NAVIGATING THE COMPLEXITIES OF THE PHARMACEUTICAL LEGISLATION

PID FORUM

An IPOPI Event

Hosted by MEPs Billy Kelleher (Renew),
Cyrus Engerer (S&D) & Tomislav Sokol (EPP)

IPOPI
INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

ipopi.org

EVENT PROGRAMME

14:30 *Welcome address*

14:45 *Setting the scene*

14:55 *What the pharmaceutical legislation brings to patients*

15:05 *Panel: The pharmaceutical legislation's impact on rare disease and PID communities*

15:35 *Open floor discussion*

15:45 *Call to Action: Ensuring the Voice of Patients in the EU Pharmaceutical Legislation*

15:50 *Closing Statements*

WiFi: *(to be inserted)*

Social media: *@ipopi_info*

Hashtag:

- *#PIDForum*
- *#Pharmapackage*

MEP Billy Kelleher

(Renew, Ireland)

Welcome Address

MEP Cyrus Engerer

(S&D, Malta)

Welcome Address

MEP Tomislav Sokol

(EPP, Croatia)

Welcome Address

Setting the Scene

Leire Solis, Health Policy and Advocacy Senior Manager

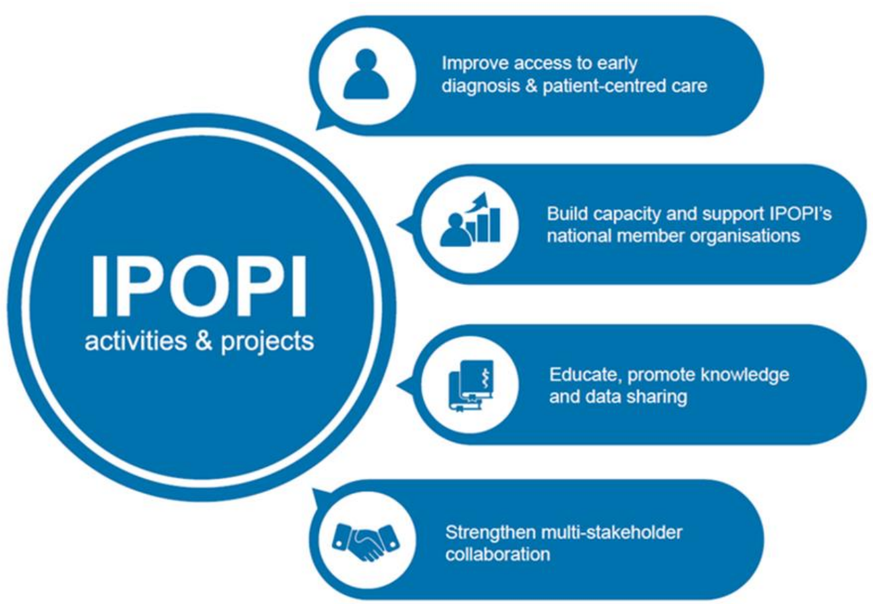
IPOPI

Introduction to IPOPI

The association of national patient organisations dedicated to improving:

- Awareness
- Access to early diagnosis
- Access to care

For patients living with primary immunodeficiencies (PIDs) worldwide



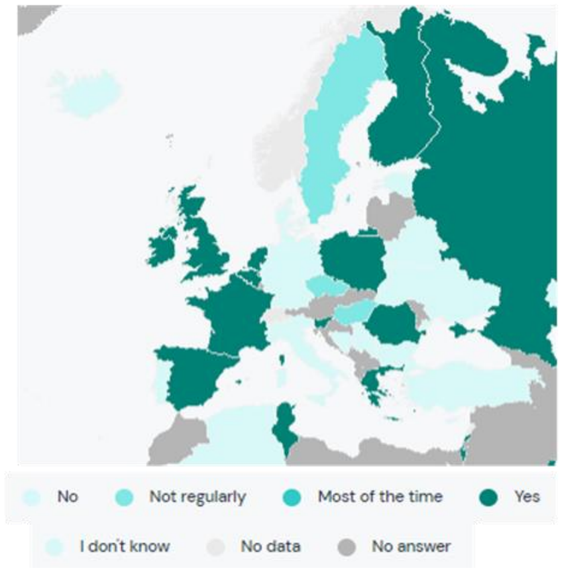
What are Primary Immunodeficiencies (PID)

- 485 different genetic rare and chronic diseases
- The immune system does not work properly or at all
- Affect children and adults
- Depending on the PID, patients can have:
  - Opportunistic infections
  - Persistent inflammation of internal organs
  - Autoimmunity
  - Severe allergies
  - Malignancies
  - Delayed growth and development
- Life-impairing and life-threatening lifelong conditions

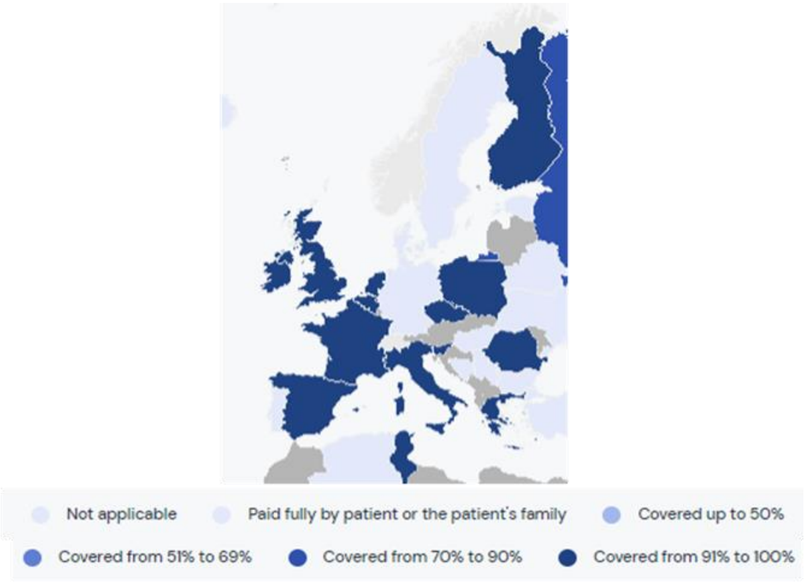
Why are we interested in the pharmaceutical package?

Antimicrobials	Antimicrobial resistance Shortages of some formulations Prophylactic treatment
Vaccines	Sporadic shortages
Immunoglobulin (Ig) replacement therapies	Recurrent shortages or tensions Uneven access to prescribed therapies
Advanced therapeutical medicinal products (ATMPs)	Uneven access in countries Withdrawals from the market Therapies only available in the US
Unmet medical needs	Not all PIDs have an adequate treatment Treatment burden

Approval ≠ availability ≠ affordability



Availability of thrombopoietin receptor agonist



Coverage/reimbursement of thrombopoietin receptor agonist

Data from PID Life Index www.pidlifeindex.ipopi.org

Key aspects of the legislation from a PID perspective

A welcomed proposal with certainly the ambition to be patient-centered and ensure quicker access to therapies in a sustained manner.

- Ensuring quicker access to new treatments & ATMPs in a sustained manner
- Tackling & preventing shortages
- Smooth interplay between existing & future legislation
- Increased patient representation & meaningful involvement

Ensuring quicker access to new treatments & ATMPs in a sustained manner

+ Reduced timelines for EMA approval

+ (in general) Simultaneous launch of new therapy in all member states?

? What happens with more specialised therapies for which the expertise / infrastructure is only present in a few member states?

Tackling & preventing shortages

+ (in general) Willingness to address supply & availability challenges

+ (in general) Increasing the notification requirements before the shortages occur

? Are the timelines suggested relevant for all therapies?

? What happens with therapies for which the availability is rather limited?

+ List of most critical medicines & recommendations on measures to be taken to improve security of supply

? Will these recommendations be shaped around the specificities of the therapies? ⟶ contingency stocks for immunoglobulins

Smooth interplay between existing & future legislation (1)

! Interplay with European Reference Networks – as a way of increasing access to knowledge, treatment and care for rare diseases

? A way of making more advanced medicines available through the networks?

! Interplay with the Cross-border healthcare directive and Regulation of the social security systems

? Is it clear what diagnostics / treatments can patients access abroad?

Smooth interplay between existing & future legislation (2)

! Interplay with the future SoHO

recurring plasma supply challenges with an impact on access to Igs

? Contingency stocks of plasma?

? Contingency stocks of immunoglobulins?

? How to tackle shortages when you lack the active ingredient?

⟶ Need to establish synergies to ensure both legislative texts go in the same direction: optimisation of the EU healthcare ecosystem

Increased patient representation & meaningful involvement

+ Patient representation in the EMA CHPM

? Patient representation in other EMA working parties?

? Patient representation in other areas such as the List of Critical Medicines

→ Definition of “unmet medical need” – patients need to be part of the discussions

What the Pharmaceutical Legislation brings to Patients

Julia Schmitz, Policy Officer, European Commission,
DG SANTE, D1 Medicines: policy, authorisation and monitoring

The EU Pharmaceutical Reform

DG SANTE

Unit D1 Medicines: Policy, Authorisation and Monitoring

EU Pharmaceutical Reform

Builds on the **Pharmaceutical Strategy** for Europe (2020)

Supports EU citizens and industry

Addresses **long-standing challenges and public emergencies**

Marks a **European Health Union milestone**

A 4-part package

Chapeau communication

New Regulation

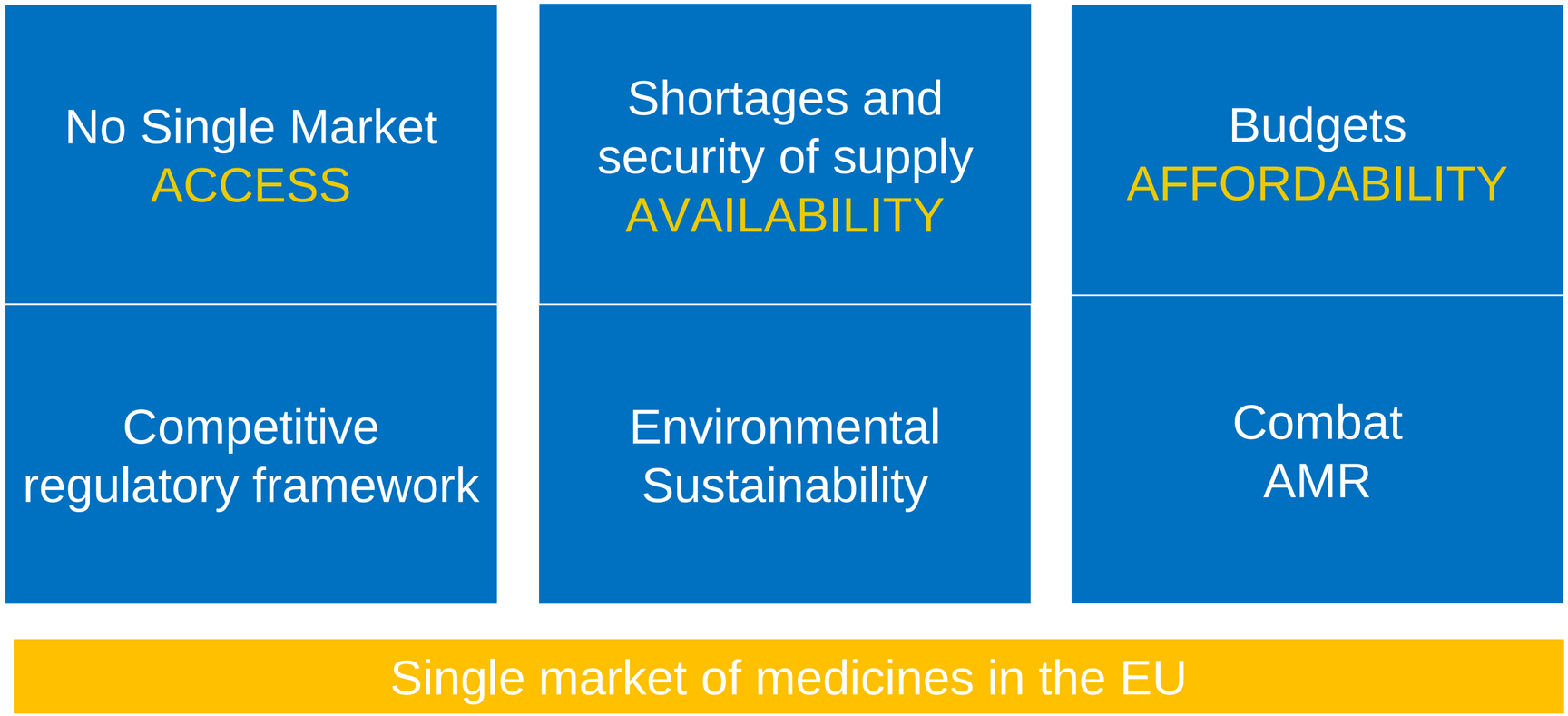
- Specific rules for the most innovative medicines such as orphans, antimicrobials
- Rules on shortages and security of supply
- EMA governance

New Directive

- Placing on the market of all medicines
- Authorisation and labelling requirements
- Strong incentives for access

Council Recommendation on AMR

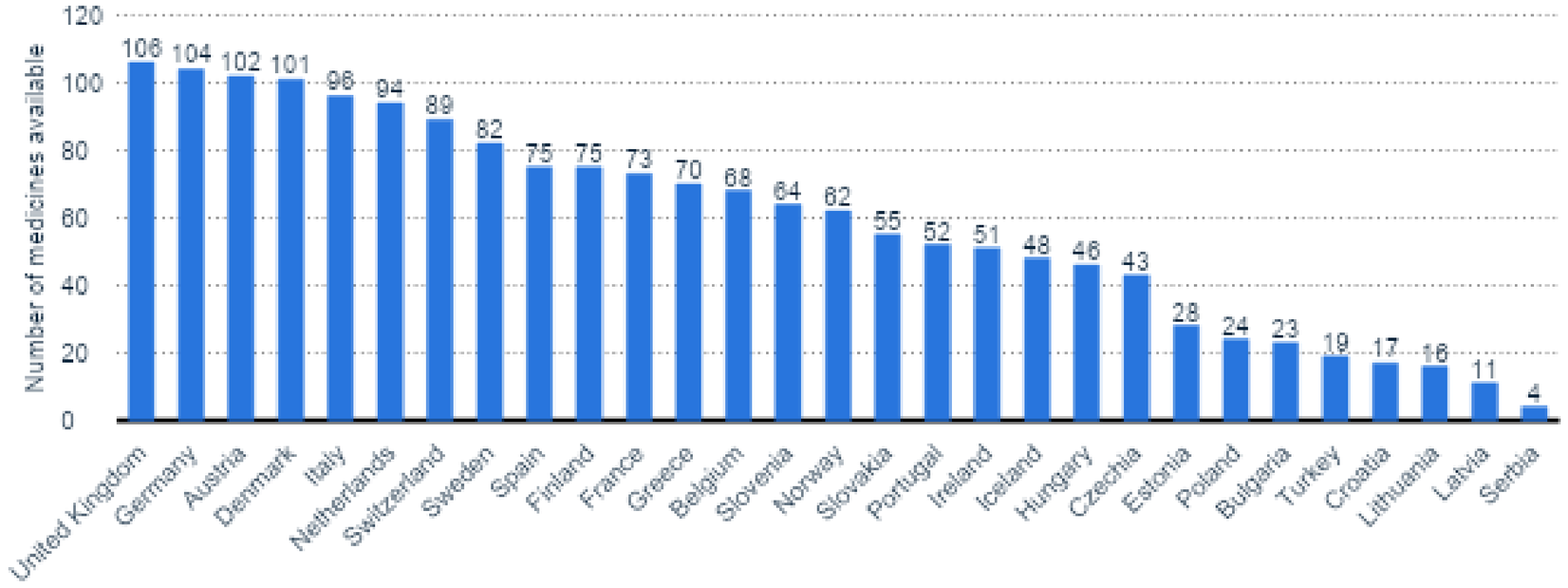
6 Key political objectives



Objective 1: Access to medicines

Current challenge: Access to new medicines varies across Europe

Number of medicines (approved by EMA between 2015-17) available to patients in Europe (as of 2018, by country)



Note(s): Europe; 2017
Further information regarding this statistic can be found on page 8.
Source(s): IQVIA; ID 1011132

statista

European Commission

Access to medicines

Current challenges

Access is not timely and differs across Member States:

90% variance between Northern/Western European countries and Southern/Eastern European countries

Average **waiting time** across the EU is **from 4 months to 29 months**

Proposed solutions

Incentives for innovation and access: More targeted approach vs current “one-size-fits-all” to regulatory protection incentives

Earlier market entry of **generic and biosimilar medicines**

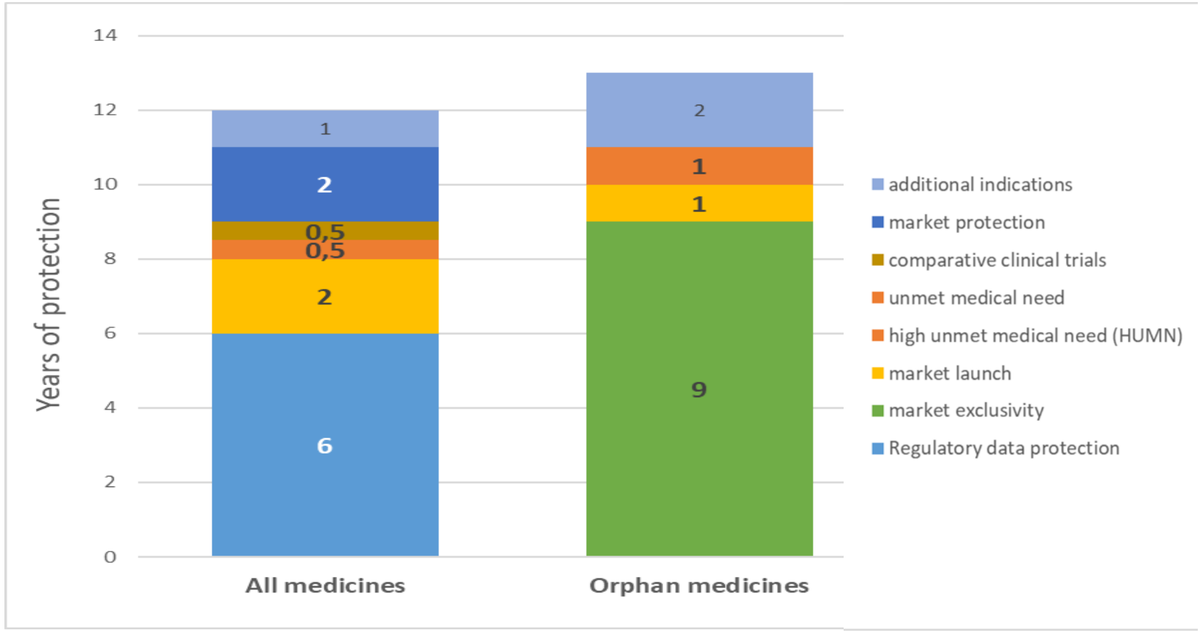
- **Faster authorisation**
- **Pre-authorisation support**

European Commission

More targeted regulatory protection incentives

Modulation of data protection

Modulation of market exclusivity



max 12 years protection

max 13 years protection (orphan medicines)

Targeted incentives for:

- Market launch in all EU Member States
- Addressing (high) unmet medical need

European Commission

Objective 2: Availability

Shortages of all medicines and security of supply of critical medicines

Challenges

Shortages: Multiple root causes

- Quality and manufacturing issues; commercial reasons, including market withdrawals, and unexpected increases in demand
- EU dependency on non-EU countries for medicines for supply of certain pharmaceutical ingredients

Growing concern for all EU countries

- **Critical shortages** of medicines; current examples thrombolytics, antibiotics
- Security of supply of **critical medicines**

Ad hoc processes for dealing with critical shortages

Proposed solutions

Improved **coordination, monitoring and management** of shortages, in particular critical shortages (MS and EMA) **Earlier** and **harmonised notification** of shortages and withdrawals (industry)

Shortage Prevention Plans

Union list of critical medicines

Stronger coordinating role for **EMA &** more powers for **Commission** (to impose a requirement for contingency stocks or other measures to improve security of supply of critical medicines)

Outside pharma package

- Other **Commission initiatives**, including the work of **HERA**
- **Joint Action** on shortages
- **IPCEI** in the area of health
- **National measures** e.g. State aid
- **EMA mandate extension** (Regulation (EU) 2022/123)

European Commission

Objective 3: Affordability

Current challenges

Pricing, reimbursement and procurement of medicines is a national competence

High prices endanger **health systems sustainability** & restrict patient access

Lack of transparency of public funding is a growing issue

Need to increase/strengthen cooperation among national authorities

Proposed solutions

Earlier market entry of generics/biosimilars to increase competition and reduce prices

Increased **transparency on public contribution** to R&D

Comparative Clinical Trials to support national decisions on pricing

Further support for **information exchange** between Member States (cooperation on pricing, reimbursement and payment policies)

Objective 4: Competitive regulatory framework

Current challenges

Longer approvals times than in other regions (US 244 days)

Administrative burden and compliance costs for the industry

The clock stop mechanism

Proposed solutions

Faster autorisation:
a) 180 days standard procedure
b) 150 days accelerated procedure

Regulatory efficiency/streamlining
simplified procedures, better use of data and digitisation

Pre-authorisation support
to promising medicines (e.g. PRIME), targeted support (SMEs, not-for-profits)

Future-proofing (e.g. adapted frameworks, regulatory sandboxes)

Objective 5: Environmental sustainability

Current challenges

Pharmaceuticals in environment can **harm environment and human health**

Presence of antimicrobials in the environment exacerbates AMR

Weak enforcement of current rules

Proposed solutions

Better enforcement of the current rules on **Environmental Risk Assessment** (part of the application)

Extending ERA to **medicines already on the market before 2005**

Stricter environmental rules for AMR, also covering manufacturing

Electronic leaflet and **electronic submission** of applications

European Commission

Objective 6: Combatting AMR

Current challenges

AMR causes **37000 deaths per year** in the EU.

It amounts to +/-1.5 bn EUR per year in healthcare costs

By 2050, **10 million deaths globally each year**

Current market failure
Lack of effective antimicrobials

Lack of market incentives
0,5 bio EUR cost of a new antibiotic

AMR toolbox

Measures on **prudent use of antimicrobials** – prescription, restricted quantities, education etc.

Regulatory incentives with **transferable exclusivity vouchers** under strict conditions (AMR voucher)

*Financial incentives with **procurement mechanisms** (HERA)*

*5 Targets, incl on the total **EU consumption of antibiotics for humans** (ECDC) reduction by 20% by 2030 (Council Recommendation)*

European Commission

Key benefits for patients

- **More targeted incentives** for medicines that **address unmet medical needs**
- **Faster authorisation of medicines** (timelines, but also scientific advice)
- **Increased patient representation under EMA structural changes** (in CHMP)
- **Improved patient access to medicines** (across EU Member States)
- **Improved availability of medicines** (addressing shortages**)**
- **More cooperation of public authorities** (marketing authorisation, HTA, P&R), with possibilities for stakeholder involvement

European Commission

Thank you

European Commission

Panel Discussion: The Pharmaceutical Legislation's Impact on Rare Disease and PID Communities

- **Luisa Antunes,** Policy Analyst, Directorate-General for Parliamentary Research Services
- **Juan García-Burgos**, Co-Chair of the Patients' and Consumers' Working Party, European Medicines Agency
- **Otilia Stanga**, President, ARPID
- **Julia Schmitz**, Policy Officer, European Commission, DG SANTE, D1 Medicines: policy, authorisation and monitoring

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Added value of patient input in EMA activities

Patient engagement – added value and impact

Scientific Advice

- 4 year study published
- Added value of patient input quantified and demonstrated

CHMP early contact

- 17 month pilot completed
- Positive impact – will be maintained as new methodology

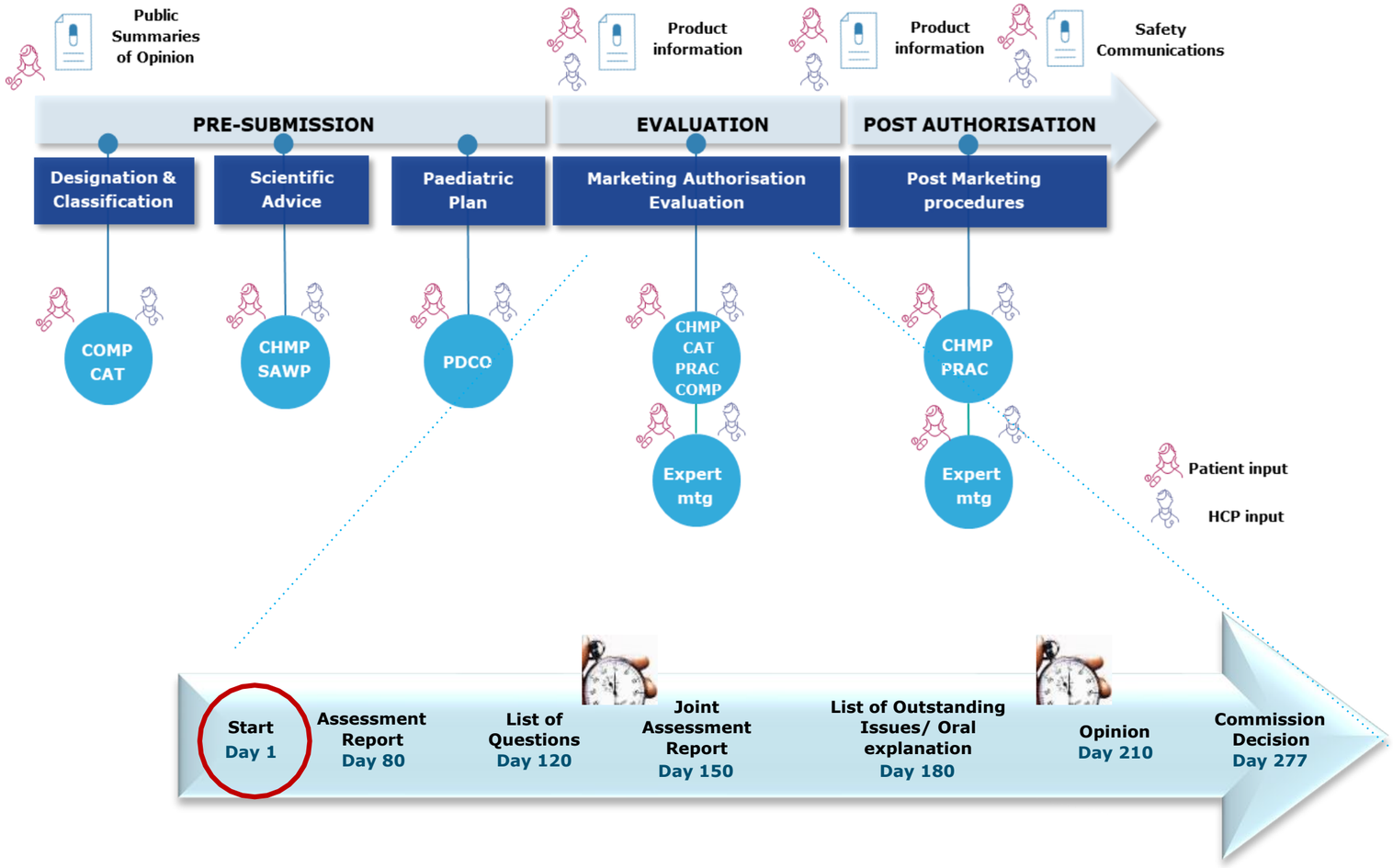
Review of documents

- Comments and suggestions by patients incorporated into published documents
- Template structure changed

Safety monitoring

- Public hearings – recommendations leading to risk minimisation measures

CHMP early contact with patient organisations



- Relevant organisations contacted at start of orphan MAA's
- Patient organisations invited to share key aspects from their perspectives of living with the condition (3-4 weeks to respond) (in advance of first AR).
- Information shared with (Co-) Rapporteurs (and company for transparency) - Rapps decide if information provides added value, is useful for assessing the dossier, and if merits being included in AR.
- Value of patient input received during pilot assessed by short questionnaire

Pilot outcome summary

- 37 procedures over 17 months (2021-2022)
- Rapporteurs were positive and input received reflected usefulness and benefit of reaching out to patient organisations at start of assessment of MAA's.
- Patients provided new insights that contributed to the D80 assessment report.
- 41% of cases contributed to the development of the first assessment report
- Information from patients related to *daily impacts*, *treatment options*, *perspectives and perceptions of adverse effects*, *what constitutes important improvements and desired benefits for new treatments* have proven to be insightful / helpful
- Pilot now a new methodology to be continued and extended to medicines of potential significant impact.

Classified as public by the European Medicines Agency

Reflecting patient perspective in the CHMP assessment

23 June 2022
EMA/CHMP/597782/2022
Committee for Medicinal Products for Human Use (CHMP)

CHMP day 120 list of questions

Overview and list of questions

Patient's engagement

Being engaged in the EMA pilot "*CHMP early contact with patient organisations*", the EMA contacted relevant patient organisations for Fabry disease during the first round of this procedure. The aim of the pilot is to enable patients to share their experience, concerns and needs related to their condition with the Rapporteurs/CHMP so that these can be considered in a timely manner during the assessment process, where appropriate.

The information in this section was received from the patients' organisations relating to Fabry disease; their feedback has been considered during the assessment of this procedure.

Fabry disease is a life-threatening, complex multi-organ disease. In addition to the life-threatening aspects of the disease, there are many symptoms that severely affect the patients´ wellbeing and quality of life on a daily basis (such as constant pain, GI symptoms or fatigue). There are several ERT

Reflecting patient perspective in the CHMP assessment report

Amsterdam, 10 November 2022
EMA/CHMP/762284/2022
Committee for Medicinal Products for Human Use (CHMP)

CHMP Day 180 second list of outstanding issues

2.1.6. Patient's engagement

Being engaged in the EMA pilot "*CHMP early contact with patient organisations*", the EMA contacted relevant patient organisations for Pompe disease during the first round of this procedure. The aim of the pilot is to enable patients to share their experiences, concerns and needs related to their condition with the Rapporteurs/CHMP so that these can be considered in a timely manner during the assessment process, where appropriate.

The information in this section was received from the patients' organisations relating to Pompe disease; their feedback has been considered during the assessment of this procedure.

All patients expressed the need to be able to adjust the dose of their enzyme replacement therapy until the optimum levels are reached (personalised dosing).

No limits in terms of manufacturing capacities should restrict the ability to use higher doses (Genzyme had experienced tensions on supply due to higher demand than expected back in 2008, but since then, no biosimilar has been introduced on the market, the price has not changed, and not all member states agree to cover higher doses).

Most patients expect that a new treatment could stabilise the disease more than existing ones; some recovery would, of course, be welcomed, but experience with alglucosidase alfa might limit this expectation.

With miglustat, diarrhoea is reported the day the product is taken, which can exacerbate this symptom for people with Pompe disease suffering from GI disorders. These episodes can be controlled (no carbohydrate products ingested the day before, and some medications can also help).

As most patients are taking alglucosidase alfa already, the administration of the miglustat and cipaglucosidase alfa combination poses no problem. However, the switch might require returning to the hospital for a short time for those receiving infusions at home, which could be a concern during the Covid-19 pandemic.

Home infusions are not applied in all member states or regions. Otherwise, when applied, all patients

Beyond the pilot phase

- The early contact methodology has now become a regular part of CHMP's contact with stakeholders.
- Now include all indications and not only rare diseases.
- Also healthcare professional organisations to be consulted.

Added value of patient engagement in crisis

- Gather critical input into crisis-related activities in COVID-19 context
- Gain insight into concerns of specific groups of patients e.g. about vaccination
- Support specific information needs, e.g. discussions on vaccines, associated social challenges, hesitancy, review of safety communications to the public…
- Channel public health messages to communities of patients and citizens more effectively
- Reinforce legitimacy of actions, trust in scientific outcomes and EU system

Any questions?

Further information

juan.garcia@ema.europa.eu

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Telephone +31 (0)88 781 6000
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Back – up slides

Data of early patients contact after the pilot (since 2022)

Month/year	Type of procedure	# responses from PCO
September 2022	3 orphans	1 response
October 2022	3 orphans 2 non orphans	4 responses
1 December 2022	3 orphans 2 non orphans	3 responses and 1 use of previous response for same indication
28 December 2022	1 orphan 1 non orphan	2 responses
24 January 2023	3 orphans 3 non orphans	ongoing

Interaction between CHMP and **Patients' representatives**

Participation in CHMP activities: 2020-2022

- **Contributing for decision on recommendations**

Number interactions	CHMP Activity
2020 – 42 (22 meetings) **2021 – 25 (14 meetings)** **2022 – 33 (15 meetings)**	**Scientific Advisory Groups/ Ad hoc Expert Groups** (neurology, oncology, haematology, viral disease)
2020 – 102; 2021 – 90	**Scientific advice, protocol assistance**
2020 – 10 (6 procedures - Hopeveus, Dapavirine, Arikayce, Gamifant, Fintepla, Sogroya**)** **2021 – 7 (5 procedures -** Evrysdi, Zolgensma, Ozawade, Raylumis, Tecentriq**) 2022 – 2 (1 procedure -** Miplyffa**)**	**Oral explanations**

Interaction between CHMP and **HCP representatives**

Participation in SAG and Ad-hoc Experts Groups – 2020– 2022

- **Contributing for decision on recommendations**

Number interactions	CHMP Activity
2020 – 40 (18 meetings) **2021 – 25 (12 meetings)** **2022 – methodology changed**	**Scientific Advisory Groups/** **Ad hoc Expert Groups** (psychiatry, neurology, oncology, haematology, immunology, and respiratory diseases)
2020 – 1 **2021 – 4** **2022 – methodology changed**	**Scientific advice, protocol assistance**

Open Floor Discussion

Call to Action: Ensuring the Voice of Patients in the EU Pharmaceutical Legislation

Leire Solis, Health Policy and Advocacy Senior Manager, IPOPI

Closing Statement

MEP Cyrus Engerer

(S&D, Malta)

THANK YOU FOR ATTENDING

THE PID FORUM!

Stay tuned for more...

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