ACCESS TO ORPHAN MEDICINAL THERAPIES

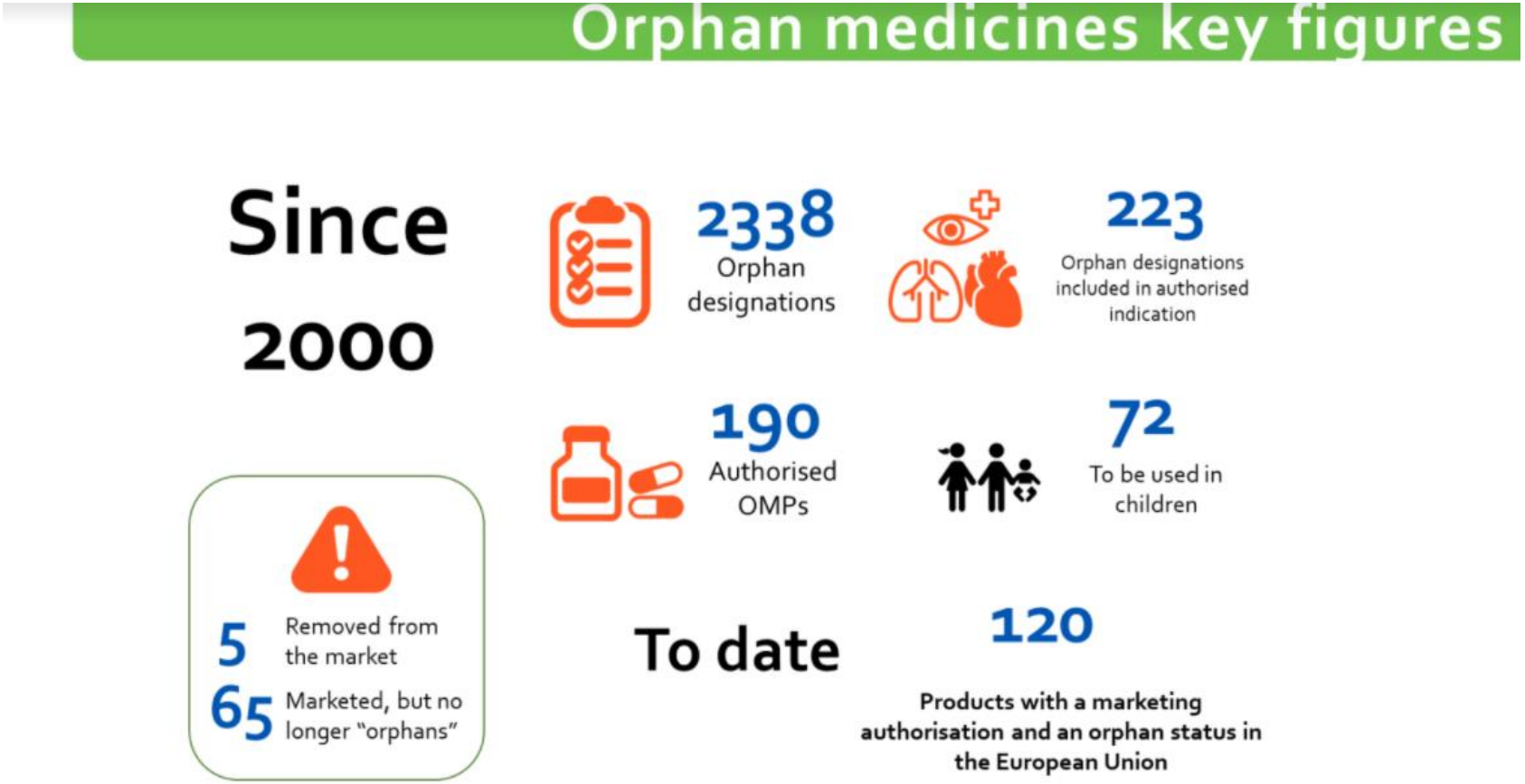
Where do we stand?

CNA / CEF meeting 27 November 2020

Simone Boselli

EURORDIS.ORG

Where are we now?



Rare disease patients' experience of treatment

Rare Barometer Voices

A EURORDIS INITIATIVE

7500 respondents :

- Respondents were able to take the survey from any country of the world
- Survey was translated in **23 languages** **91 different countries** represented

Most treatments available for rare disease patients are symptomatic treatments

69% have already experienced a treatment

31% have never experienced any treatment

Only **5 %** have already experienced a **curative** treatment

3% a treatment to **prevent** the disease

31% a treatment to **slow down** the disease

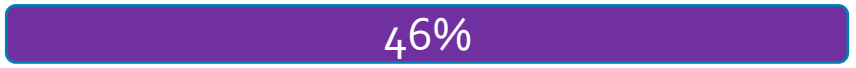
62% a symptomatic treatment

A significant part of treatments experienced by patients have not been approved yet

Among treatments mentioned by rare disease patients in this survey…

were **experienced within the context of a clinical trial**

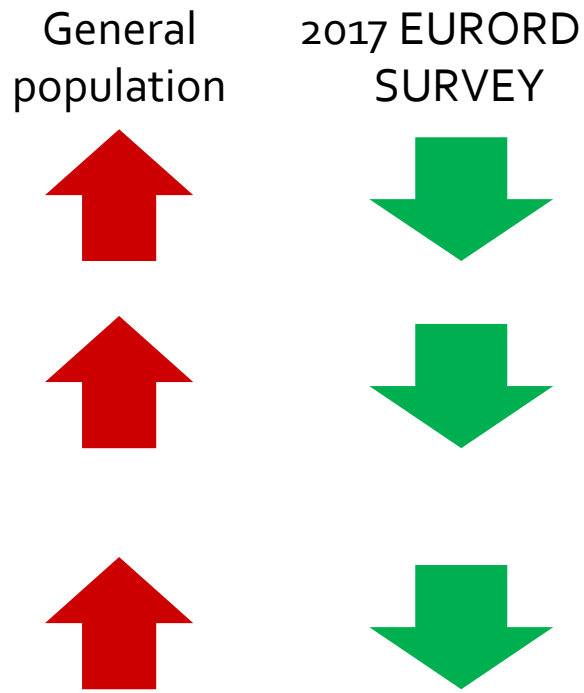
were **received through compassionate use programmes**



were **initially meant to cure a different disease**

Access to treatment is limited as compared to the general population but improved since 2017

- over the previous 12 months **22%** did not get the medical treatment they needed because **the treatment was not available in their country.**
- **14%** of rare disease patients that responded to the survey were **prevented from accessing the treatment they need because the waiting list was too long**.
- Over 10% of respondents said that they could not access the treatment they needed in the past 12 months because **they did not get the financial support to travel and receive the treatment in another country.**
- **Eastern Europe** stands out as the region where rare disease patients are facing the most difficulties in accessing treatments



Evaluation of OMP & Paediatric regulation

Key findings:

1.thanks to the treatment with medicines for rare diseases, **patients benefited from an improvement in their quality of life**; and
2. the **benefits the legislation brought for children appear to outweigh the costs imposed on both industry and society**

Both regulations have fostered the development and availability of medicines for patients with rare diseases and for children. They have redirected private and public investment towards previously neglected areas through incentives, obligations and rewards. Member States alone could not have achieved this result due to the small number of patients concerned and the fragmentation of the market.

The number of medicines for patients with rare diseases and for children has increased. Medicines for patients with rare diseases have also become available faster and have reached a higher number of patients in the Member States.

The Regulation on medicines for children increased the number of clinical trials in children and, consequently, the development of new medicines for them. It reduced the 'off-label' use of medicines for adults in children, which were not specifically tested or adapted for use in children ('off-label' use) and favoured the creation of a 'paediatric research environment' in Europe.

The evaluation found that **incentives remain relevant to encourage the development of medicines for rare diseases**.

Both regulations have not adequately managed to support development in areas where the need for medicines is greatest. Products tend to be developed in certain more profitable therapeutic areas for which the number of available treatments is increasing. **The evaluation questions focus on whether the threshold of fewer than 5 in 10 000 patients is the right tool for identifying rare diseases which need specific support in medicines development**.

The development of new medicines for children remains mainly driven by adults' needs. As a result, it does not necessarily address the greatest therapeutic needs of children

For some rare diseases the market has started to look more similar to 'standard' medicines. Hence, in such cases the EC argues it could be questionable whether a 10-year market exclusivity is justified.

For medicines for children, the cost of conducting clinical studies in children can be compensated by extending the patent (Supplementary Protection Certificate, 'SPC'). **While this reward is partly fulfilling its role, it has not shown to be effective in stimulating the development of medicines whose development for adults is not attractive.** Obtaining this reward may be complex, as companies have to request it individually at the various national patent offices.

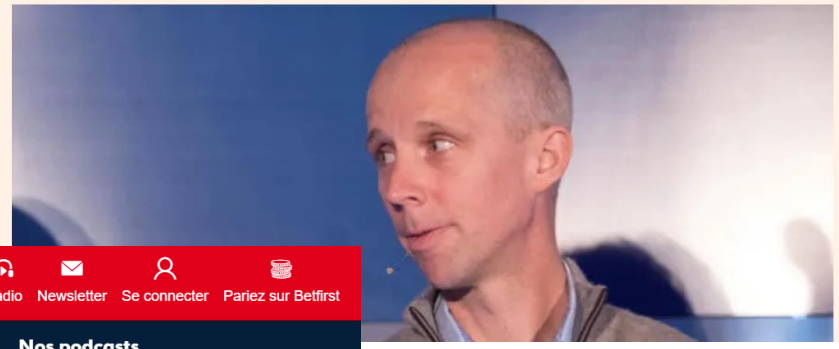
Promising science for transformative treatments

Affordability, value assessment and high prices remain a critical issue

Drug prices + Add to myFT

Bluebird Bio prices gene therapy at nearly $1.8m

Boston-based biotech start-up will only seek full payment if the treatment works



LES SPORTS+ Journal Alertez-nous Radio Newsletter Se connecter Pariez sur Betfirst

Actu | LES SPORTS+ | Régions | Conso | Médias | Lifestyle & people | Buzz | Nos podcasts

L'appel à l'aide d'un couple de Rebecquois: "Il ne reste que quelques semaines pour sauver la vie de Maciek, 2 ans"

Société | Interview

Simon Legros

Publié le 26-11-20 à 13h46 - Mis à jour le 26-11-20 à 14h29

Comme la petite Pia, Maciek est atteint de "SMA", le syndrome de l'amyotrophie spinale. Et ses jours sont comptés.

EURORDIS initiatives - an update

RARE IMPACT.

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

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

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 THROUGH 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: why?

Context

- Difficult patient access for rare disease treatments
- Gene and cell therapies face particular difficulty
- To date, just a handful of patients have received treatment with current ATMPs

Objective

- Define the challenges to patient access to the advanced therapies
- Propose actionable solutions to address these challenges
- Prepare stakeholders to the challenges
- Propose solutions

VISION

To ensure patients obtain quick access to the gene and cell for rare diseases in Europe and to create a sustainable model for manufacturers and payers

RARE IMPACT: what have we done?

Phase 1
Challenge Identification

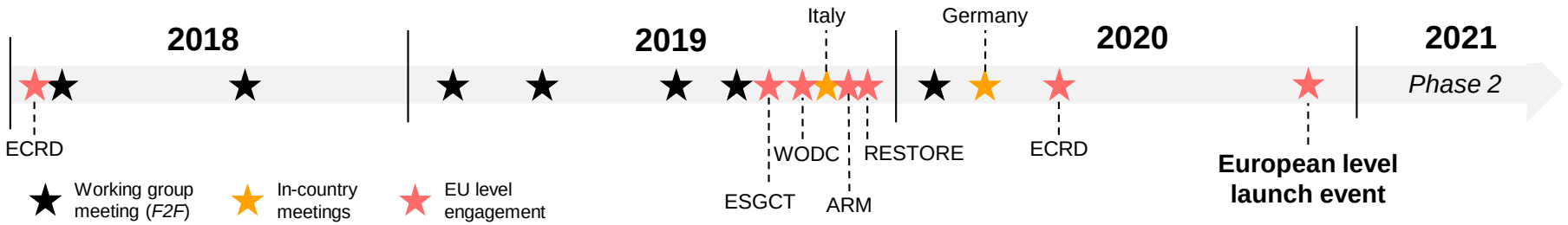
- Primary and secondary research to identify challenges
- Discuss challenges with external stakeholders

Phase 2
Solution Ideation

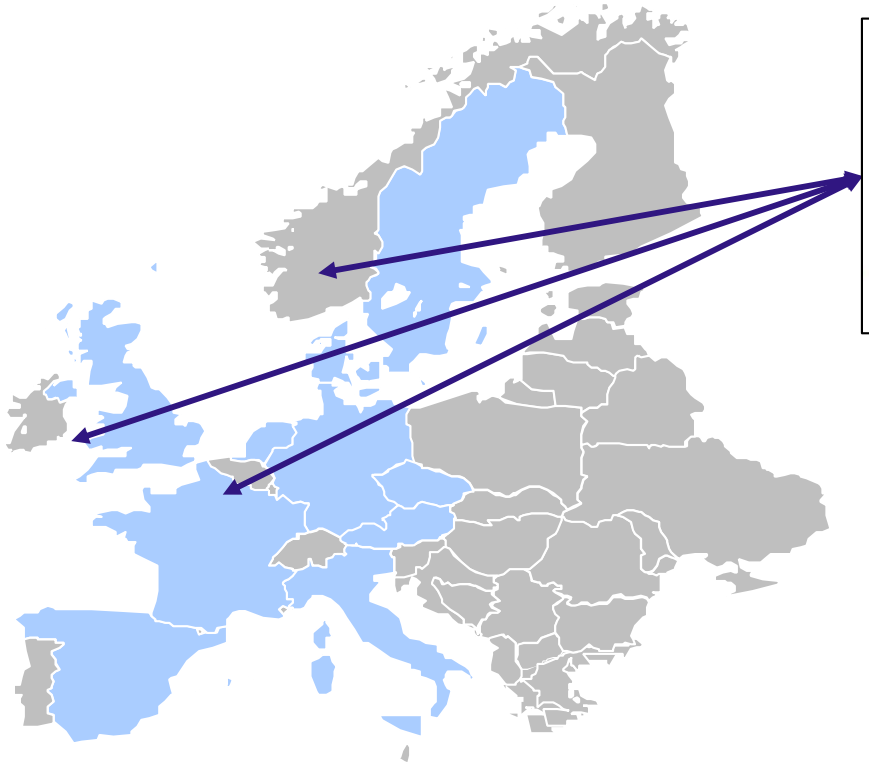
- Summarise relevant conceptual solutions from literature
- Develop European and country level specific solutions
- Validate solutions with external stakeholders

Phase 3
Solution Implementation

- Develop engagement plan and identify engagement materials/tools
- Present the solutions to decision-makers across EU countries



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

Final Agenda

Moderation: **Simone Boselli,** EURORDIS-Rare Diseases Europe

14h00 - 14h05 Welcome: **Yann Le Cam,** EURORDIS-Rare Diseases Europe

14h10 - 14h20 Patient perspective: **Avril Daly,** Retina International

14h20 - 14h30 RARE IMPACT: overview of initiative, results and recommendations: **Karolina Hanslik,** EURORDIS and **Adam Hutchings,** Dolon

14h30 - 15h20 **Stakeholders' views on the RARE IMPACT recommendations:**

- Patients: **Dr Mariette Driessens,** Dutch Genetic Alliance-VSOP
- Researchers: **Prof. Hans-Dieter Volk,** Charité-Universitätsmedizin Berlin
- Patients: **Dr Hervé Nabarette,** AFM-Téléthon
- Industry: **Tresja Bolt,** Bluebird bio

Short poll about the RARE IMPACT solutions and possible short break

- HTA: **Marcus Guardian,** EUnetHTA Joint Action
- European Commission: **Rocio Salvador Roldan,** DG SANTE
- European Medicines Agency: **Dr Xavier Kurz,** Task force on registries
- European Parliament: **MEP Katerina Konecna,** GUE/NGL

15h20 - 15h50 **Panel discussion and questions**

15h50 - 16h00 **Conclusions: Yann Le Cam,** EURORDIS-Rare Diseases Europe

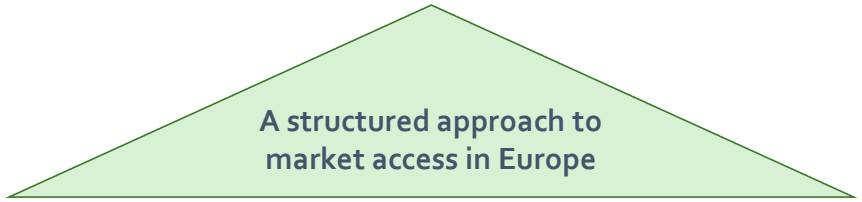
Final report addresses the 4As of ATMPs

The report addresses challenges across four identified areas in the **accessibility, assessment, availability and affordability** of gene and cell therapies across the European Union. It highlights seven solutions , including calls for:

- Greater collaboration between EMA, HTA bodies and Heads of Medicines Agencies on guidance on HTA assessment;
- Coordinated approach on the development and use of registries serving multiple purposes (e.g. the follow up of patients, assessment and reimbursement);
- Greater cooperation and clarity on use of the cross border healthcare provisions;
- Better information and explanation on the cost and value of the advanced therapies; and
- Further discussions to remove barriers to innovative payment mechanisms.

Breakind the Access Deadlock – a reminder

> *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*

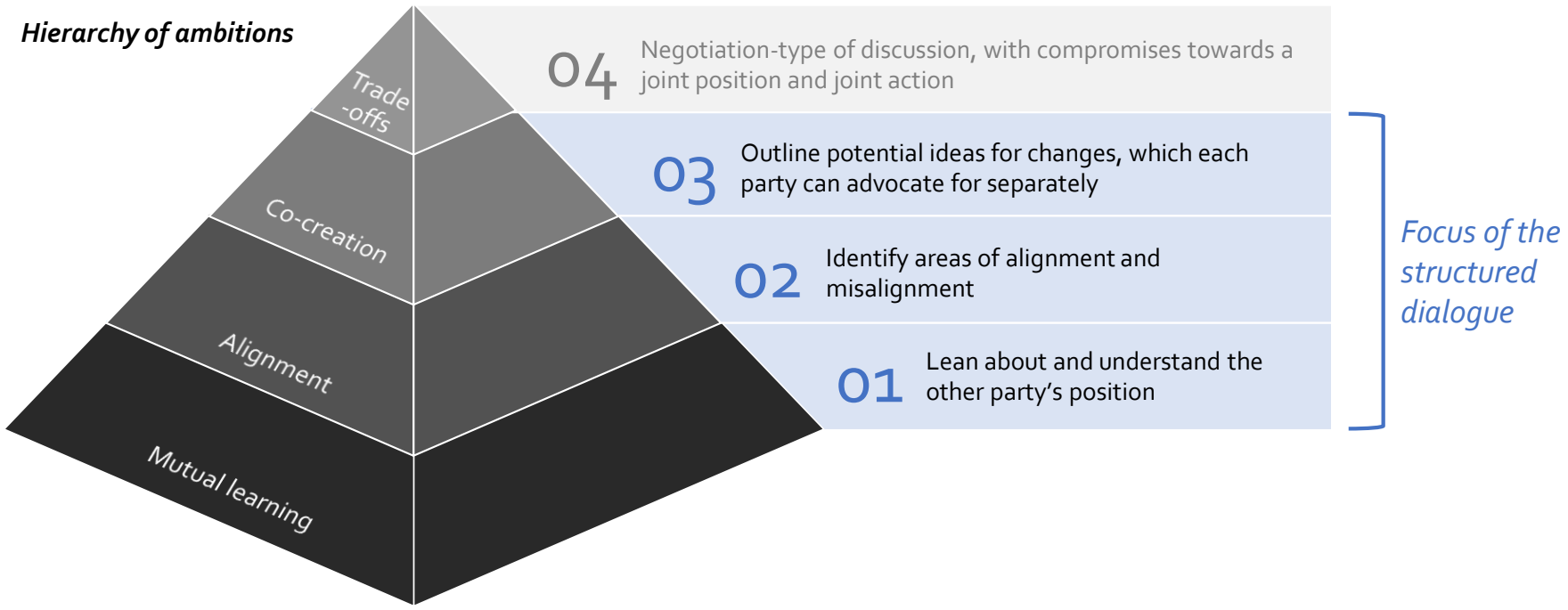


Structured voluntary cooperation between healthcare systems in the European Union

PILAR 1	PILAR 2	PILAR 3	PILAR 4
A new blueprint to cut costs and fast-track R&D	Early dialogue and European cooperation on the determination of value	A European cooperation framework for fair prices and sustainable healthcare budgets	A continuum of evidence generation linked to healthcare budget spending

Structured dialogue with EFPIA

- **The intention of the dialogue is to identify alignment and opportunities to improve access for rare disease patients across Europe**
- **The dialogue will not result in joint positioning**



Topic of the first dialogue

	Scope of the first dialogue	Key questions
Topic 1: **Managing evidential uncertainty: use of real-world evidence and adaptive processes**	**Areas of focus:** • Use of real-world evidence to inform payer decision-making • Adoption of iterative value assessment processes to manage uncertainty **Topics excluded** • Adaptive frameworks for regulatory assessment (e.g. conditional approval) • Link between iterative value assessment and pricing	**How can RWE collection be optimised at EU-level?** • *Should RWE be collected at EU-level? Through which mechanism(s)?* • *How should RWE generation be funded? Would the creation of a 'EU Fund for Evidence Generation' stand to improve patient access in rare diseases?* • *Should experiments (e.g. TRUST4RD, RWE4DECISIONS) be scaled up? How so?* • *Of the key drivers / barriers to RWE, which can be leveraged / addressed by EURORDIS and/or EFPIA?* **How can adaptive processes be used to leverage RWE?** • *Is it desirable and feasible to strive for an iterative HTA process?* • *Should adaptive processes occur at country or EU – level? (to be discussed along with topic 2)* • *Of the key drivers / barriers to adaptive processes, which can be leveraged / addressed by EURORIDS and/or EFPIA?*

Topic of the first dialogue (2)

	Scope of the first dialogue	Key questions
<u>Topic 2</u>: **Enhancing multi-country or EU collaboration on value determination**	**Areas of focus:** • Multi-stakeholder dialogues for early value determination, e.g. MoCA • Multi-country or pan-European processes for value assessment / HTA **Topics excluded** • Regulatory processes • Joint pricing negotiations • Joint procurement	**Overarching question: If a mandatory, EU-wide HTA is not to happen, what would an alternative be?** **1. Could a non-binding, EU-wide initiative (e.g. MoCA) be an effective alternative to the EU regulation on HTA?** • *Has MoCA contributed to improving access? How?* • *What can be learnt from experience with MoCA?* • *What are limitations to MoCA's impact to access?* • *How can MoCA's impact on access be increased?* • *Should MoCA be scaled up? How?* • *Can voluntary collaborations lead to large impacts on patient access in rare diseases?* **2. Is there an opportunity for smaller groups of countries to engage in formal joint HTA?** • *To date, have small-scale joint HTA initiatives contributed to improving access? How?* • *Of the key drivers / barriers to multi-country collaborations, which can be leveraged / addressed by EURORIDS and/or EFPIA?*

Note that the discussion will be limited to collaboration for clinical benefit assessment.
The link between joint HTA and joint negotiations or joint procurement will not be explored

Proposal for expert group on pharmaceutical strategy

Rationale

In the context of the Pharmaceutical Strategy, the recent evaluation of the OMP & Paediatric Medicines regulation, and the persistent challenges in access, we believe we need to have in place an extended team supporting EURORDIS staff in analysing the situation, proposing new solutions, and fostering dialogue

Mandate

- To support EURORDIS Staff in analysis of OMPs evaluations & definition of potential solutions to maintain an appropriate incentives framework for the development of therapies for rare diseases
- To provide sounding board for advocacy activities specifically to improve access to orphan medicinal product

Expected duration

- Until end of 2021 (estimated potential date for legislative proposal for revision of OMP regulation)

A PHARMACEUTICAL STRATEGY FOR EUROPE

CNA/CEF Meeting

27 November 2020

Simone Boselli & Matteo Scarabelli

EURORDIS.ORG

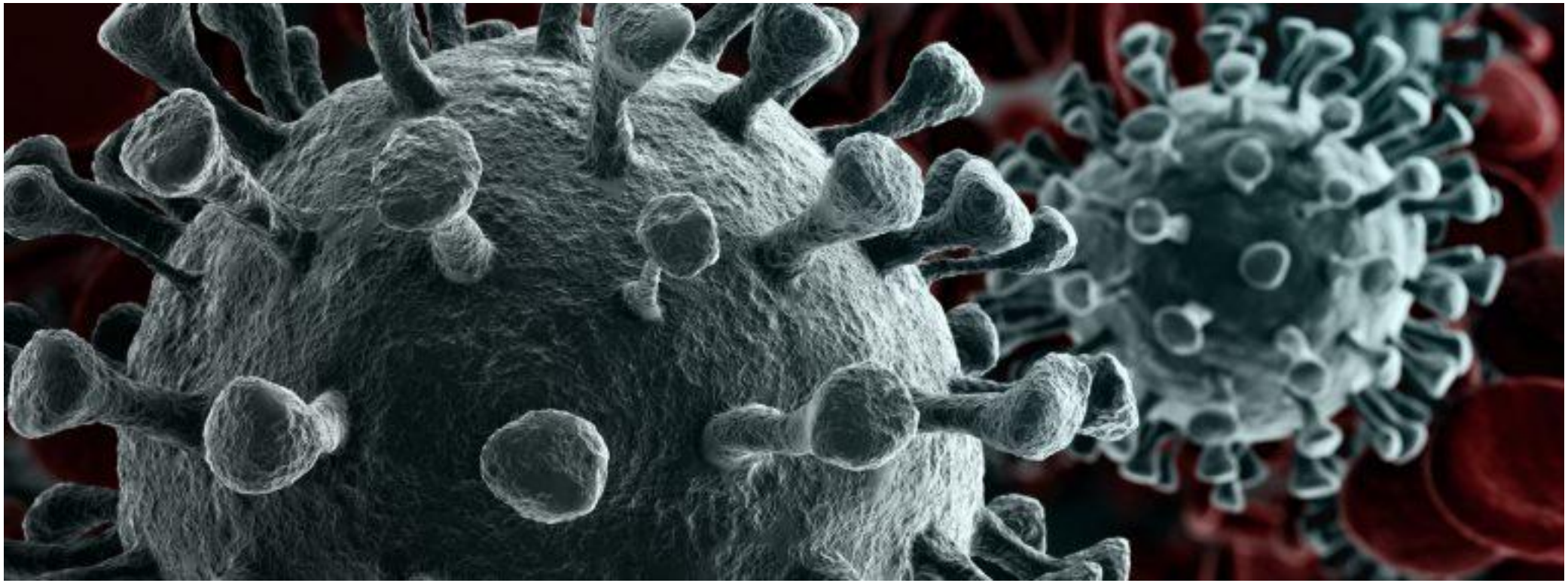
It all began with a letter from Ursula

Stella,

I want you to look at ways to help ***ensure Europe has the supply of affordable medicines to meet its needs****. In doing so, you should* ***support the European pharmaceutical industry*** *to ensure that it remains an innovator and world leader.*

Ursula

Then...

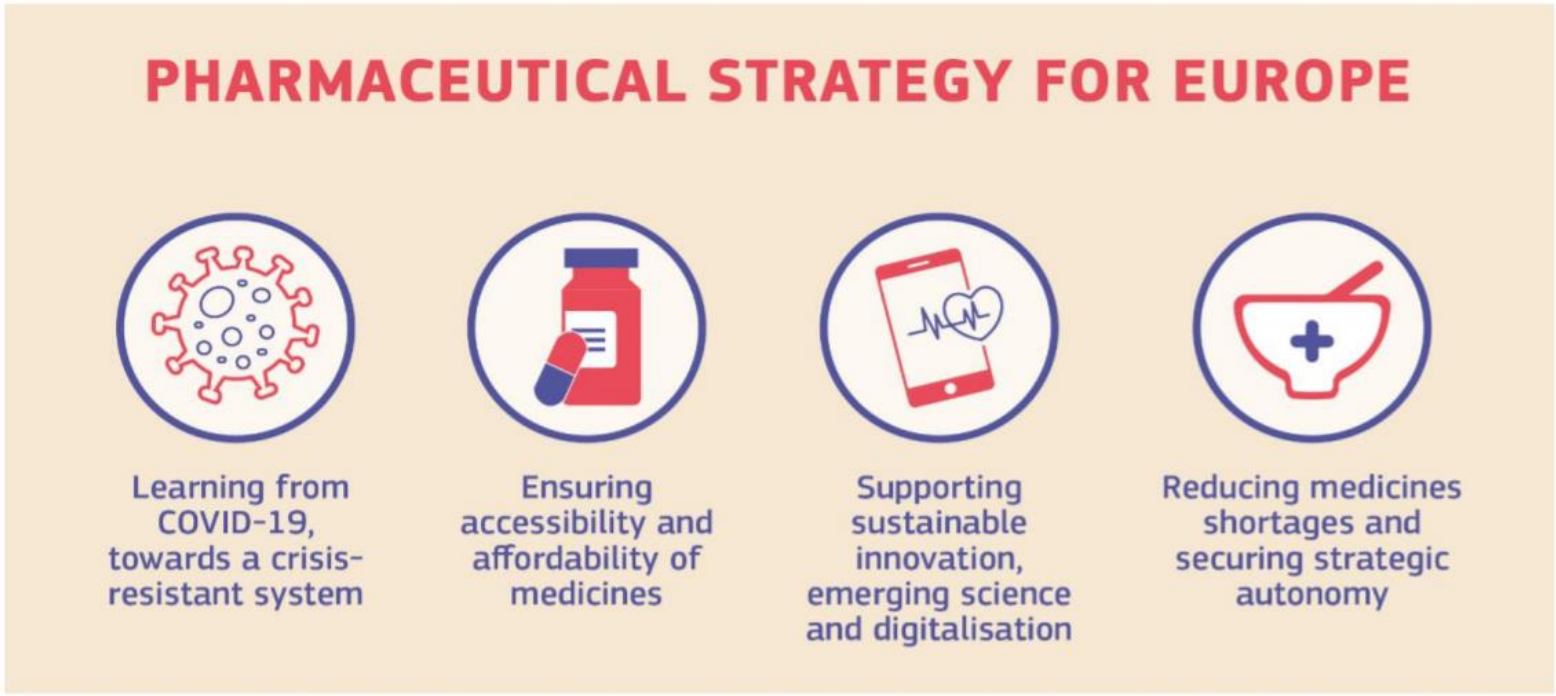


The context (exacerbated by Covid19)

And finally …

"Stella KYRIAKIDES, European Commissioner for Health and Food Safety, […] **we adopted an ambitious pharmaceutical strategy for Europe**. It is an important building block in a genuine European Health Union [...] This strategy addresses both the short term challenges linked to covid-19 and the long term challenges connected with unmet medical needs, Europe's strategic autonomy, and sustainable health systems […] **There are thousands of European Union citizens who are today suffering from rare diseases**. And there are thousands of families who have children with cancer and they do not have access to the medicines that we would want them to have. That would give them a better quality of life and hope. And **to make sure that no matter where you live, no matter what state, what member state you belong to, you have equal access to safe and affordable and innovative medicines**."

What's in the pharmaceutical strategy



What is the European Commission aiming to do?

Unmet needs

- **Research and innovation** for new treatments, vaccines and antibiotics
- Align **clinical trials** to patient and health system needs

Access to affordable medicines

- EU level cooperation on **pricing** and **reimbursement policies**
- More **competition** from generic and biosimilar medicines
- Promotion of **health technology assessment**

Digitalisation and new technologies

- Investment in **research, development and manufacturing** of new medicines
- Enable cutting-edge products, scientific developments and **technological transformation**

Anticipation and response to major health crises and open strategic autonomy

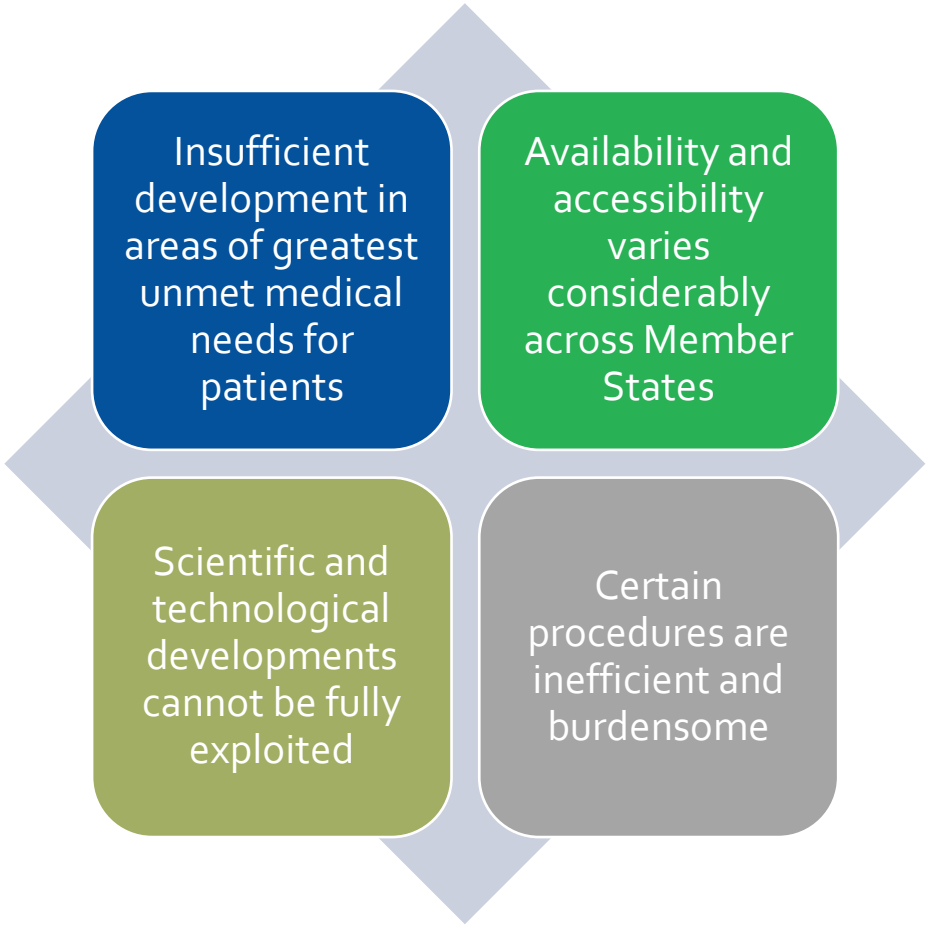
- **Stronger supply chains**, with strategic stockpiling and more production and investment in Europe
- Establishment of a **EU Health Emergency Response Authority (HERA)**
- Reduction of the **environmental impact** of medicines

A long list of actions …

Proposed reform	Timeline
Review the Orphan and Paediatric Regulations to offer more-tailored incentives that take account of intellectual property rights to improve the therapeutic landscape, address unmet needs (e.g., in paediatric cancer) and improve access and affordability	2022
Work with the European Parliament and Council to secure approval for pan-European HTA	2021
Incorporate the EMA's priority medicines scheme (PRIME) in the regulatory framework to accelerate product development and authorisation in areas of unmet needs	2022
Enable parallel scientific advice on clinical study design for medicines by HTA bodies and the EMA	2022
Review the pharmaceutical legislation to address market competition considerations for off-patent drugs and thus improve access to generics and biosimilars, including interchangeability and the "Bolar" exemption	2022
Work with the EMA and member states to understand the root causes of market access delays	2021
Promote innovative procurement approaches for drugs and medical devices in the framework of the Big Buyers initiative (a European Commission platform to facilitate collaboration in strategic public procurement) and the new "innovation partnership" tender procedure for medicines with limited demand.	2021
Revise the pharmaceutical legislation to improve the competitive functioning of the markets and to take account of how market effects impact on affordability	2022
Share knowledge on best practice in pricing, payment and procurement policies to improve the affordability and cost-effectiveness of medicines and health system sustainability (including increased uptake of biosimilars)	2021-2024
Work with member states on non-legislative measures to improve transparency (e.g., guidelines on principles and costing methods for establishing the R&D costs of medicines)	2021-2024
Optimise the supplementary protection certificates system to make it more transparent and efficient	2022
Pass legislation to create a European Health Data Space	2021
Establish an interoperable data access infrastructure for the European Health Data Space to facilitate secure cross-border exchange and analysis of health data	2021-2025
Support innovative clinical trial designs and more patient-oriented drug development by fully implementing the Clinical Trials Regulation	2021
Work with industry and academia to test a framework for repurposing of off-patent medicines and inform possible regulatory action	2021
Revise the pharmaceutical legislation to adapt to cutting-edge products, scientific developments (e.g., genomics or personalised medicine) and technological transformation (e.g., data analytics and digital tools) and provide tailored incentives for innovation	2022

Of interest to therapies for rare diseases

Review of OMPs Regulation: major problems identified



Review of OMPs Regulation: major problems identified

Option 1

- The criterion for granting an orphan designation to a medicine under development will remain the number of people affected (current threshold of 5 in 10 000). **The market exclusivity will remain the main incentive provided (but its duration will be variable). The length would depend on the type of development (innovative products; re-purposed products; second/multiple indications).**

Option 2

- This option builds on Option 1. However, it proposes changes to the current criteria for designation in order to better identify rare diseases. **We will propose changes to the current threshold of total number of cases of a disease at a specific time. In parallel, we will also explore if a different criterion could be used to identify specific rare diseases (e.g. rare cancers) by measuring the number of people that acquired the disease during a specified time-period (incidence).** Different criteria would apply depending on the type of the disease.

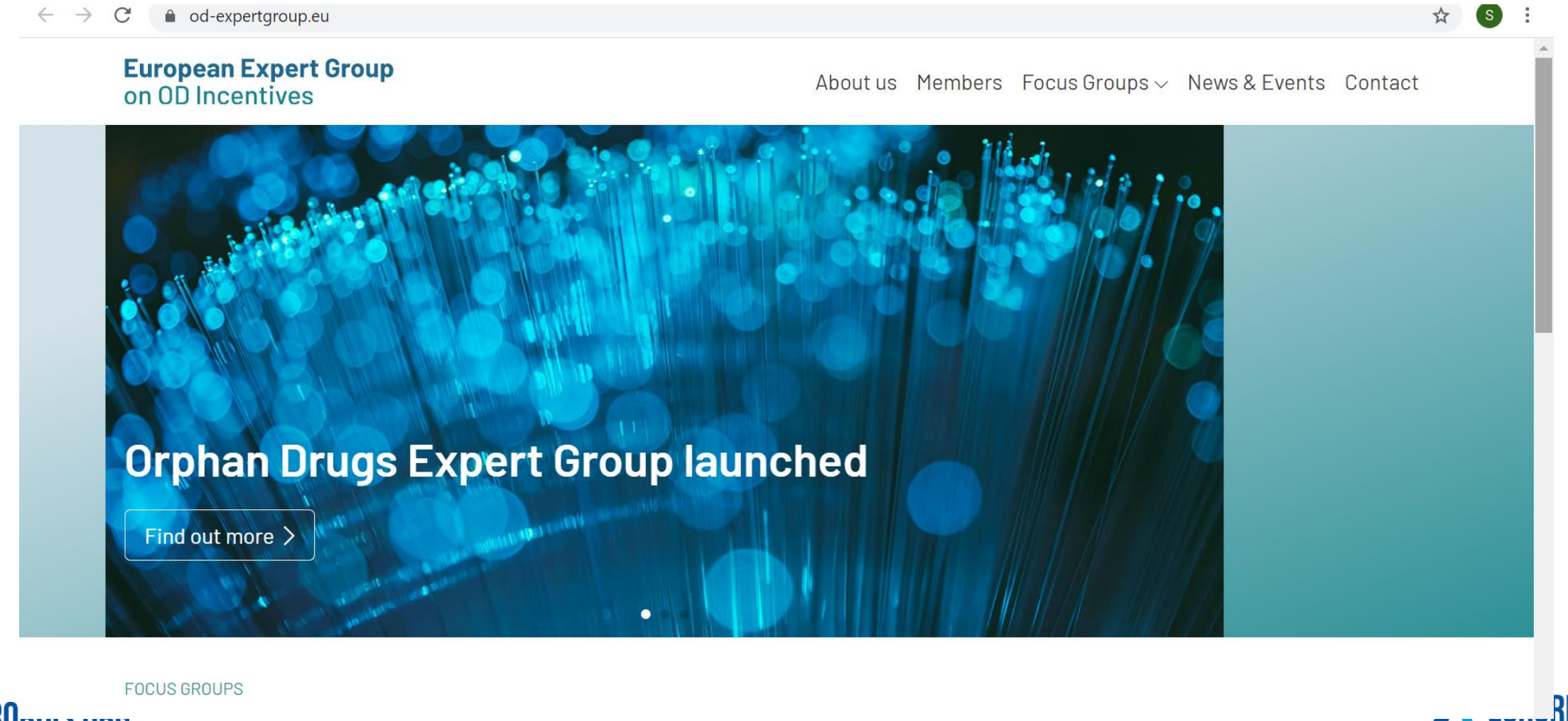
Option 3

- As regards the criteria for designation and incentives, this option builds on Option 2 and will consider an alternative incentive. Market exclusivity as per Option 2 will remain the standard incentive provided to medicines for rare diseases. **For products addressing an unmet need in rare diseases and rare paediatric diseases, we will explore novel incentives that complement or replace the market exclusivity.**

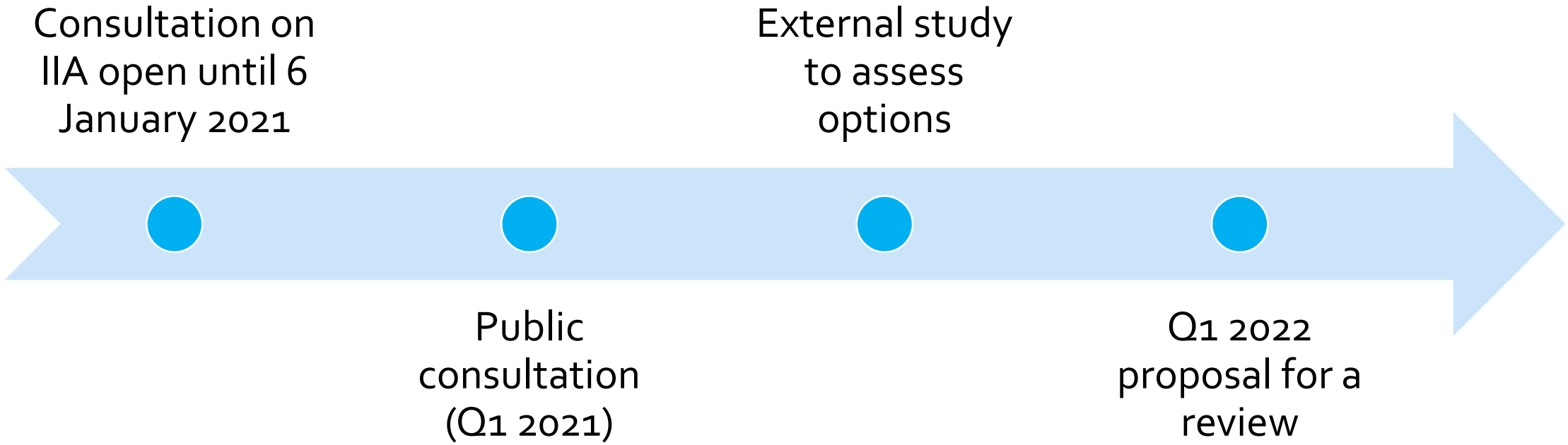
Option 4

- This option builds on Option 3 for criteria for orphan designation incentives. **Market exclusivity will no longer be an incentive provided for all medicines for rare diseases. However, for products addressing an unmet need in rare diseases and rare paediatric diseases, market exclusivity or novel incentives will be explored as main reward.**

European Expert Group on OD Incentives



Next steps


Consultation on IIA open until 6 January 2021
Public consultation (Q1 2021)
External study to assess options
Q1 2022 proposal for a review

Pharma Strategy & HTA Regulation

European HTA as part of the Health Union

27 November 2020

National Alliances | European Federations

Pharmaceutical Strategy Consultation

EUROPEAN COMMISSION Communication

- Key messages
  - Support for an EU-wide HTA
  - More R&D, Regulatory, HTA alignment
  - Regulatory simplification (less fragmentation and duplication)
  - Sharing information and Strategies about P&R

"what are the most effective ways the EU can help improve affordability of medicines for health systems?"
[Multiple Choice 3/5]

TOP RESPONSES

48% Better assessing the value and the effectiveness of a new medicine compared with existing ones

46% Better coordination among EU countries on P&R and Access

40% More transparency on R&D costs

Legislative process' scheme

Legislative Proposal

European Commission

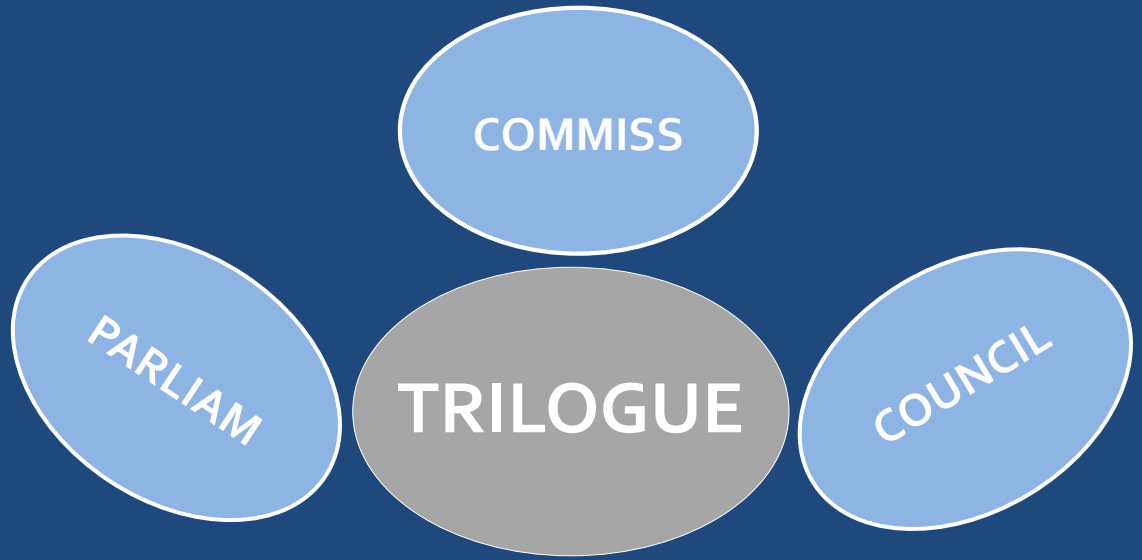
European Parliament

Amended and voted

working on the same original text

European Council

Amended and agreed



European Parliament

VOTE ✓

31/01 2018

European Commission

Proposal

/10 2018

Agreement possible

2018 **2019** **2020** **2021** **? TRILOGUE**







/11 2018

European Council

Talks started

Council of Ministers of Health

Health Attachés

Representatives of National Governments to the EU for health

Compromise Text (GE)

Our expectations… for a valuable agreement

SCOPE

- **Limited (not less than today)**
- - > **scale up**
- **Possibility to extend** to complex/costly/advanced therapies in the Annual Work Plan
- Selected **Medical Devices**

ONE DATA STOP

- No double submission for industry (**only EU submission**)

NO DOUBLE EMA

- **No revaluation** of EMA data

EC's ROLE

- **Oversee** the work and its use
- Ensure **propre engagement**

EU HTA USE

- EU HTA report as **part of the national report**
- No **obligation,** but **convenience**

PUBLIC

- Summary documents for **patients and professionals**

Any progress is welcome…

European Council of Health Ministers

to do more than today, not less

to set a minimal framework:
scientific debates afterwards

to start working together
(sooner the better)

to build a piece of the European
Health Union

When the time comes…

- For those who are interested :
- **Dedicated zoom session** to discuss the details once they will be known

  - *Key aspects to navigate the Law and the System*
    - *Scenarios*
      - *Functioning*
        - *What the future will look like*

Let us know!

THANK YOU

Any questions?

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Two strand of activities currently underway

'EUCOPE Expert Group'

- Started recently (1 September), the scope of this multistakeholder group is to look specifically at the incentives framework
- Supported by EUCOPE and several companies
- Chaired by Maurizio Scarpa and Renate Sommer (former MEP)

'EFPIA group on Access to OMPs'

- Focusing on discussion and proposals to facilitate a process that allows prices to align with value and ability to pay and
- Proposals to ensure equity of access and solidarity across EU MS
- Engaged participation from EFPIA DG to individual companies and national pharmaceutical associations

Focus group

Focus Group 1 PRIORITIZATION & OPTIMIZATION	Focus Group 2 REGULATORY PATHWAYS	Focus Group 3 DEVELOPMENT INCENTIVES
Alastair Kent (Independent)	**Dr Alexander Natz** (EUCOPE) Partly substituted by **Vittoria Carraro**	**Prof Dr Annemieke Aartsma-Rus** (Leiden University Medical Center)
Dr Daria Julkowska (EJP on Rare Diseases)	**Dr Jean-Michel Heard** (MetabERN)	**Prof Dr Marc Dooms** (University Hospitals Leuven)
Dr Denis Horgan (EAPM)	**Martine Zimmermann** (Alexion)	**Prof Dr Maurizio Scarpa** (MetabERN)
Emmanuel Chantelot (BMS)	**Patrick Deboyser** (Collegio di Parma)	**Prof Dr Michael Schlander** (University of Heidelberg)
Dr Erik Tambuyzer (BioPontis)	**Simon Bennett** (Biogen)	**Dr Michela Gabaldo** (Fondazione Telethon)
Dr Lucia Monaco (IRDiRC)	**Simone Boselli** (EURORDIS)	**Peter Bogaert** (Covington)
Dr Renate Sommer (Former ENVI MEP)	**Prof Dr Maurizio Scarpa** (MetabERN)	**Vittoria Carraro** (EUCOPE)
Thomas Bols (PTC)	**Prof Dr Michael Schlander** (University of Heidelberg)	**Andrea Bonetti** (Chiesi)
Toon Digneffe (Takeda)		**Dr. Alexandra Tolia** (FundPlus)
Simone Boselli (EURORDIS)		**Maria Cavaller** (EURORDIS)
Linda Abdelall (EUCOPE), substituted by Vittoria Carraro		

Key high level highlights

1 Need to improve R&D ecosystem

Increase the amount of development-ready research through a better R&D ecosystem that also ensures take-up of research and further development through industry:

- Coordinated funding effort through a **PPP EU-funding instrument**/ alliance for rare disease
- EU level **collaborative data base** on (development-ready research) to improve knowledge/ data-sharing and collaboration on basic research and diagnosis + financial incentive for data sharing through funding conditionality
- **De-risking of basic research** to ensure company take-up through guidance for clinical preparedness + financial incentives through funding conditionality

- Money must come from somewhere
- Will policy makers be inclined to increase money available when uncertainty about benefits to society at large and incentives are already deemed to generous
- Does the group have to give something in return?

2 Need to improve & modulate incentives

Concrete ideas for improving incentives, but less concrete ideas and less agreement on whether modulation should be done and how it should look.

- Add targeted **financial incentives for the clinical phase**: fiscal incentives, priority voucher system, grant system for clinical trial funding
- Current level of incentives should be broadly kept, and **modularity added on top for priority areas**, including (i) more funding, (ii) special regulatory pathways, (iii) modulated incentives (e.g. exclusivity period).
- Some sense that **priority areas** need to be identified (within 95%) by disease area/ prevalence: ultra-rare, genetic diseases, pediatric have been mentioned but not agreed upon

- No clear agreement on the ambition of *this* initiative: addressing underserved areas or also improving treatments in crowded areas
- Potential opposition: ethical issues, categorization-fights, who takes the decision?

Key high level highlights (2)

3 Need to increase flexibility, predictability and speed of regulatory pathways

- Increase **collaboration and flexibility** of collaboration with EMA working towards a common goal: (i) single point of contact, (ii) iterative advice frameworks, (iii) strengthening role of the COMP
- Increasing **predictability and legal certainty of incentives in OMP regulation** through (i) clearer definition of significant benefit (indirect comparison standards), (ii) prevent undermining ME through off-label use/ pharmacy compounding
- Better use of RWE to **strengthen evidence-base** at all stages (pre & post approval): common infrastructure/ multi-stakeholder partnership for early coordination on RWE generation/ patient registries (e.g. around ERN)

- Some proposals might be seen as too protective of industry

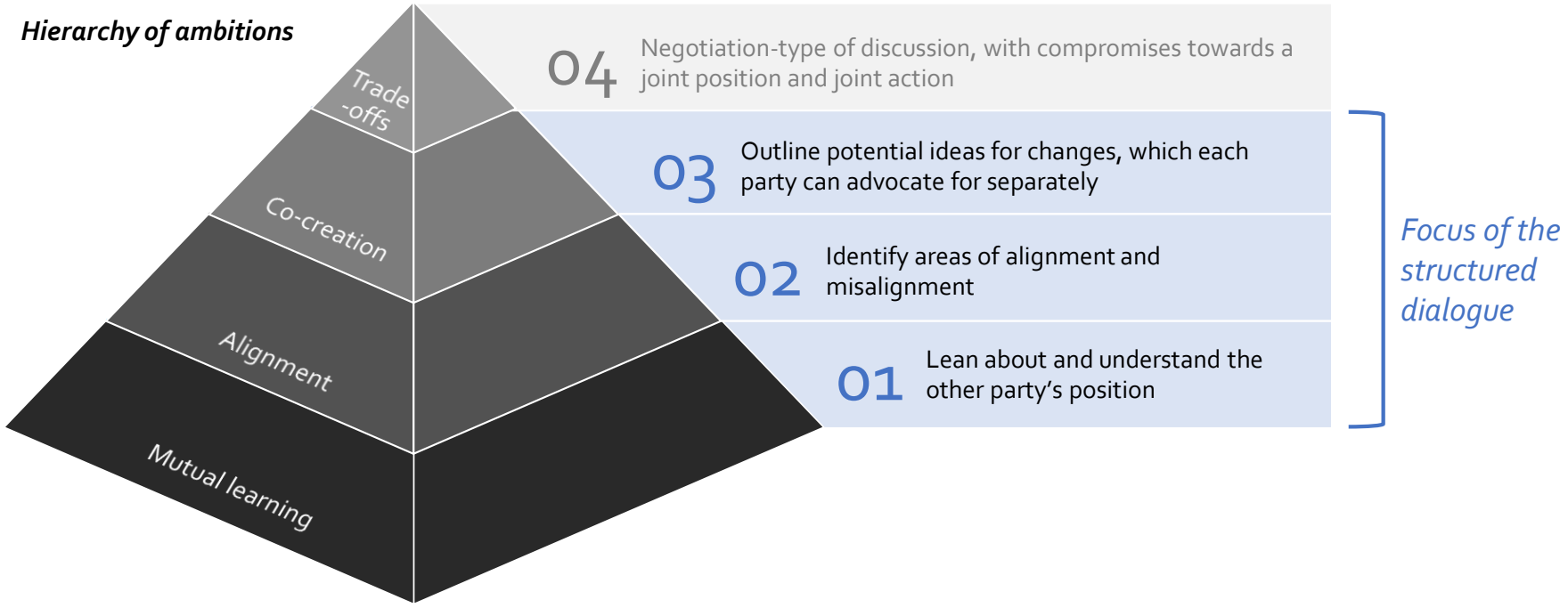
4 Better alignment between supply and demand side incentives

- Common **EU HTA/value assessment** aligned with previous stages of the regulatory pathway (e.g. for recognition of efficacy or significant benefit)
- Establish **early dialogues** between stakeholders (pharma, EMA, HTA, payers)
- Create **risk and value sharing** (best-) practices recognized at European level and equally applied across member states: Outcome-based, innovative pricing models
- Full **harmonization of regulatory pathway** and product quality requirements across member states
- **EU/US/global harmonization** on ultra-rare for trial design & product quality

- Many of these ideas are moonshots – useful to identify first steps in the right direction

Structured dialogue with EFPIA

- **The intention of the dialogue is to identify alignment and opportunities to improve access for rare disease patients across Europe**
- **The dialogue will not result in joint positioning**



Topic of the first dialogue

	Scope of the first dialogue	Key questions
Topic 1: **Managing evidential uncertainty: use of real-world evidence and adaptive processes**	**Areas of focus:** • Use of real-world evidence to inform payer decision-making • Adoption of iterative value assessment processes to manage uncertainty **Topics excluded** • Adaptive frameworks for regulatory assessment (e.g. conditional approval) • Link between iterative value assessment and pricing	**How can RWE collection be optimised at EU-level?** • *Should RWE be collected at EU-level? Through which mechanism(s)?* • *How should RWE generation be funded? Would the creation of a 'EU Fund for Evidence Generation' stand to improve patient access in rare diseases?* • *Should experiments (e.g. TRUST4RD, RWE4DECISIONS) be scaled up? How so?* • *Of the key drivers / barriers to RWE, which can be leveraged / addressed by EURORDIS and/or EFPIA?* **How can adaptive processes be used to leverage RWE?** • *Is it desirable and feasible to strive for an iterative HTA process?* • *Should adaptive processes occur at country or EU – level? (to be discussed along with topic 2)* • *Of the key drivers / barriers to adaptive processes, which can be leveraged / addressed by EURORIDS and/or EFPIA?*

Topic of the first dialogue (2)

	Scope of the first dialogue	Key questions
<u>Topic 2</u>: **Enhancing multi-country or EU collaboration on value determination**	**Areas of focus:** • Multi-stakeholder dialogues for early value determination, e.g. MoCA • Multi-country or pan-European processes for value assessment / HTA **Topics excluded** • Regulatory processes • Joint pricing negotiations • Joint procurement	**Overarching question: If a mandatory, EU-wide HTA is not to happen, what would an alternative be?** **1. Could a non-binding, EU-wide initiative (e.g. MoCA) be an effective alternative to the EU regulation on HTA?** • *Has MoCA contributed to improving access? How?* • *What can be learnt from experience with MoCA?* • *What are limitations to MoCA's impact to access?* • *How can MoCA's impact on access be increased?* • *Should MoCA be scaled up? How?* • *Can voluntary collaborations lead to large impacts on patient access in rare diseases?* **2. Is there an opportunity for smaller groups of countries to engage in formal joint HTA?** • *To date, have small-scale joint HTA initiatives contributed to improving access? How?* • *Of the key drivers / barriers to multi-country collaborations, which can be leveraged / addressed by EURORIDS and/or EFPIA?*

Note that the discussion will be limited to collaboration for clinical benefit assessment.
The link between joint HTA and joint negotiations or joint procurement will not be explored

Proposal for expert group on pharmaceutical strategy

Rationale

In the context of the Pharmaceutical Strategy, the recent evaluation of the OMP & Paediatric Medicines regulation, and the persistent challenges in access, we believe we need to have in place an extended team supporting EURORDIS staff in analysing the situation, proposing new solutions, and fostering dialogue

Mandate

- To support EURORDIS Staff in analysis of OMPs evaluations & definition of potential solutions to maintain an appropriate incentives framework for the development of therapies for rare diseases
- To provide sounding board for advocacy activities specifically to improve access to orphan medicinal product

Expected duration

- Until end of 2021 (estimated potential date for legislative proposal for revision of OMP regulation)