FONDAZIONE
PER LA RICERCA FARMACOLOGICA
GIANNI BENZI
ONLUS

APS Bottega del sorriso
ASSOCIAZIONE DI PROMOZIONE SOCIALE

Opportunities vs challenges for patients and future expectations

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The EU Pharma legislation promises

1. BETTER ACCESS TO EFFECTIVE AND AFFORDABLE MEDICINES
2. REDUCING SHORTAGES
3. MORE MEDICINES FOR CHILDREN AND RARE DISEASES
4. A STRONGER VOICE FOR PATIENTS
5. EASIER ACCESS TO INFORMATION
6. MORE ENVIROMENTALLY SUSTAINABLE MEDICINES

How the promises would be reached?

REPURPOSING TO COVER UNMET NEEDS

- ☺ Incentives on availability in MSs ⇨ *standard market protection extended if the product is made accessible in all MSs*
- ☺ More affordable/cheaper medicines ⇨ *periods of market protection reduced overall*
- ☺ Antimicrobials ⇨ new incentive – not used in the EU so far
- ☺ UMNs special support and incentives
- ☺ Repurposing pushed

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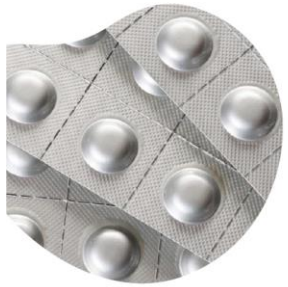
How the promises would be reached?

1 BETTER ACCESS TO EFFECTIVE AND AFFORDABLE MEDICINES



Strong incentives promoting accessible, available and innovative medicines:

- New medicines **more readily available** to citizens across the EU, in all Member States.
- **Generic and bio-similar medicines** more quickly available, increasing competition and lowering prices.
- More new medicines addressing **unmet medical needs**.
- More **off-patent medicines** repurposed for new therapies.
- More game-changing **antimicrobials**.

€ More transparency on public funding received by pharmaceutical companies to develop medicines will support national authorities in price negotiations with these companies and help make new medicines more affordable.

REPURPOSING TO COVER UNMET NEEDS

A DEDICATED ARTICLE IN THE PROPOSED REGULATION!

- ☺ Not-for-profit entities may submit non-clinical or clinical evidence for a new therapeutic indication for unmet needs
- ☺ Opinion favourable ⇨ MAH shall submit a variation to update the product information with the new therapeutic indication

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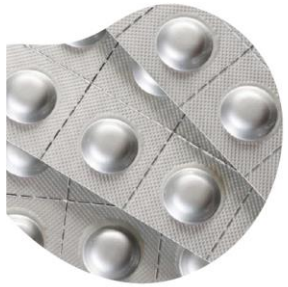

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REPURPOSING TO COVER UNMET NEEDS

- Paediatric medicines ⇨ EMA may impose paediatric studies (PIP) in a disease different from the one proposed by the developer on the basis of the drug mechanism of action
- More information on off-label uses ⇨ paediatrics, pregnancy and unmet needs

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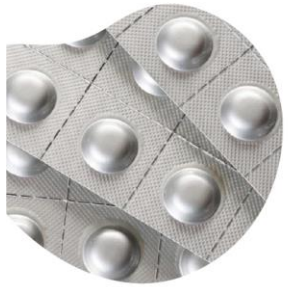

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…and quicker access expected!

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How the promises would be reached?

- Streamlining and simplification of procedures (better coordination between scientific committees, transferring the responsibility for orphan designation to the Agency, introduction of a Global Marketing Authorisation)
- Reduced timelines (e.g. reduction of the scientific evaluation period) + specific incentives to quickly bring a medicine on the market

…and quicker access expected!

How the promises would be reached?

2 REDUCING SHORTAGES

Better availability of medicines, in particular critical medicines, to EU citizens at all times:

- **Earlier warnings** from companies on shortages and withdrawals of medicines, including establishment of prevention plans.
- Establishment of **list of medicines critical for EU health systems**, helping to identify supply chain vulnerabilities and improve security of supply.
- **Better monitoring and mitigation of shortages**, at both national and EU level, with a stronger guiding role for the European Medicines Agency and the European Commission on security of supply.

More duties for authorities and companies, e.g.

- Earlier notification of possible shortages
- Obligation to offer MA transfer to another company before withdrawal

- No role for patients
- Reporting and monitoring harmonised/centralised… as far as marketing is nationally-based…

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How to reach the Pharma promises?

3 MORE MEDICINES FOR CHILDREN AND MEDICINES FOR RARE DISEASES

- Quicker access to **more therapeutic solutions** for children and patients suffering from rare diseases.
- New incentives for **more research and investment in medicines** for children and for patients suffering from rare diseases.
- Creation of a European network of patient representatives, academics, medicines developers, investigators and centres with expertise to support with the development of medicines for children.
- Screening of all **new medicines for potential use in treating children**.
- More **research into rare diseases**.

- Attempt to simplify regulatory procedures ⇨ quicker authorisation expected
- + obligations, e.g. to study medicines for children in certain situations not considered in the Paediatric Regulation
- + rewarded innovation in orphan R&D

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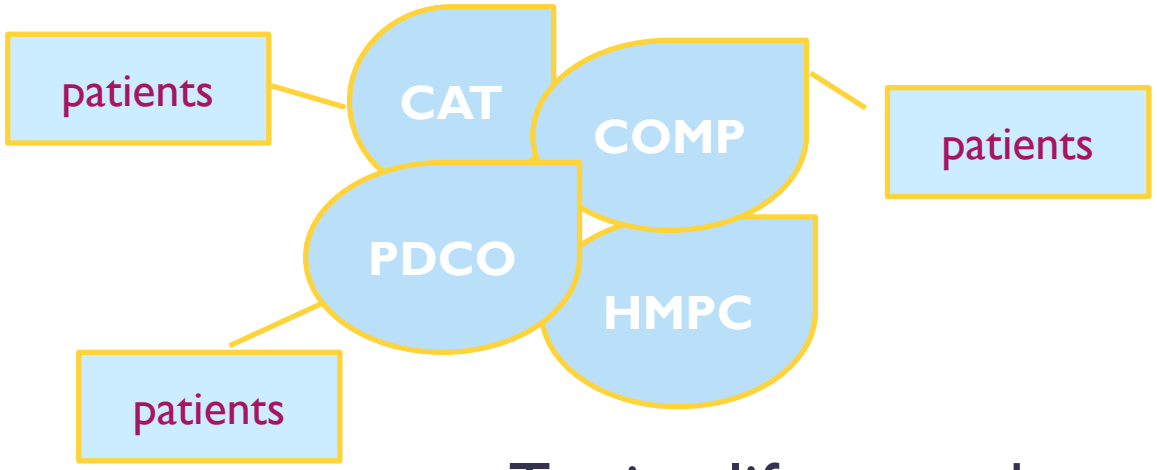
How to reach the Pharma promises?

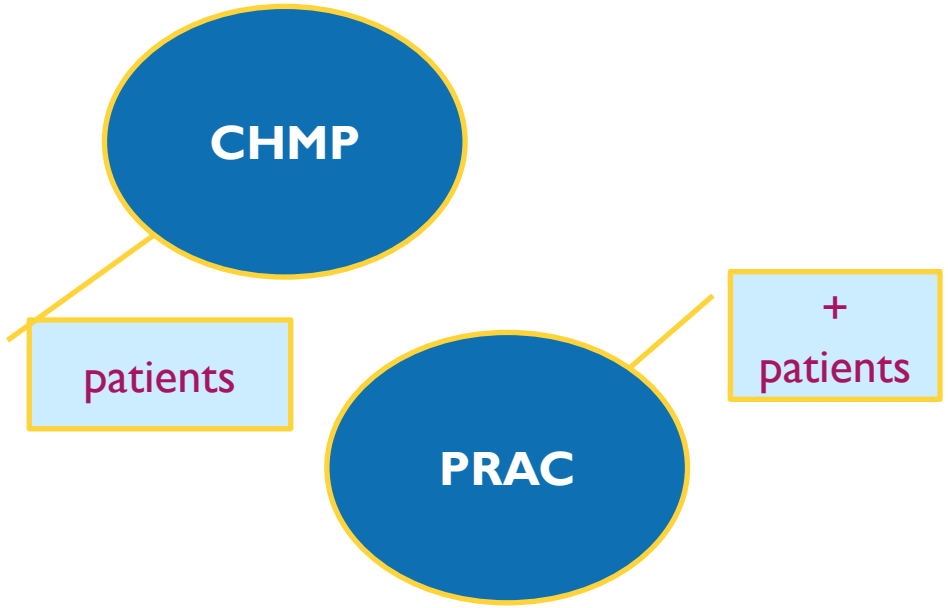
4 A STRONGER VOICE FOR PATIENTS

- Increased representation of patients in the approval of medicines, including through the appointment representatives in EMA Committees.

- Patient representatives:
  - CHMP: 4 members + 4 alternates
  - PRAC: 2 members + 2 alternates

~~COMMITTEES~~ ⇨
WORKING PARTIES/EXPERTS





- To simplify procedures
- To remove inconsistencies between opinions

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How to reach the Pharma promises?

5 EASIER ACCESS TO INFORMATION

Electronic product information:

Access for patients and healthcare professionals to the **latest information** about their medicines

Reducing paperwork for medicine-developers and generics companies **reducing costs and addressing shortages**

Information to be provided on:

- public funding for R&D including clinical trials
- EMA internal rules and procedures

- Obligations to provide additional information on environmental risk
- Additional responsibilities for companies

6 MORE ENVIROMENTALLY SUSTAINABLE MEDICINES

Production of medicines in a way that is better for the environment, biodiversity and human health

- **Strengthened environmental risk assessments for all medicines,** including those already authorized, to limit the potential adverse effects of medicines on the environment and public health.
- **Fewer pharmaceutical substances** including antimicrobials **in the environment** due to more environmentally friendly production, use or proper disposal of medicines.

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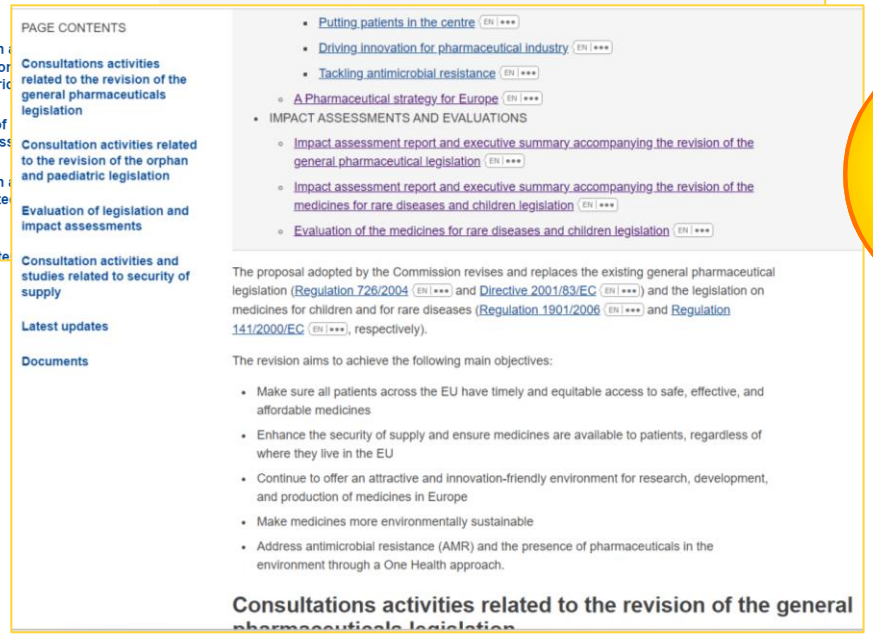
Where



- Lots of documents…
- Orphan and Paediatric Regulations merged with the pharma legislation

https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe/reform-eu-pharmaceutical-legislation_en

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Challenges

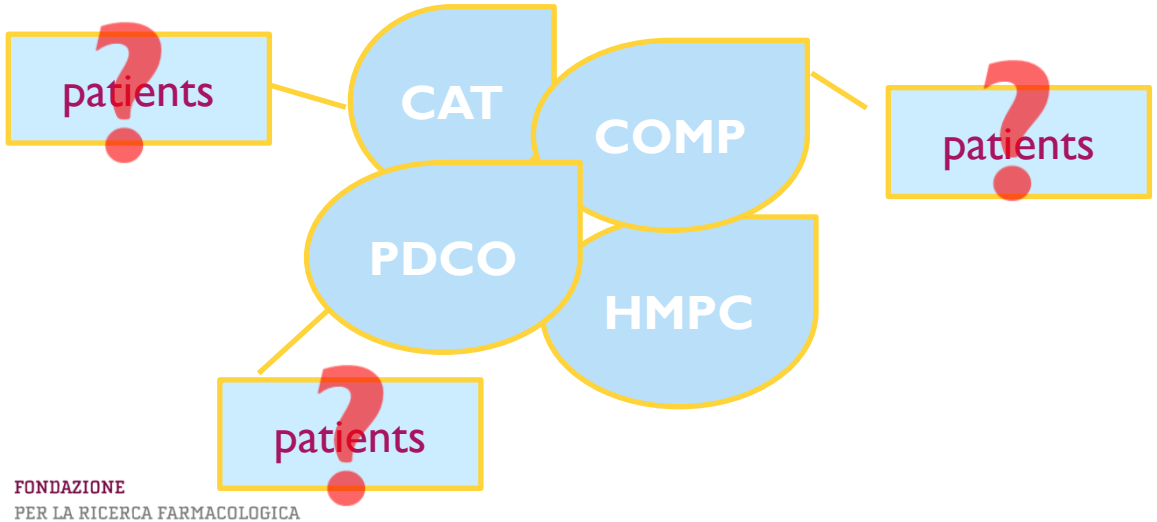
Strenghtening patients involvement

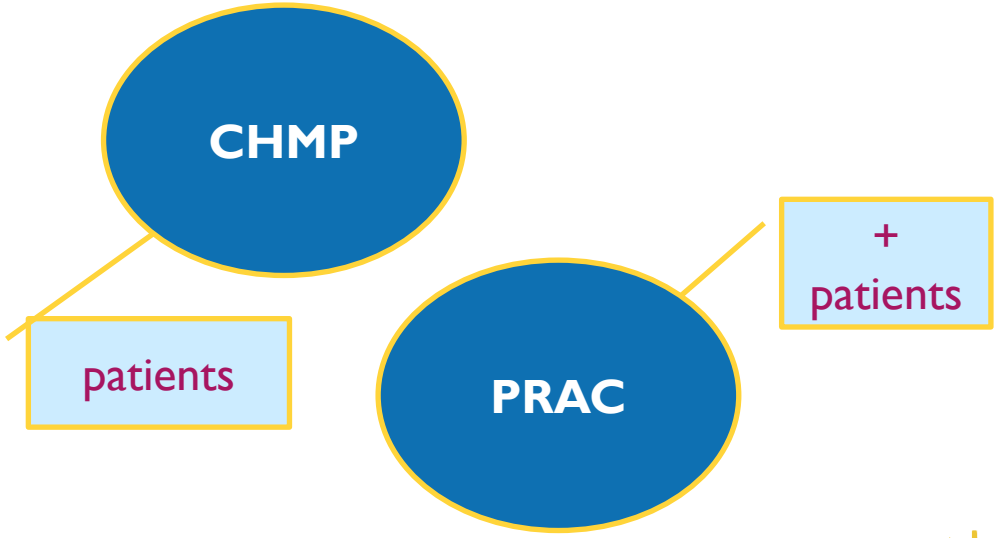
4 A STRONGER VOICE FOR PATIENTS

- Increased representation of patients in the approval of medicines, including through the appointment representatives in EMA Committees.

- Patients should be more 'expert' to join the CHMP
- More involvement of patients?

~~COMMITTEES~~ ⇨ WORKING PARTIES/EXPERTS





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Well-identification of unmet needs

- Criteria established in a directive ⇨ any difference by countries?
- Specified (what do "*satisfactory method*", "*remaining high morbidity or mortality*", "*relevant patient population*" mean?) and updated by EC/EMA ⇨ input from authorities/bodies and EU/national initiatives
- All orphans address unmet needs…incidence threshold not set ⇨ no harmonisation

Article 83

Medicinal products addressing an unmet medical need

1. A medicinal product shall be considered as addressing an unmet medical need if at least one of its therapeutic indications relates to a life threatening or severely debilitating disease and the following conditions are met:
   (a) there is no medicinal product authorised in the Union for such disease, or, where despite medicinal products being authorised for such disease in the Union, the disease is associated with a remaining high morbidity or mortality;
   (b) the use of the medicinal product results in a meaningful reduction in disease morbidity or mortality for the relevant patient population.
2. Designated orphan medicinal products referred to in Article 67 of [revised Regulation (EC) No 726/2004] shall be considered as addressing an unmet medical need.
3. Where the Agency adopts scientific guidelines for the application of this Article it shall consult the Commission and the authorities or bodies referred to in Article 162 of [revised Regulation (EC) No 726/2004].

- Unclear how these medicines will be identified ⇨ multi-stakeholders consultation?
- Which role for patients? …*should be key!*

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Efficiently streamilined procedures to provide quicker patients with medicines

Some measures streamlining procedures, but… would it be reached?

- Simplification expected turning committees into working parties ⇨ *further efforts expected from CHMP*
- Introduction of Orphan Designation validity ⇨ *more work needed from EMA*
- Modified PIP procedure: evolutionary and simplified PIP ⇨ *how much streamlined?*

More transparency provisions on trials

***Publicly-available infos on trials and medicines**: an opportunity for patients to know what is on the market, how it has been evaluated and the trials conducted*

- Transparency on trial funding impacts on patients
- …but more tangible advantages for patients will come from the CTR implementation making mandatory the publication of trials (we learnt from EudraCT and the EU trial register that if not mandatory, the trials publication risks to not be published)

Alignment across EU countries reacheable?

The revision aims to

- "make sure all patients ***across the EU*** have timely and equitable access to safe, effective, and affordable medicines"
- "enhance the security of supply and ensure medicines are available to patients, ***regardless of where they live in the EU***"

☺ Incentives on availability in MSs ⇨ standard market protection extended if the product is made accessible in all MSs

☺ Reporting and monitoring of shortages harmonised/centralised

More alignment with other regions?

Only proposed provision allowing + alignment with the US: EU Market Exclusitivty for orphans reduced (5-10 years based on the type of medicine) ⇨ closer to US (7 years)

…but…

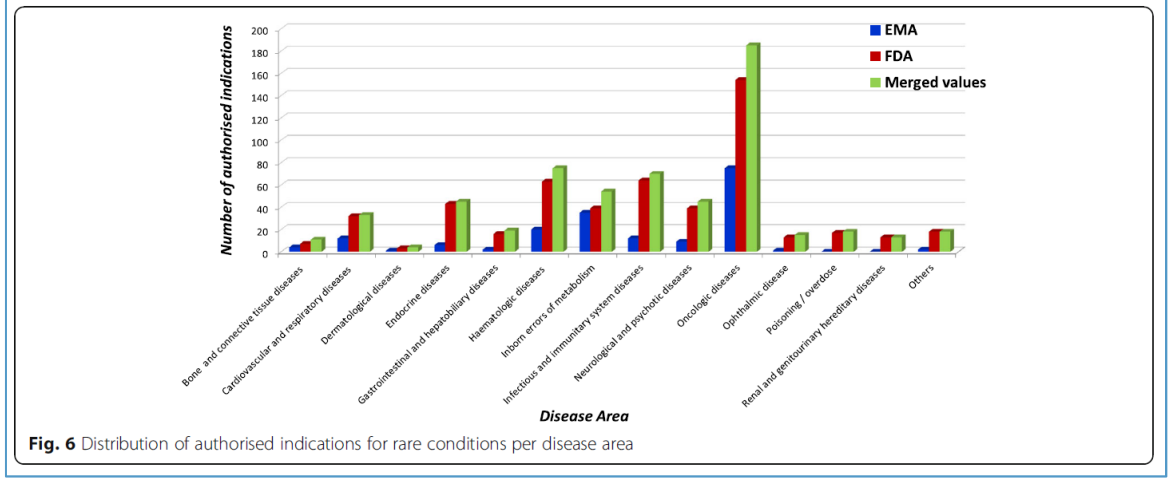

Fig. 6 Distribution of authorised indications for rare conditions per disease area

Table 1 Key incentives of the orphan legislation in the EU and US

Incentives	In EU	In US
Marketing exclusivity	10 years + 2 if paediatric	7 years
Clinical development costs	–	tax credits (up 50% of clinical development costs)
Orphan designation	free of charge	free of charge
Support from agency during the development process	free of charge protocol assistance	free of charge OOPD (Office of orphan Products Development) assistance
MAA	40% fee reduction; free of charge for SMEs and for paediatric products	fee reduction
Fee reductions for SMEs	90% of fee reduction for post authorisation inspections; free of charge pre-authorisation inspections, post-authorisation activities, including annual fees, during the first year after marketing authorisation	–
Public funds	(possible) incentives from EC (i.e. research grants)	grants and contract for development of orphan drugs
	(possible) incentives in single Member States for research, development and MA	

Giannuzzi V et al. OJRD, 2017

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Conclusions

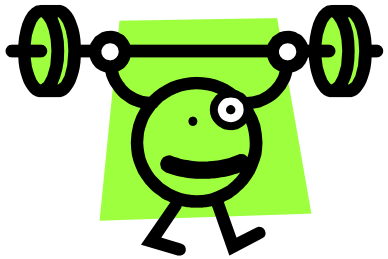
Important provisions proposed to improve the pharma system

- some of them to be implemented for the first time
  - umet medical need incentives
  - obligation to conduct paediatric studies on the basis of the drug mechanism of action
  - special measures for repurposing
- others more strongly addressed through
  - simplification of procedures
  - generics support

…but…..

Challenges

- Strenghtening patients involvement
- Well-identification of unmet needs
- Efficiently streamilined procedures to provide medicines quicker
- Enhanced transparency not only trials
- Alignment across EU countries
- Alignment with other regions



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