RARE IMPACT.

Improving patients' access to gene and cell therapies for people with rare diseases in Europe

RARE IMPACT initiative

1st International Conference on Rare Diseases
Rare Alliance Greece
2 March 2021

EURORDIS RARE DISEASES EUROPE

DOLON

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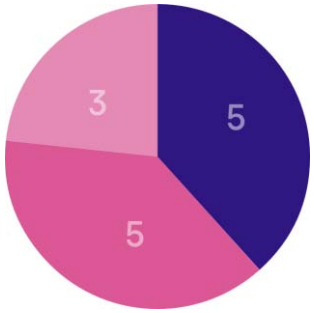
RARE IMPACT was founded in response to the patient access challenges to the first wave of ATMPs

Introduction

Why RARE IMPACT? | Who are we?

30 million Europeans are living with rare diseases and **72%** of rare diseases are **genetic in origin**

To date, **patients' access** to these advanced therapies has been **hampered** by both **practical and technical challenges**

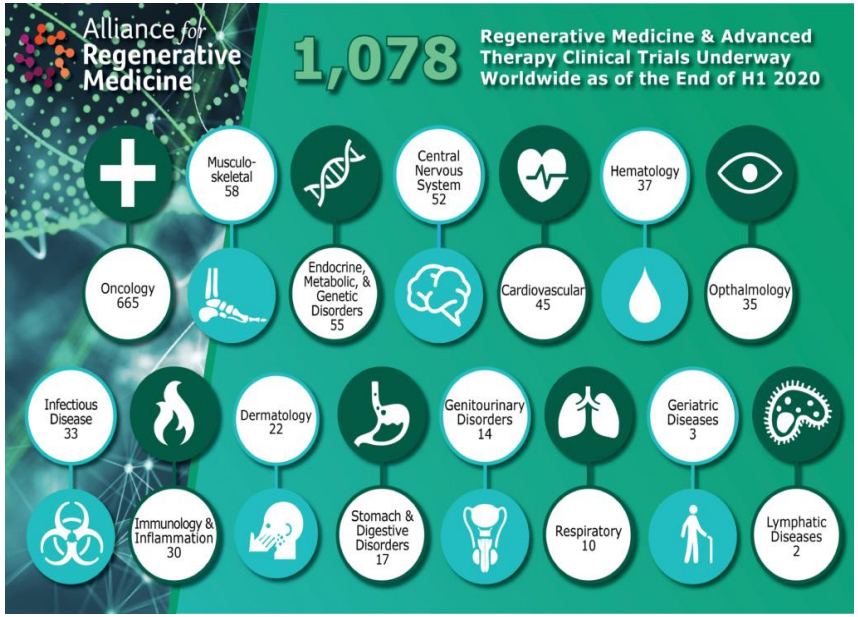


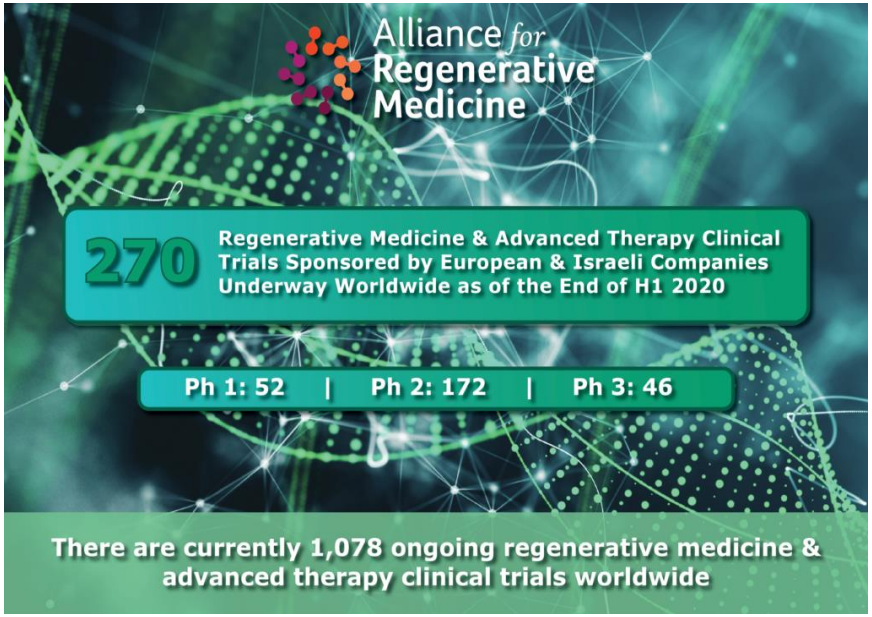
Gene and cell therapy availability – Jan '20
- Available in ≤ 3 European markets: 5
- Withdrawn from market: 5
- Available in > 3 European markets: 3

The ultimate ambition of the RARE IMPACT initiative is to optimise patient access to effective gene and cells therapies

Around 270 Advanced Therapies Clinical Trials ongoing in Europe
(*source: ARM, 2020*)

Why RARE IMPACT?





Of gene therapies up for approval over the next five years, 45% are anticipated to focus on cancer treatments and **38% are expected to treat rare inherited genetic disorders.**
(*source: ASGCT*)

RARE IMPACT is a consortium of manufacturers of gene and cell therapies and umbrella organizations

Few changes in RARE IMPACT phase II.

Introduction

Why RARE IPACT? | **Who are we?**

EURORDIS RARE DISEASES EUROPE + DOLON

Manufacturers:

BIOMARIN, NOVARTIS, Takeda, VERTEX, ultragenyx pharmaceutical, Sangamo THERAPEUTICS, Pfizer, Spark THERAPEUTICS, SAREPTA THERAPEUTICS, Chiesi People and ideas for innovation in healthcare, GILEAD Creating Possible, REGENXBIO, SANOFI, Celgene, bluebirdbio, AUDENTES THERAPEUTICS, PTC THERAPEUTICS, Orchard therapeutics

Non-profit organisations:

FONDAZIONE GENETHON CURE THROUCH INNOVATION, Telethon, AFMTELETHON INNOVER POUR GUERIR

Trade associations:

efpia European Federation of Pharmaceutical Industries and Associations

EUCOPE European Confederation of Pharmaceutical Entrepreneurs AISBL

Alliance for Regenerative Medicine

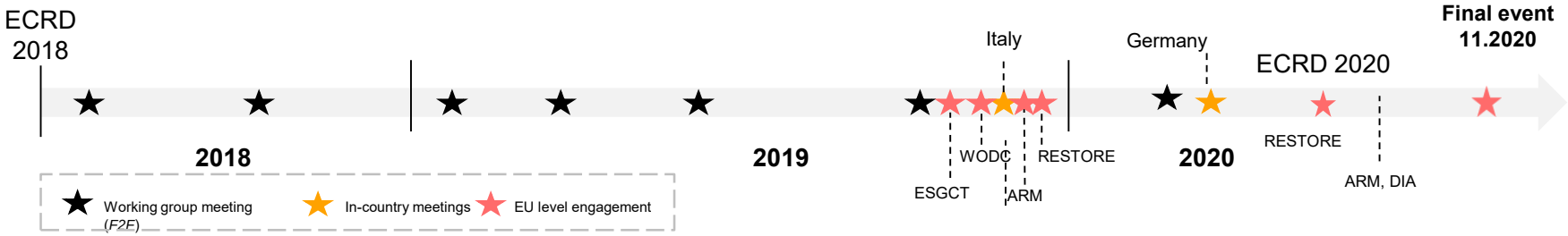
RARE IMPACT: 2018 - 2020 (PHASE 1)

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



RARE IMPACT: what has been done?

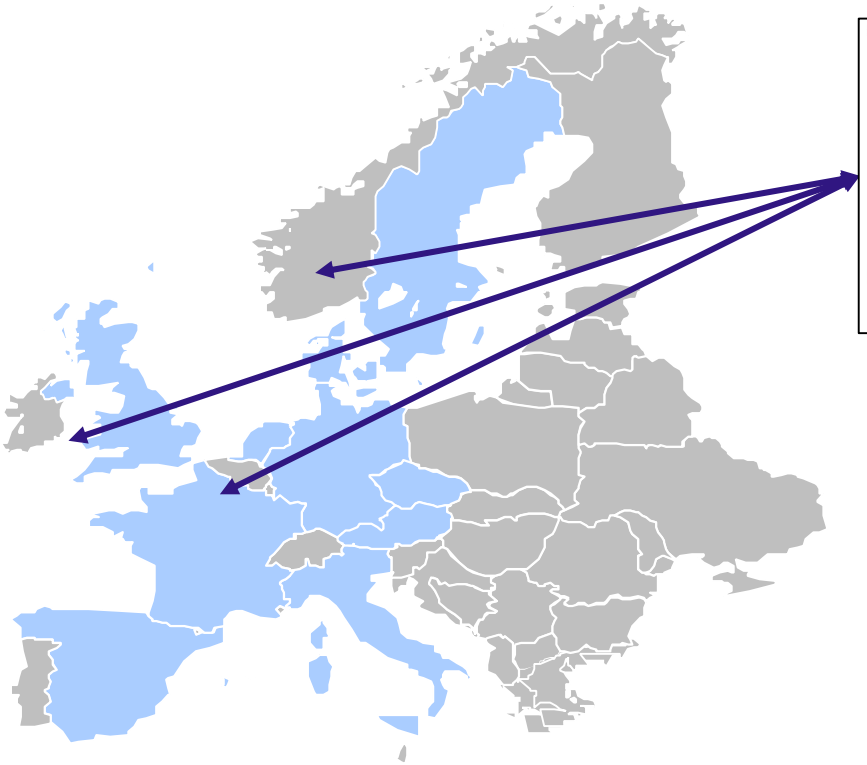
- Multi-stakeholders' consortium: mutual learning (working group meetings)
- Knowledge gathering and exchange (through literature review and engagement of external stakeholders)
- Engagement with patient organisations and policy makers (at national and EU level)
- Awareness raising on the challenges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

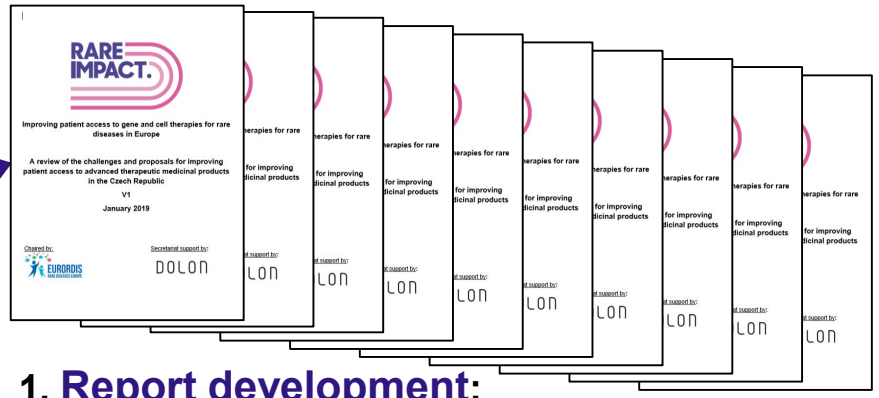
RARE IMPACT.

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

www.rareimpact.eu

RARE IMPACT achievement: 10 country reports





1. Report development:

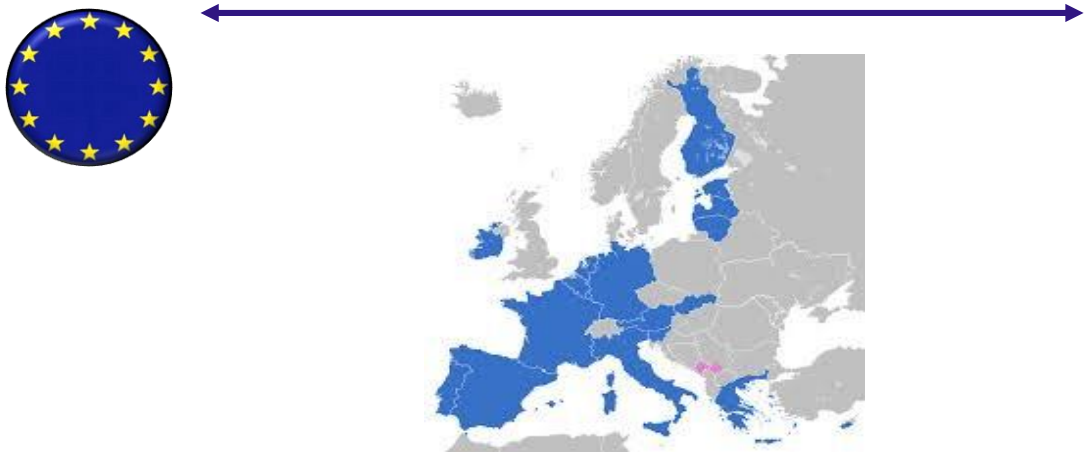
- Direct calls with country-level patient associations, payers, policymakers and academics (>35 interactions)

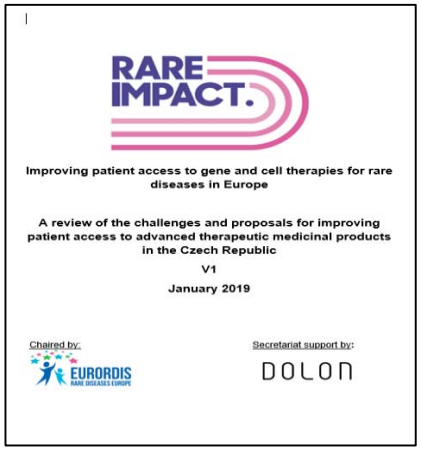
2. Report sharing:

- Calls and meetings with country-level patient associations
- Multi-stakeholder in-country meetings
- Discussions to be continued…

Geographic scope

RARE IMPACT: European Report presenting the challenges and solutions





- RARE IMPACT **policy solutions** to improve patient access to advanced therapy medicinal products at **EU level**
- RARE IMPACT **final event** of 23 November 2020: a **call to EU stakeholders to take forward the solutions in a dialogue** to improve access to the advanced therapies

EU REPORT: There is a gap between regulators and HTA bodies in evidence considerations

Assessment | Affordability | Availability | Accessibility

CHALLENGE

Gap between Regulators and HTA:

1. Assessment methods
2. Registry requirements

SOLUTIONS

1 **European-level guidance on ATMP assessment methods**

2 **Multi stakeholder collaboration on development and use of registries and real-world datasets**

Affordability challenges can be overcome through collaboration on innovative approaches

Assessment | **Affordability** | Availability | Accessibility

CHALLENGE

1. **Barriers to annuity payments and innovative payment options**
2. **Concern about sustainability of ATMP prices and innovation model**

SOLUTIONS

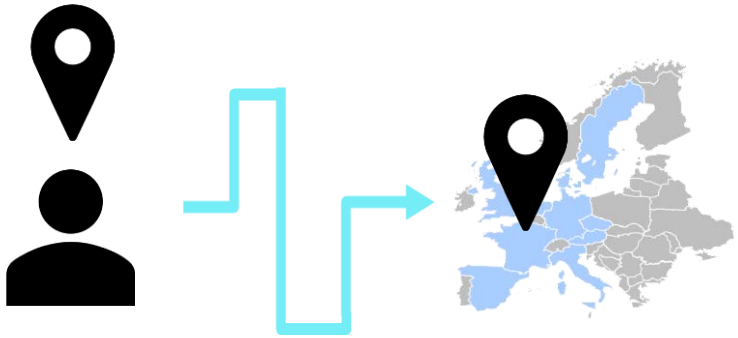
1. **Work with stakeholders at the national level to remove barriers to annuity and innovative payment models**
2. **Explain and discuss the ATMP innovation model with all stakeholders**

Availability challenges require EU level guidance to ensure equitable access to approved therapies

Assessment | Affordability | **Availability** | Accessibility

CHALLENGE

1. Variability in use of cross-border healthcare legal provisions
2. Inconsistent implementation of hospital exemption legislation



SOLUTIONS

1. Issue guidance on cross-border pathways at the European level
2. Seek clarification from European Commission on intention and implementation of hospital exemption

Certifying ATMP centres is critical: standardised approach needed to avoid delaying patient access

Assessment | Affordability | Availability | **Accessibility**

CHALLENGE

1. There are variable ATMP certification requirements for centres of excellence

SOLUTIONS

1. Assess and educate around the existing system in place for treatment centre qualification

2. Encourage stakeholder dialogue to explore options to reduce administrative burden and ensure consistency across Member States

RARE IMPACT (PHASE 2): Overview of the 3 new workstreams

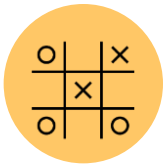
WORKSTREAM		DESCRIPTION	LEAD BY
1		Price and the economics of ATMPs: Stakeholder engagement in support of patient access and innovation	Dolon
2		Evidence generation for ATMPs	EURORDIS
3		ATMPs and the criteria of selection Centres of Expertise	EURORDIS

Overview of workstream 1

Price and the economics of ATMPs: Stakeholder engagement in support of patient access and innovation

GOAL

Enabling a stakeholder dialogue in support of patient access and innovation



APPROACH

Phase 1: Explore and explain the economics of innovation and price setting for ATMPs

Phase 2: External engagement for collective exploration of ATMP economics & innovation

DELIVERABLES

1. **Narrative document**
2. **Interactive learning exercises**
3. **Supportive workshop materials**

Workstream 2: Evidence generation and ATMPs

Opportunity:

ATMPs are **innovative, potentially transformative therapies** for patients with high unmet need

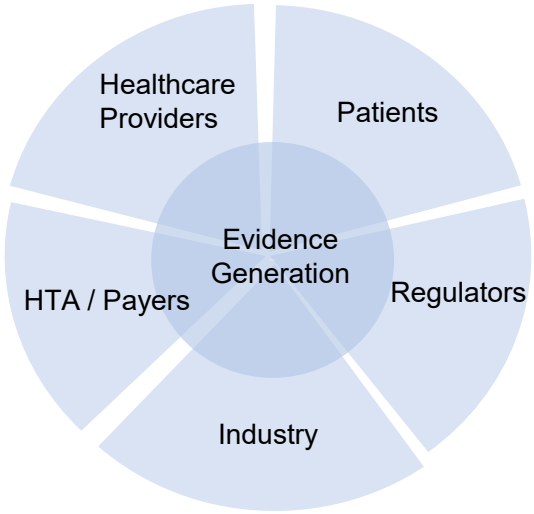
Challenge:

High level of uncertainty surrounding the value of ATMPs for all stakeholders:

- Small sample of treated patients
- Long-term outcomes not well-established pre-approval

What evidence is required to establish value of ATMPs?

Need for alignment between stakeholders on evidence base



Path to ATMP access

- **Efficient**
- **Predictable**

Deliverables:

- **Overview of the uncertainties** that impact the multiple stakeholders
- Recommendations for **alignment on standards for evidence generation**
- Recommendations for **improved coordination of registries** to support ATMP evidence base

Workstream 3: ATMPs and the criteria of selection Centres of Expertise

Approach:

- To **gather knowledge on the process for the selection** of CoEs that deliver ATMPs (at hospital, company, national and EU levels)
- To conduct **mapping exercise and research to comparison of the quality and technical criteria** for cell, tissue, cell-gene and gene products
- To develop a proposal a **core set of quality and technical criteria** (general and specific) in order to promote greater vertical alignment from regulatory to provider requirements

Final scope:

- To **compare the regulatory and manufacturers requirements** for Centres of Expertise that deliver ATMPs to identify the common and divergent approaches, for **quality, safety and disease specific knowledge** (short-term)
- To promote **better alignment between requirements and criteria** set between regulatory and manufacturers (long-term)

Thank you for your attention!