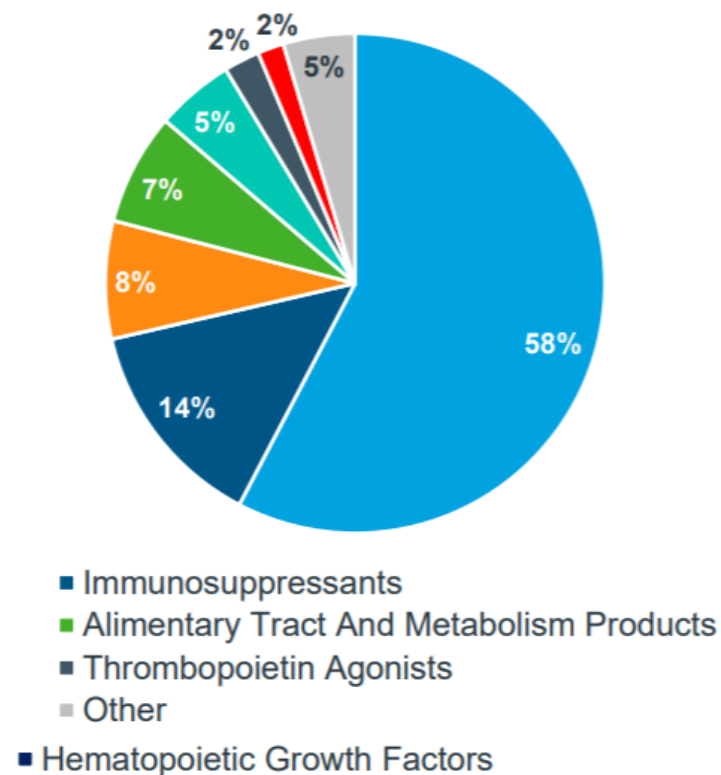
Source: IQVIA (2017)

# Expenditures is focused only in specific diseases areas

Number of Orphan drugs by therapy area in Europe, 2016



Orphan drugs sales by therapy area in Europe, 2016





# System failure and misconceptions are hampering access to treatments to those who need it the most

## Drugs

### Calls for action on patients denied £100,000 cystic fibrosis drug

Exclusive: Ministers urged to consider revoking patent on life-extending Orkambi, which NHS cannot afford

● The patients facing shorter lives due to drug's expense

Sarah Boseley  
Health editor

Sun 3 Feb 2019 23.11 GMT



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Maggie  
De Block



À PROPOS DE MAGGIE

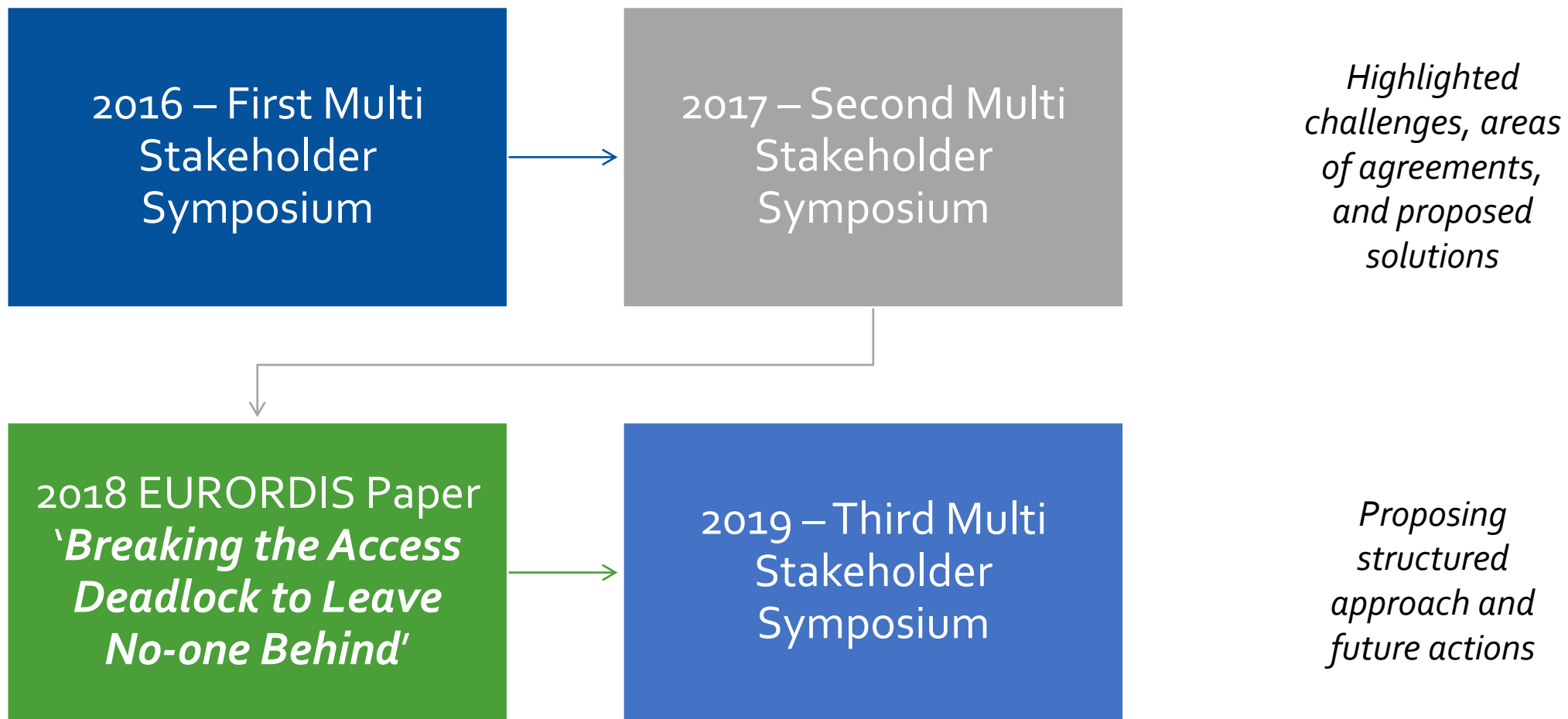
ACTUALITÉS

MERCHTEM

« PRENDRE UNE JEUNE PATIENTE EN OTAGE POUR FAIRE MONTER LE PRIX D'UN MÉDICAMENT, C'EST CRIMINEL »



# Where are we coming from



# What do we aim to achieve in the next two days?

3RD MULTI-STAKEHOLDER  
**Symposium**  
13-14 FEBRUARY 2019  
CROWNE PLAZA HOTEL-LE PALACE  
BRUSSELS, BELGIUM

ON IMPROVING  
**PATIENTS' ACCESS**  
TO RARE DISEASE  
**THERAPIES**



Let's make a pact to ensure patients' sustainable access  
to rare disease therapies

# Breakout sessions and related recommendations

*A New Blueprint to Spend Efficiently and Fast-track R&D*

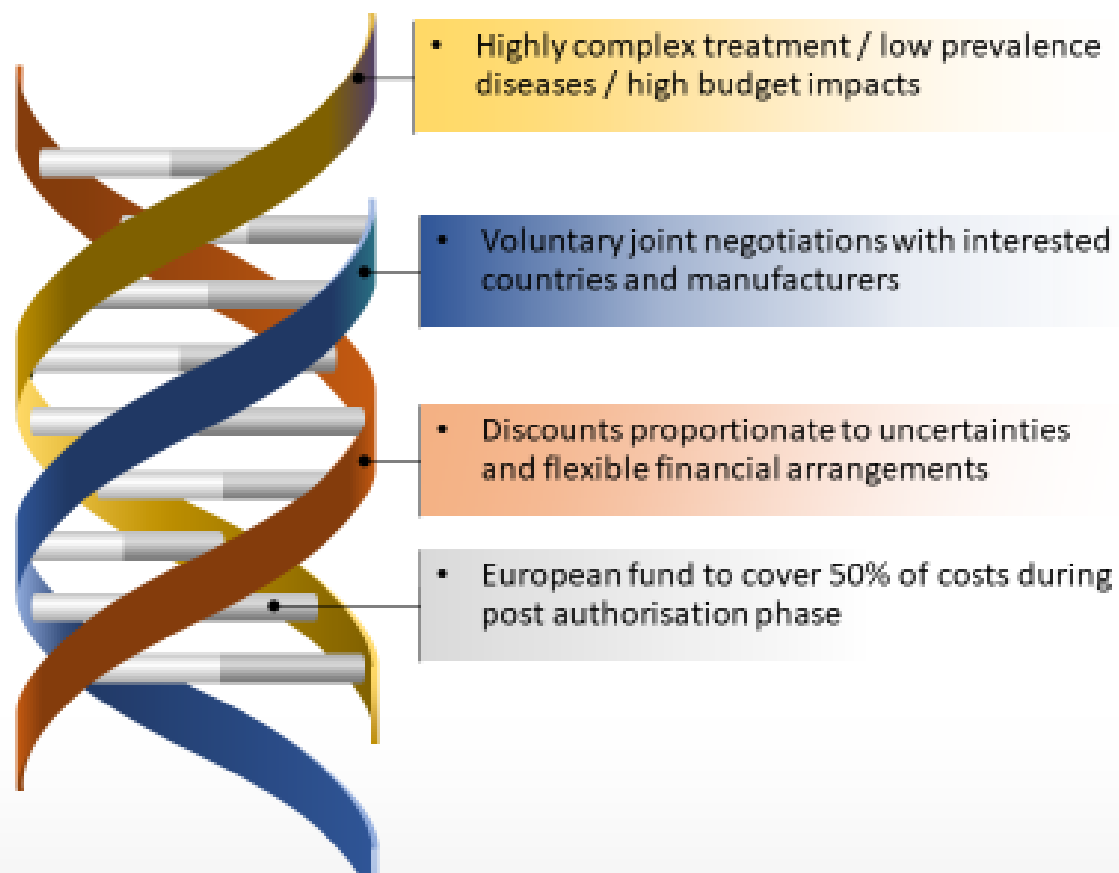
*A Continuum Approach to Evidence Generation Linked to Healthcare Budget Spending.*



*Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value*

*A Transparent European Cooperation Framework for the Determination of Fair Prices and of Sustainable Healthcare Budget Impacts*

# Moving forward – what we are suggesting



*A structured  
collaborative  
approach between  
ALL players  
involved*

Time for action is now



*The man who moves a mountain begins by carrying away small stones  
(Confucius)*



# THANK YOU

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