ACCESS TO POTENTIALLY CURATIVE THERAPIES?

Breakout 1 – parallel session

EMM 2021

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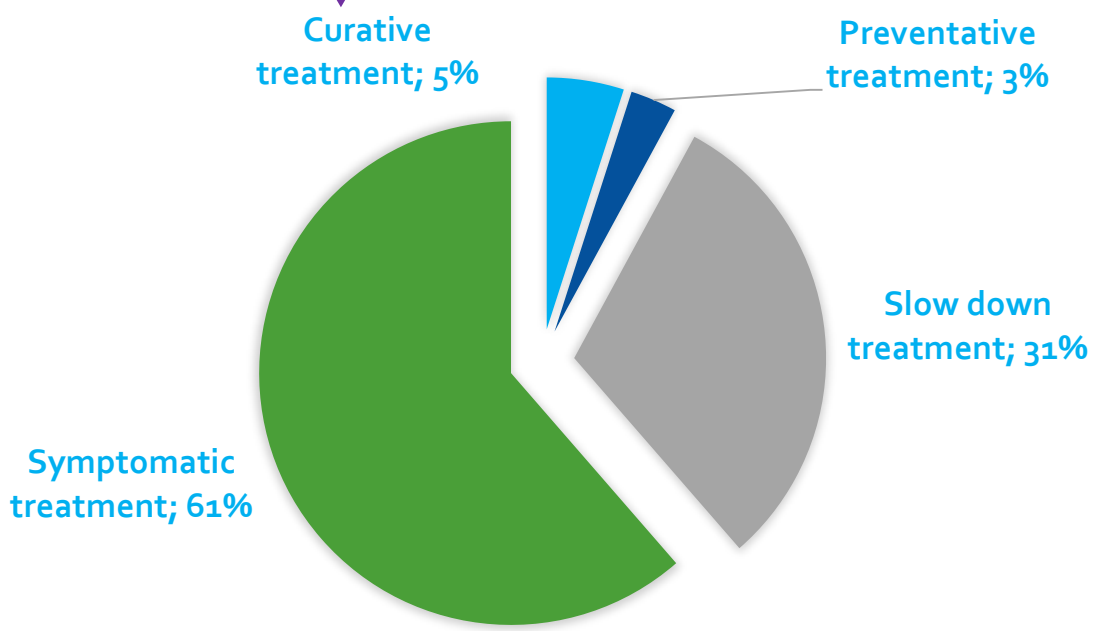
What are the challenges for people with rare diseases

69% have already experienced a treatment

31% have never experienced any treatment

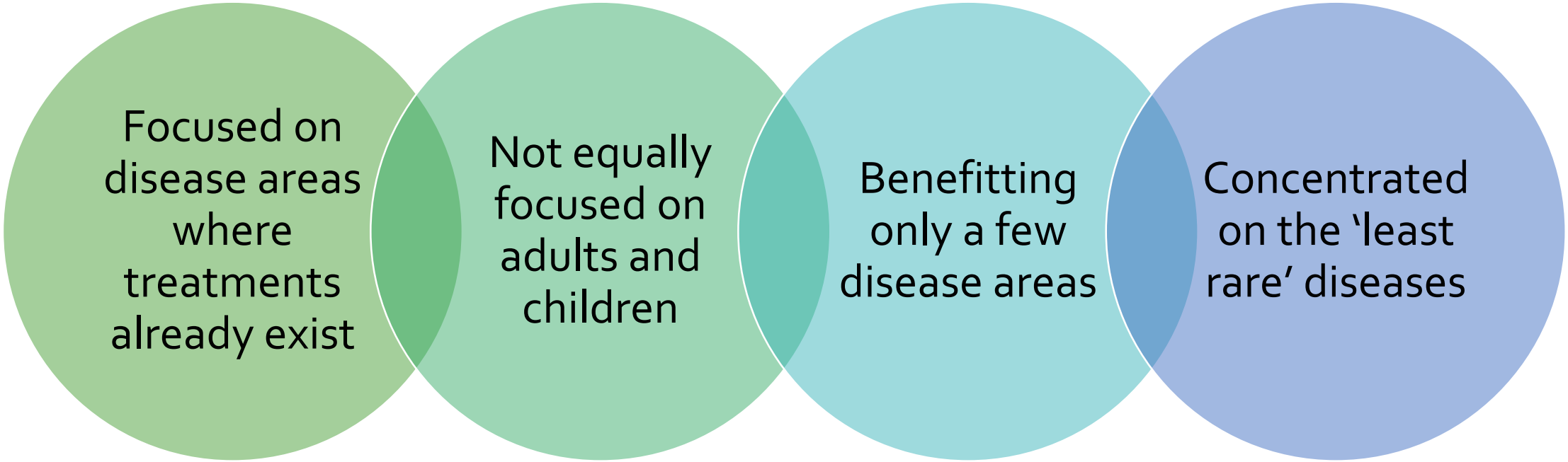


Problem statement

- The problem
  - Potential of science and technology currently not translated into actual health benefits
  - Issue concerning availability, accessibility and affordability

Coordinated, more strategic policies are required to address shortcomings and inequalities and ultimately improve the health outcomes and quality of life of people living with a rare disease.

Limitations of the current OMP development framework



What do we mean by Available, Accessible and Affordable Treatments?

By 2030

More and better quality curative, stabilising, palliative, assistive, rehabilitative and preventive technologies and therapies available, accessible and affordable

A European competitive ecosystem in the development of RD therapies and a more robust pharma and biotech manufacturing presence

1000 new therapies available

Therapies 3 to 5 times more affordable than current available treatments

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Rare 2030 Recommendations

The Triple A

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Our key vision

*Establish **streamlined regulatory, pricing and reimbursement policies**. These policies should encourage a continuum of **evidence generation** along the full life cycle of a product or technology as well as the patient journey from diagnosis to treatment access. A European ecosystem able to attract investment in **areas of unmet need, foster innovation, and address the challenges of healthcare system sustainability**.*

8 interconnected recommendations

1. *Long-term, integrated European and National Plans and Strategies*

2. *Earlier, Faster and more Accurate Diagnosis*

3. *Access to High Quality Care*

4. *Integrated and Person-Centred Care*

5. *Partnerships with Patients*

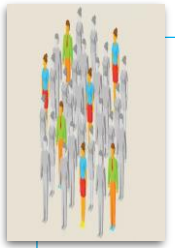
6. *Innovative and Needs-Led Research and Development*

7. *Optimising Data for Patient and Societal Benefit*

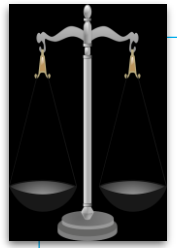
8. ***Available, Accessible and Affordable Treatments***

Key recommendations

early-stage multi-stakeholder identification of unmet needs and subsequent priorities and investments



a threshold of eligibility that includes incidence in addition to prevalence of 5/10000 individuals and avoids artificial breakdown of non-rare diseases;



a graduated system of incentives, rewarding earliest dialogue and areas with no therapeutic options yet;

a strengthened mandate for the Committee on Orphan Medicinal Products at the European Medicines Agency (EMA);

a functional and efficient EU Health Technology Assessment (HTA) Framework and in the interim increased uptake of joint EMA/HTA assessment at the European level;

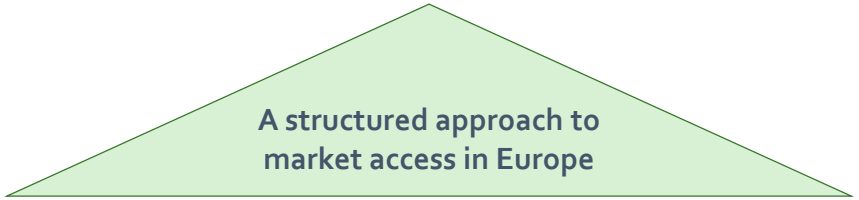
the introduction of a common European Table of Negotiations to allow for structured collaboration amongst Member

a continuum of comparative evidence generation throughout the patient journey and product/technology lifecycle collected in disease registries

An **EU-Fund to co-finance** the generation of evidence across EU Member States and reduce uncertainties during the first years following approval, for advanced therapies for the rarest diseases (affecting less than 1/100 000.)

EURORDIS suggested approach (2018)

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



Structured voluntary cooperation between healthcare systems in the European Union

PILLAR 1

A new blueprint to cut costs and fast-track R&D

PILLAR 2

Early dialogue and European cooperation on the determination of value

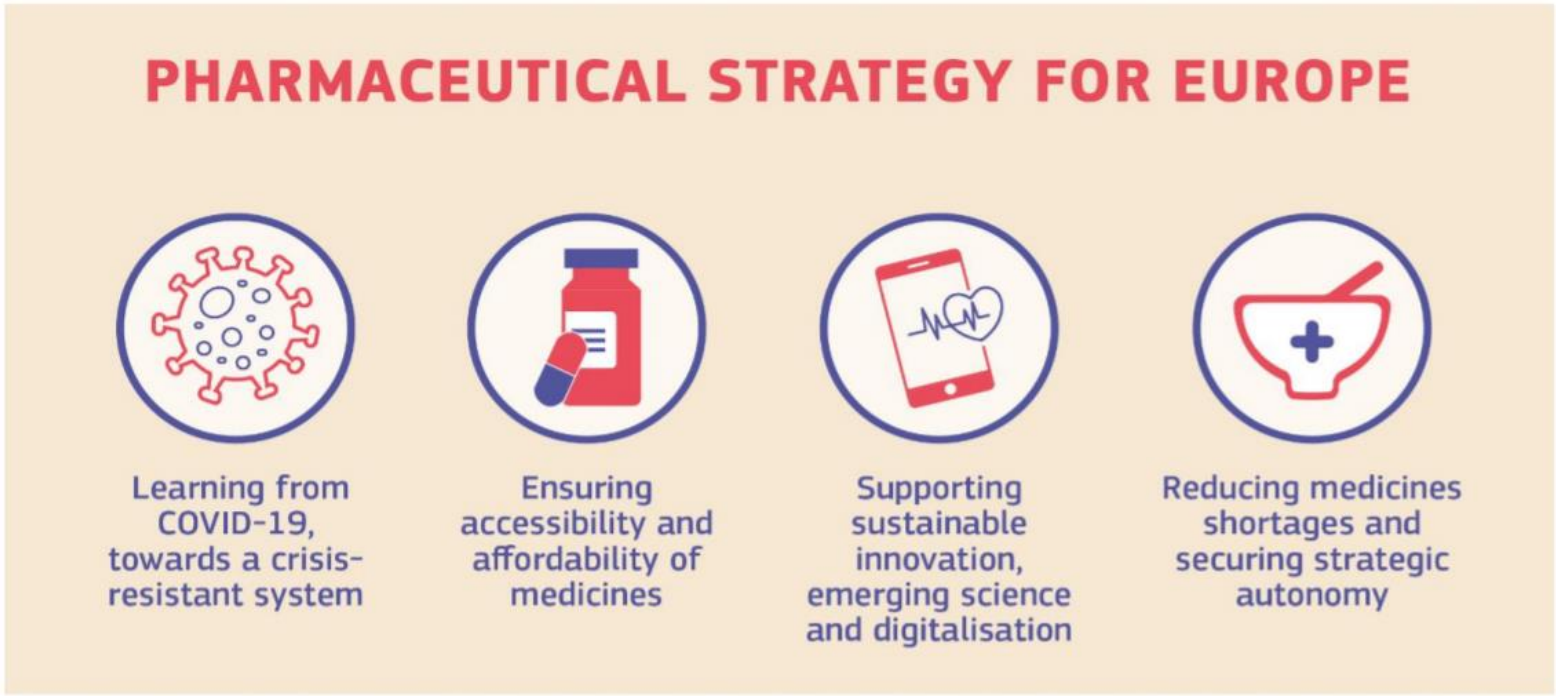
PILLAR 3

A European cooperation framework for fair prices and sustainable healthcare budgets

PILLAR 4

A continuum of evidence generation linked to healthcare budget spending

The policy opportunity



Some learning lessons from COVID

Collaboration before competition	Maturing of genome sequencing system	Massive use of data and computing to identify suitable candidate	Regulatory flexibility (namely, rolling data review)
Growing acceptance of other type of clinical trials	Procurement to match innovation with unmet need(s)	Mature scientific knowledge stemming from rare disease research	Investments (public, private and both)!

Access to potentially curative treatment

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RARE IMPACT: what has been done?

- Multi-stakeholders' consortium: mutual learning (working group meetings)
- Knowledge gathering and exchange (through literature review and engagement of external stakeholders)
- Engagement with patient organisations and policy makers (at national and EU level)
- Awareness raising on the challenges and solutions ideation on improved access to cell and gene therapies by people with rare diseases

RARE IMPACT.

Improving patient access to gene and cell therapies for rare diseases in Europe

www.rareimpact.eu

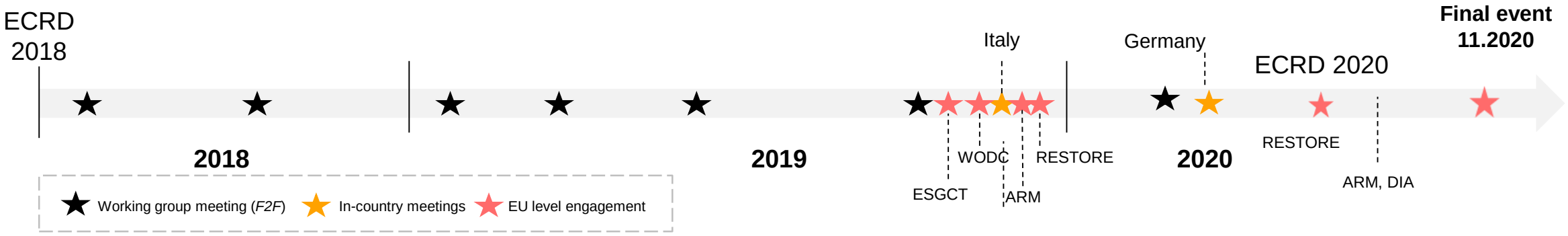
RARE IMPACT: 2018 - 2020 (PHASE 1)

Context

- ATMPs bring **hope** and **opportunity** to people living with a rare disease
- **Difficult patient access** to ATMPs
- Growing number of ATMPs (approval and pipeline), but just **a handful of patients have received treatment** with current ATMPs

Objectives

- Definition of the **challenges to patient access** to the advanced therapies “4 As” (**assessment, affordability, availability** and **accessibility**),
- Propose **actionable solutions** to address these challenges
- Engagement with **stakeholders** to ensure patients obtain better access to the gene and cell for rare diseases in Europe



Overview of the workstreams

WORKSTREAM		DESCRIPTION	LEAD BY
1		Price and the economics of ATMPs: Stakeholder engagement in support of patient access and innovation	Dolon
2		Evidence generation for ATMPs	EURORDIS
3		ATMPs and the criteria of selection for Centres of Expertise	EURORDIS

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THANK YOU

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