EUCOPE
European Confederation of Pharmaceutical Entrepreneurs AISBL

OMP Working Group Meeting

4 April 2023

Competition Law Compliance Policy

EUCOPE brings together representatives innovative companies to discuss common issues, challenges and trends affecting the pharmaceutical industry. This activity can be perfectly legitimate. However, certain competition law risks may arise in relation to EUCOPE's activities.

EUCOPE's European Union ("EU") compliance policy ("Policy") explains these competition law risks and aims to ensure compliance by all members and EUCOPE staff with the rules applicable in the EU. EUCOPE itself and its members are subject to these rules when engaging in any EUCOPE related activities. Any anticompetitive behavior adopted by a member can result in serious financial, criminal and/or disciplinary penalties, as well as other harm (e.g., reputational harm) for EUCOPE, represented companies and for meeting participants personally.

Competition Law Compliance Policy

There are certain matters which **should not** be discussed with competitors before, during or after any such meetings. These include:

- Territorial restrictions, allocation of customers, restrictions on types of services, or any other kind of market division;
- Prices, price changes, conditions of sale (including payment terms and guarantees), price differentials, discounts;
- Current market conditions and issues, including industry pricing policies or patterns, price levels; capacity (including planned or anticipated changes regarding those matters), except where limited to the discussion of historical or public information;

[cont'd]

Competition Law Compliance Policy

- Individual costs, cost accounting formulas, methods of calculating costs;
- Individual company figures on market shares, sources of supply, capacity;
- Information as to future plans of individual companies concerning technology, capacity, marketing or sales; and
- Matters relating to individual suppliers or customers.

Attention: these rules equally apply to informal discussions before, after, or during each meeting. If any sensitive information is discussed or disseminated, insist that the discussion be terminated immediately and make sure that your objection is recorded in the minutes. If necessary, leave the meeting and immediately inform EUCOPE's General Counsel.

Agenda

I. Welcome & introduction

II. OMP Regulation revision

- Latest intel on OMP proposal
- EUCOPE priorities in the OMP context
- Reflections from the companies

Agenda (cont)

III. Insights on the Spanish Presidency– Juan Rivera, Senior Managing Director, FTI Spain

IV. Moonshot update

V. England Rare Disease Action Plan – Ed McIntosh, Evoke Incisive Health

VI. Rare Disease Partnership update – Daria Julkowska, European Joint Programme on Rare Diseases

VII. AOB

Next meetings

- 27 April: HERA Steering Group Meeting
- 03 May: Pharmaceutical Strategy Task Force Meeting
- 09 May: Q2 Regulatory Working Group Meeting
- 25 May: P&R/Market Access Working Group Meeting
- 05 July: Cell & Gene Therapy Working Group Meeting

I. Welcome & introduction

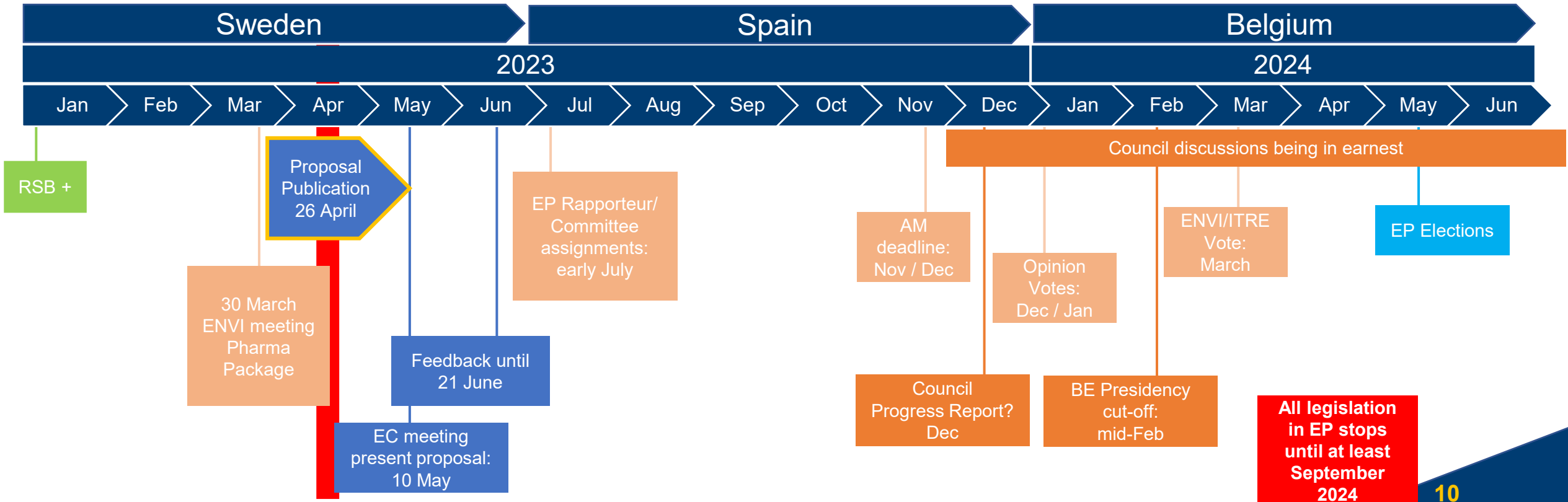
II. OMP Regulation revision

Legislative Timeline

Minimum 8/9 months required for Parliament procedure

Key questions:

- Political hurdles (different stances between and within political Groups)
- Inter-committee alignment (file allocation and positioning)



NB: These timings are indicative and rough estimates, not official – it assumes a 'fast' process

Proposal delay

- A high-level political decision at 11th hour
  - Commissioner had green-lit proposal
  - Many Cabinets were unaware of the delay initially
- Significant political pressure for the Commission to publish and they are maintaining April commitments
- Many MEPs expressed frustration with delay
- Criticism on the industry is growing significantly
- Commissioner Kyriakides promised that the proposal should be published on 26 April

Parlement européen

Ms Ursula von der Leyen
President of the European Commission
Rue de la Loi 200
1049 Bruxelles

Brussels, March 23,2023

Madam President,

On March 22, 2023, we learned with consternation the third postponement of the publication of the revision of pharmaceutical legislation.

We all know that this legislative package was ready to be published.
We all refrained in the past weeks to comment on the leaked version of the proposals.

Patiently, we were waiting for the publication of the package on March 29th and its presentation by Commissioner Kyriakides on March 30th in the Environment, Public Health and Food Safety Committee. Waiting in vain, unfortunately.

After postponing the revision of the REACH regulation and the Food Information to Consumers' legislation, the Commission now seems to be once more giving in to industrial pressure.
The report of this crucial legislation, without any justification and any alternative date of publication, is unacceptable.

This legislation is key to ensure the fair and equitable access of medicines to all patients, to all Europeans. It is time to listen to them and give them answers. They deserve to be heard as much as the pharmaceutical industry is.

Today, we are urging you to publish this important legislative package no later than April 2023 to allow the legislative process to start.

Counting on your responsiveness and your determination to act, please accept, Madam President, the expression of our sincere greetings.

Véronique Trillet-Lenoir
Députée européenne Renew Europe

MEP Overview

- Interest from both ENVI and ITRE
  - Expect a rapporteur for each file (could be the same MEP)
    - S&D expressed significant interest in Directive
  - IMCO as a possible third committee
  - ITRE expressed interest and competence in the **incentive framework** and **research policy** (including pediatric policy)

- SANT committee to focus on hearings until proposal is published – still won't have any legislative power

ENVI


MEP Kympouropoulos EPP, EL


MEP Wölken S&D, DE


MEP Trillet-Lenoir RE, FR


MEP Weiss EPP, DK


MEP Cerdas S&D, PT


MEP Ries RE, BE

ITRE


MEP Busoi EPP, RO


MEP Toia S&D, IT


Dominique RIQUET RE, FR


MEP González Casares (S&D, ES)


MEP Solís Pérez (Renew, ES)

Proposal Overview & EUCOPE Priorities

High Unmet Medical Need

Article 65

Commission proposal

Article 65

Products addressing a high unmet medical need

1. A product shall be considered to address a high unmet medical need if at least one of its indications diagnoses, prevents or treats an orphan condition for which:

(a) no satisfactory diagnosis, prevention or treatment method exists or

(b) a satisfactory diagnosis, prevention or treatment method exists and it has been demonstrated by the applicant that such a product will bring exceptional therapeutic advancement.

2. In paragraph 1 both (a) and (b) the product shall provide for a meaningful reduction in disease morbidity or mortality for the relevant part of the population.

3. A product for which an application has been submitted in accordance with Article 22 [revised Directive 2001/83/EC] shall not be considered to be a product addressing a high unmet medical need.

4. The Agency shall draw up scientific guidelines for the application of this Article after consulting the European Commission and the parties involved in the cooperation mechanism defined in Articles 130(zb) and 155.

EUCOPE comments: The definition is narrow yet vague, including concepts such as ‘exceptional therapeutic advancement’ and an unqualified ‘meaningful’ requirement.

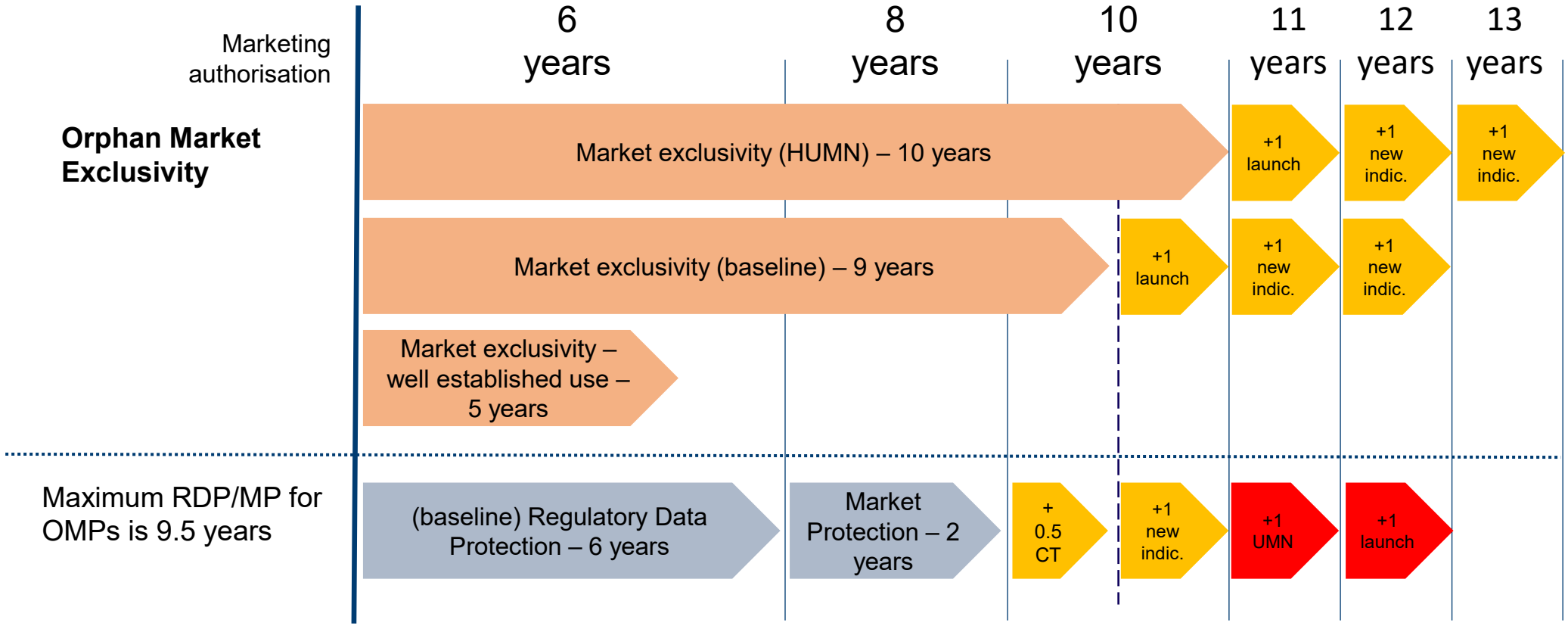
Beyond the OME implications, HUMN introduces a ‘stamp’ that will have major P&R implications

EUCOPE proposed approach:

a) Introduce an alternative modulation approach not based on HUMN

b) Ensure a wider definition established with input from a broader range of stakeholders

Commission's Orphan Modulation Framework



Orphans that receive an extended OME for launching in all Member States and expanding into new indications, do not receive the RDP extension for UMN or launching

Significant Benefit definition

Article 2.7

Commission proposal

(7) 'significant benefit' means a clinically relevant advantage or a major contribution to patient care of an orphan medicinal product **if such an advantage or contribution benefits a substantial part of the target population.**

EUCOPE comments: The assessment is already undertaken by the EMA – but being included in the legal text would remove flexibility from the system.

Significant benefit is the 'gateway' to OMP incentives, therefore raising the barrier to entry is a concern.

EUCOPE proposed approach:

Ensure a sufficiently broad understanding of Significant Benefit in the legislation.

GOMA – Global Orphan Marketing Authorisation

Article 67.6

Commission proposal

6. **Where a marketing authorisation holder holds more than one orphan marketing authorisations for the same active substance, those authorisations shall not benefit from separate market exclusivity periods. The duration of the market exclusivity shall start from the date when the first orphan marketing authorisation was granted in the Union.**

EUCOPE comments: Poses a major risk for smaller developers exploring the use of a compound for different indications and contradicts the Commission's aim of addressing areas of (H)UMN.

The intension is to avoid 'evergreening' and can be seen as an alternative to introducing incidence.

The notion of +1 year RDP did not work for repurposing, thus the same mistake shouldn't be made in the orphan context.

EUCOPE proposed approach:

Extend the number of possible extensions and the duration of each exclusivity period.

Launch Conditionality

Article 67.3

Commission proposal

3. The periods of market exclusivity referred to in paragraph 2 (a) and (c) shall be prolonged by an additional year, when the orphan marketing authorisation holder can demonstrate that the conditions referred to in Article 70(2) (a) and Article 71(1) [of revised Directive 2001/83/EC] are fulfilled, *mutatis mutandis*, for the purpose of market exclusivity.

The procedures set out in Articles 71(2) to (5) [of revised Directive 2001/83/EC] and in Article 72 [of revised Directive 2001/83/EC] shall also apply to the prolongation of market exclusivity.

5. The orphan medicinal products which benefit from the prolongation of market exclusivity referred to in paragraphs 3 and 4 shall not benefit from the additional period of data protection referred to in Article 70(2)(a) or Article 70 (2) (b) [of revised Directive 2001/83/EC].

Directive article 71

2. To receive the prolongation of data protection period, the marketing authorisation holder shall apply for a variation of the relevant marketing authorisation between 34 and 36 months, or for SMEs or not-for-profit entities between 46 and 48 months, after the date of the initial marketing authorisation.

The application for a variation shall contain documentation from the Member States in which the marketing authorisation is valid to:

(a) confirm that the conditions of the first paragraph have been satisfied in their territory; or

(b) waive those conditions in their territory for the purpose of this prolongation.

EUCOPE comments: The proposed system does not address the fundamental challenges underlying access and it's potential impact is uncertain.

What constitutes launching is also unclear from the existing text.

EUCOPE proposed approach:

Continue to advocate for alternative approaches to improve access to OMPs, such as cross-border frameworks.

EUCOPE will call for the period of time to launch to be extended, while ensuring the additional exclusivity remains at +1 year

Other changes of concern

- Lack of clarity around the **transition periods**, e.g. how will the legislation be grandfathered in or impact existing therapies;

- What is the impact of **losing Orphan Designation** at point of marketing authorization;

- **OD eligibility** should consider “all existing methods should be taken into account that at least partly overlap with the medicinal product referred to in the first sentence of this paragraph”

EUCOPE Proposal Feedback

21 June *tentative* deadline

EUCOPE will share **Feedback** with Members for their input:

- 10 May – EUCOPE shares first draft with full membership
- 22 May – Deadline member comments on feedback
- 8 June – EUCOPE circulates updated feedback for final comments or ‘red flags’
- 14 June – deadline members final comments
- 20 June – EUCOPE publishes feedback

EUCOPE’s external engagement will continue to focus on delivering our pre-legislative messages in the coming months

Engagement timeline and strategic milestones

EUCOPE
European Confederation of Pharmaceutical Entrepreneurs AISBL

Pre-legislative phase

Swedish Presidency Starts 1 Jan 2023

COM proposal for OMP review to be published (April)

Legislative phase

Spanish Presidency Starts, 1 July

2024

1st engagement window

2nd engagement window

3rd engagement window
Adapt positioning to proposal

Jan-Feb | March-April | May-June | July-Aug | Sept-Oct | Nov-Dec

OMP amendments

Proposal feedback

HUMN criteria

UMN & Launch messaging

Joint Procurement position Paper

Gastein

RDD messaging

podcast

podcast

Economic counter-points

Assistant breakfast

podcast

podcast

1:1 MEP & Attaché engagement

European Expert Group on OD Incentives

Final report

Leveraging engagement, contacts and briefing sessions of

European Expert Group on OD Incentives

European Alliance for Transformative Therapies

Public engagement opportunities/ milestones

RAREDISEASEDAY.ORG

DIA EUROPE 2023 ADVANCING HEALTH PRIORITIES 22-24 March 2023 | Basel

NORDIC RARE DISEASE SUMMIT

EUROPEAN HEALTH FORUM GASTEIN

RareConversations

WORLD OrphanDrug Congress Europe

EUROPEAN HEALTH SUMMIT

Engagement | Evidence | Comms

III. Insights on the Spanish Presidency– Juan Rivera, Senior Managing Director, FTI Spain

FTI Consulting for EUCOPE

Insights on the Spanish Presidency of the Council of the European Union

Health Policy

FTI CONSULTING

04.04.2023

Spanish context & general overview of the ES Presidency of the EU

Strategic autonomy will be the main overarching theme

- Priorities are still being defined – last chances to include issues in the agenda.

Energy

Health

Food Security

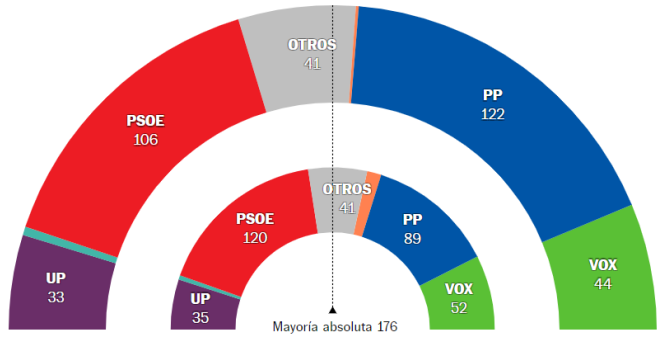
Digital Technologies

Strategic importance of the ES Presidency

- Is the first presidency of the "trio" formed by Spain, Belgium and Hungary.
- It's the last presidency before May 2024 EU elections, meaning that key files should ideally be finalised during the Spanish Presidency.
  - 22 informal meetings in Spain, an informal European Council Meeting in Granada, and around 120 legislative files under processing.

Impact of Spanish elections

- Local and regional elections in May 2023. National elections will take place in December 2023.



Healthcare and life sciences during the ES Presidency

Files under processing

- **European Health Data Space (EHDS)**
- Regulation on standards of quality and safety for substances of human origin intended for human application (SoHO)
- Regulation on fees and charges payable to the European Medicines Agency
- **Pharma package** (expected 26 April, and to include a review of the orphan medicinal products regulation)

(Non-legislative)

- **Europe's Ceating Cancer Plan implementation**
- Healthier together – EU non-communicable diseases (NCD) initiative
- HealthLifestyle4All initiative
- EU action plan on rare diseases
- **Promotion of an international agreement on pandemics management** through the WHO.

Political priorities

One Health	**Rare diseases**	**HIV & Stigma**
Addiction	**Antibiotic resistance**	**Cancer**
Childhood obesity	**Mental health**	**Pandemics management**

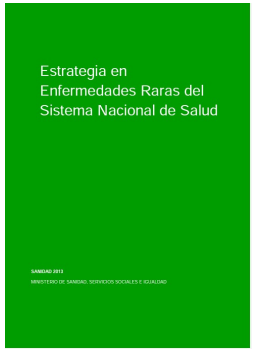
Calendar

Rare Diseases

"Rare diseases will be an important part of the Spanish Presidency of the Council of the EU in order to reach the European Action Plan that we crave for"

- 3 million people suffer rare diseases in Spain (30 million in Europe)
- 47% are dissatisfied with the medical assistance.
- Public coverage of drugs is scarce (21%) and 51% of families have access difficulties.
- Costs amount to an average of 20% of annual income.

Policy framework



National Rare Diseases Strategy (2002), updated in 2009 and 2014, and evaluated annually by the Ministry of Health, Autonomous Communities and Health experts in the Health Interterritorial Council (CISNS). Actions on the following fields:

- Information about rare diseases.
- Prevention and early diagnosis.
- Primary attention.
- Therapies, orphan medicines, and advanced sanitary products.
- Research.
- Education.

EU

1999
Approval of Orphan Medicines Regulation

2008
Europlan: European project for rare diseases National Plans Development

2017
Creation of the European Reference Network by the European Commission

2023
Pharma package – Review of the Orphan Medicines Regulation .

What actions has the Spanish Government taken so far?

Ministry created the **Rare diseases registry**, and advances on the National **Health Data Space** and EHDS at EU level.	Boost diagnosis capabilities through **genetic diagnosis** (5P & GenES Plans – 100M) and 4 **new newborn screening** programs.	**Support innovative therapies**, flexibilizing testing in diseases without authorized treatments.	**Attract industrial investments** through a **1.500B PERTE Vanguard Health** funded with NextGenEU Funds.	20M allocated to **research rare diseases** in 2023 Spanish Budget, through the Carlos III Institute.

Stakeholders: movers & shakers

José Manuel Miñones Conde

Health Minister

- PSOE
- Close ally of PM Pedro Sánchez
- Doctor of Pharmacy
- Previously Government Delegate in Galicia
- Took over the office from Carolina Darias

Inmaculada Navarro Pérez

Health Counsellor – Permanent Representation of Spain in the EU

- Civil Servant
- Took over the position at the beginning of 2023
- Held various positions at the Spanish Ministry of Health
- Has a pharmacy degree and professional experience in the pharmaceutical industry

Dolors Montserrat

MEP (EPP)

- Chairs the Committee on Petitions
- Member of the Committee on the Environment, Public Health and Food Safety
- Member of the Subcommittee on Public Health
- Considered as a possible candidate of the PP in the race for the Barcelona mayor's office, however, in the end the party opted for another candidate

Nicolás González Casares

MEP (S&D)

- Member of the Subcommittee on Public Health
- Supporting the transfer of certain health competences to the European level

Susana Solís Pérez

MEP (Renew)

- Member of the Subcommittee on Public Health
- Rather unlikely that her term will be renewed, given the recent developments in the Spanish political landscape concerning her party

José Manuel Miñones Conde

Health Minister

Party
Socialist Party

Previous Position
Government delegate in Galicia

Education
Doctorate in Pharmacy from the University of Santiago de Compostela

On Monday, 27 March, prime minister Pedro Sánchez announced that the new health minister would be José Miñones Conde. The former government delegate in Galicia took over the position from Carolina Darias, who resigned to focus on her campaign to become mayor of Las Palmas de Gran Canaria. Same as his predecessor, Miñones is a member of the Socialist Party and widely considered a close ally of prime minister Pedro Sánchez.

José Manuel Miñones Conde started his political career rather late, in 2005, in a neighbourhood association. Four years later he became Councillor for Education, Administrative Reform, New Technologies and Health of the municipal government of Ames, a small town of 30.000 inhabitants in Galicia. After spending some years in the opposition, he became mayor of said municipality in 2015. In March 2015, Miñones was named Government Delegate in Galicia by prime minister Sánchez – the position he held until he officially took over the position of health minister of Spain on Tuesday, 28 March 2023. He has a doctorate in Pharmacy from the University of Santiago de Compostela and pursued a scientific career before he initiated his path in politics.

Experts with Impact™

Lunch break
Until 13:15

IV. Moonshot update

Context and update

Multi-stakeholder initiative launched in 2022

Initiative that **brings together the existing knowledge, expertise and experience** of stakeholders in the rare disease community with a **focus on translational research to boost PPP in the 'white spots'** and the development of novel therapies for rare diseases.

- **ENHANCE the translational research ecosystem** to fill the research pipelines with new areas of investigation and novel therapies,
- **OPTIMISE clinical trials and regulatory pathways** for very small patient populations to de-risk and optimise development,
- **DEVELOP infrastructure** to accelerate the journey to diagnosis and treatment.

Following a February workshop – the initiative is refining its work programme along the three priorities

V. England Rare Disease Action Plan – Ed McIntosh, Evoke Incisive Health

UK Rare Disease Action Plan

The UK rare disease landscape has shifted in recent years

2019

Department of Health & Social Care

abpi

The 2019 Voluntary Scheme for Branded Medicines Pricing and Access - Chapters and Glossary

NHS Genomic Medicine Service

2020

NSC UK National Screening Committee

Generation genome and the opportunities for screening programmes

2021

NICE National Institute for Health and Care Excellence

NHS commercial framework for new medicines

NHS Genomic Medicine Service Alliances to help embed genomics into patient care pathways

2022

NICE National Institute for Health and Care Excellence NHS

Classification: Official

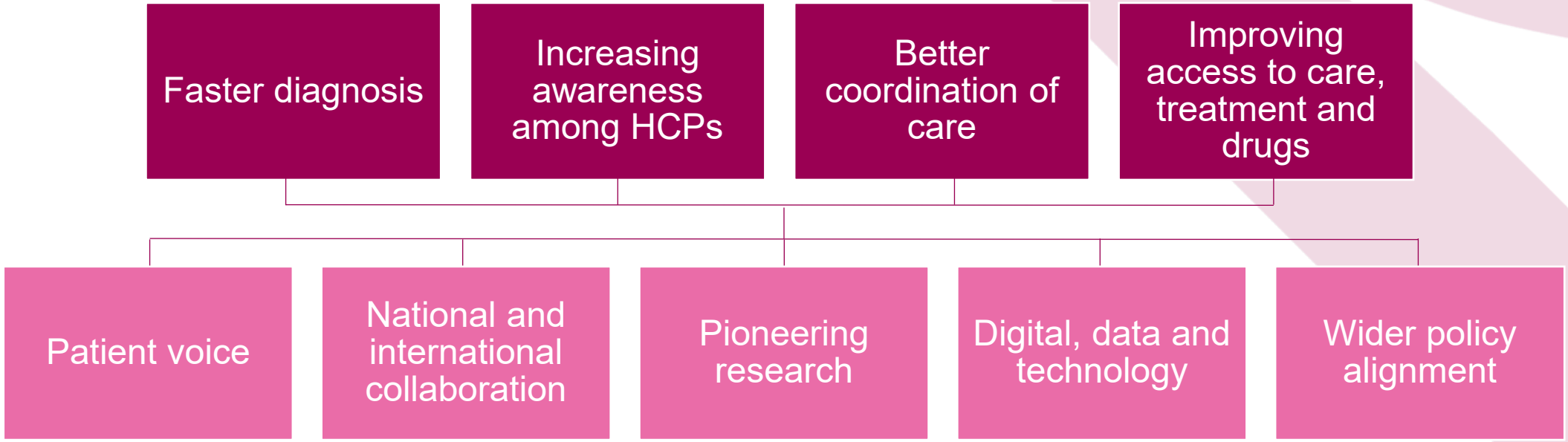
Publication approval reference: PAR1686

The Innovative Medicines Fund Principles

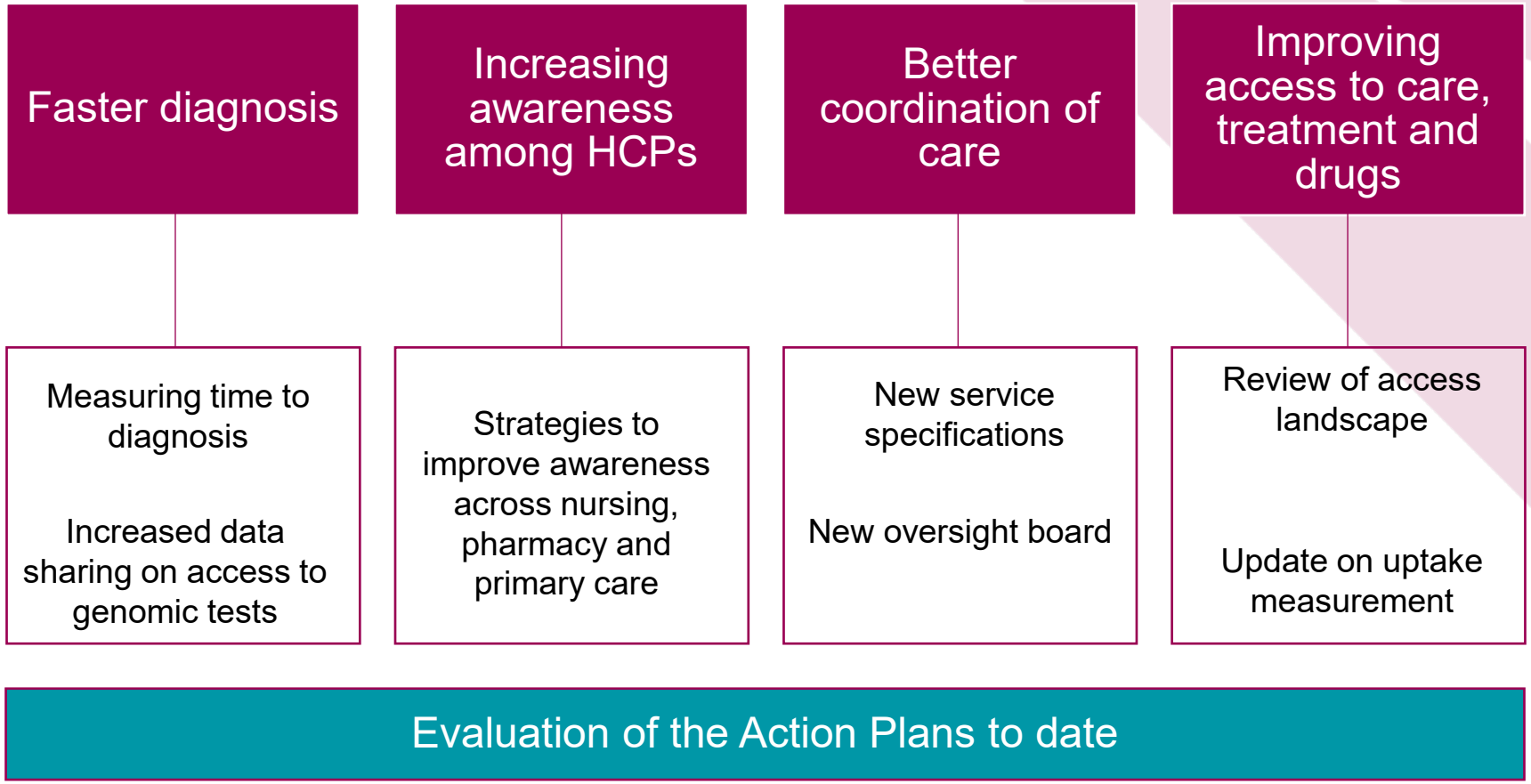
Accelerating genomic medicine in the NHS

A strategy for embedding genomics in the NHS over the next 5 years.

The UK Rare Disease Framework sets out high-level ambitions for further progress



The 2023 Action Plan for England provides an insight into challenges and opportunities for the rare disease community



There is further change on the horizon, creating opportunities to deliver on the Framework's priorities

Regulation	Diagnosis	Access
• Clinical trials legislation • Mutual recognition arrangements • New fast-track licensing process	• NHS Newborn Blood Spot Screening Programme/genomic test directory expansion • WGS screening evaluation	• Updated Commercial Framework • Modular updates to NICE processes • NHS England Life Sciences review

Any questions?

VI. Rare Disease Partnership update – Daria Julkowska, European Joint Programme on Rare Diseases

VII. AOB