NAVIGATING THE COMPLEXITIES OF THE PHARMACEUTICAL LEGISLATION

An IPOPI Event
Hosted by MEPs Billy Kelleher (Renew), Cyrus Engerer (S&D) & Tomislav Sokol (EPP)
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 (PID) 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?

<table>
<thead>
<tr>
<th>Category</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Antimicrobials</td>
<td>Antimicrobial resistance, Shortages of some formulations, Prophylactic treatment</td>
</tr>
<tr>
<td>Vaccines</td>
<td>Sporadic shortages</td>
</tr>
<tr>
<td>Immunoglobulin (Ig) replacement therapies</td>
<td>Recurrent shortages or tensions, Uneven access to prescribed therapies</td>
</tr>
<tr>
<td>Advanced therapeutical medicinal products (ATMPs)</td>
<td>Uneven access in countries, Withdrawals from the market, Therapies only available in the US</td>
</tr>
<tr>
<td>Unmet medical needs</td>
<td>Not all PIDs have an adequate treatment, Treatment burden</td>
</tr>
</tbody>
</table>
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 lgs
  
  ? 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

<table>
<thead>
<tr>
<th>Builds on the</th>
<th><strong>Supports</strong></th>
<th>Addresses long-standing challenges and public emergencies</th>
<th>Marks a European Health Union milestone</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Pharmaceutical Strategy</strong> for Europe (2020)</td>
<td><strong>EU citizens and industry</strong></td>
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A 4-part package

<table>
<thead>
<tr>
<th>Chapeau communication</th>
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<tbody>
<tr>
<td><strong>New Regulation</strong></td>
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<tr>
<td>- Specific rules for the most innovative medicines such as orphans, antimicrobials</td>
</tr>
<tr>
<td>- Rules on shortages and security of supply</td>
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<td>- EMA governance</td>
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<tr>
<td><strong>New Directive</strong></td>
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<tr>
<td>- Placing on the market of all medicines</td>
</tr>
<tr>
<td>- Authorisation and labelling requirements</td>
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<tr>
<td>- Strong incentives for access</td>
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<tr>
<td><strong>Council Recommendation on AMR</strong></td>
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</table>


6 Key political objectives

- No Single Market
  ACCESS

- Shortages and security of supply
  AVAILABILITY

- Competitive regulatory framework

- Environmental Sustainability

- Budgets
  AFFORDABILITY

- Combat AMR

Single market of medicines in the EU
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 1011138
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
More targeted regulatory protection incentives

Targeted incentives for:
- Market launch in all EU Member States
- Addressing (high) unmet medical need

Modulation of data protection
- 1 year
- 2 years
- 0.5 years
- 2 years

Modulation of market exclusivity
- 2 years
- 1 year
- 1 year
- 9 years

max 12 years protection
max 13 years protection (orphan medicines)
Objective 2: Availability
Shortages of all medicines and security of supply of critical medicines

Challenges

- 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)

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

Multiple root causes: Shortages
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 authorisation:
  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
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)
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
Thank you

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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
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.
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

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 clapglucosidase 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)

<table>
<thead>
<tr>
<th>Month/year</th>
<th>Type of procedure</th>
<th># responses from PCO</th>
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<tbody>
<tr>
<td>September 2022</td>
<td>3 orphans</td>
<td>1 response</td>
</tr>
<tr>
<td>October 2022</td>
<td>3 orphans, 2 non orphans</td>
<td>4 responses</td>
</tr>
<tr>
<td>1 December 2022</td>
<td>3 orphans, 2 non orphans</td>
<td>3 responses and 1 use of previous response for same indication</td>
</tr>
<tr>
<td>28 December 2022</td>
<td>1 orphan, 1 non orphan</td>
<td>2 responses</td>
</tr>
<tr>
<td>24 January 2023</td>
<td>3 orphans, 3 non orphans</td>
<td>ongoing</td>
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</table>
Interaction between CHMP and **Patients’ representatives**

Participation in CHMP activities: 2020-2022

- **Contributing for decision on recommendations**

<table>
<thead>
<tr>
<th>Number interactions</th>
<th>CHMP Activity</th>
</tr>
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<tbody>
<tr>
<td><strong>2020 – 42 (22 meetings)</strong></td>
<td><strong>Scientific Advisory Groups/ Ad hoc Expert Groups</strong> (neurology, oncology, haematology, viral disease)</td>
</tr>
<tr>
<td><strong>2021 – 25 (14 meetings)</strong></td>
<td></td>
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<tr>
<td><strong>2022 – 33 (15 meetings)</strong></td>
<td></td>
</tr>
<tr>
<td><strong>2020 – 102; 2021 – 90</strong></td>
<td><strong>Scientific advice, protocol assistance</strong></td>
</tr>
<tr>
<td><strong>2020 – 10 (6 procedures - Hopeveus, Dapavirine, Arikayce, Gamifant, Fintepla, Sogroya)</strong></td>
<td></td>
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<tr>
<td><strong>2021 – 7 (5 procedures - Evrydsi, Zolgensma, Ozawade, Raylumis, Tecentriq)</strong></td>
<td><strong>Oral explanations</strong></td>
</tr>
<tr>
<td><strong>2022 – 2 (1 procedure - Miplyffa)</strong></td>
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Interaction between CHMP and **HCP representatives**

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

- **Contributing for decision on recommendations**

<table>
<thead>
<tr>
<th>Number interactions</th>
<th>CHMP Activity</th>
</tr>
</thead>
<tbody>
<tr>
<td>2020 – 40 (18 meetings)</td>
<td><em>Scientific Advisory Groups/ Ad hoc Expert Groups</em> (psychiatry, neurology, oncology, haematology, immunology, and respiratory diseases)</td>
</tr>
<tr>
<td>2021 – 25 (12 meetings)</td>
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<tr>
<td>2022 – methodology changed</td>
<td></td>
</tr>
<tr>
<td>2020 – 1</td>
<td><em>Scientific advice, protocol assistance</em></td>
</tr>
<tr>
<td>2021 – 4</td>
<td></td>
</tr>
<tr>
<td>2022 – methodology changed</td>
<td></td>
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</table>
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...
NAVIGATING THE COMPLEXITIES OF THE PHARMACEUTICAL LEGISLATION

PID FORUM

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