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SCIENCE MEDICINES HEALTH

Programmes for academic developers: PRIME, Orphan medicines, Paediatric medicines, Advanced therapy medicines, Repurposing medicines

Session 4 - Fostering innovation in medicines

Academia Info Day 10 November 2023

Presented by Ralf Herold, Regulatory Science & Academia Workstream (TRS-ACD),
Regulatory Science and Innovation Task Force (TRS)

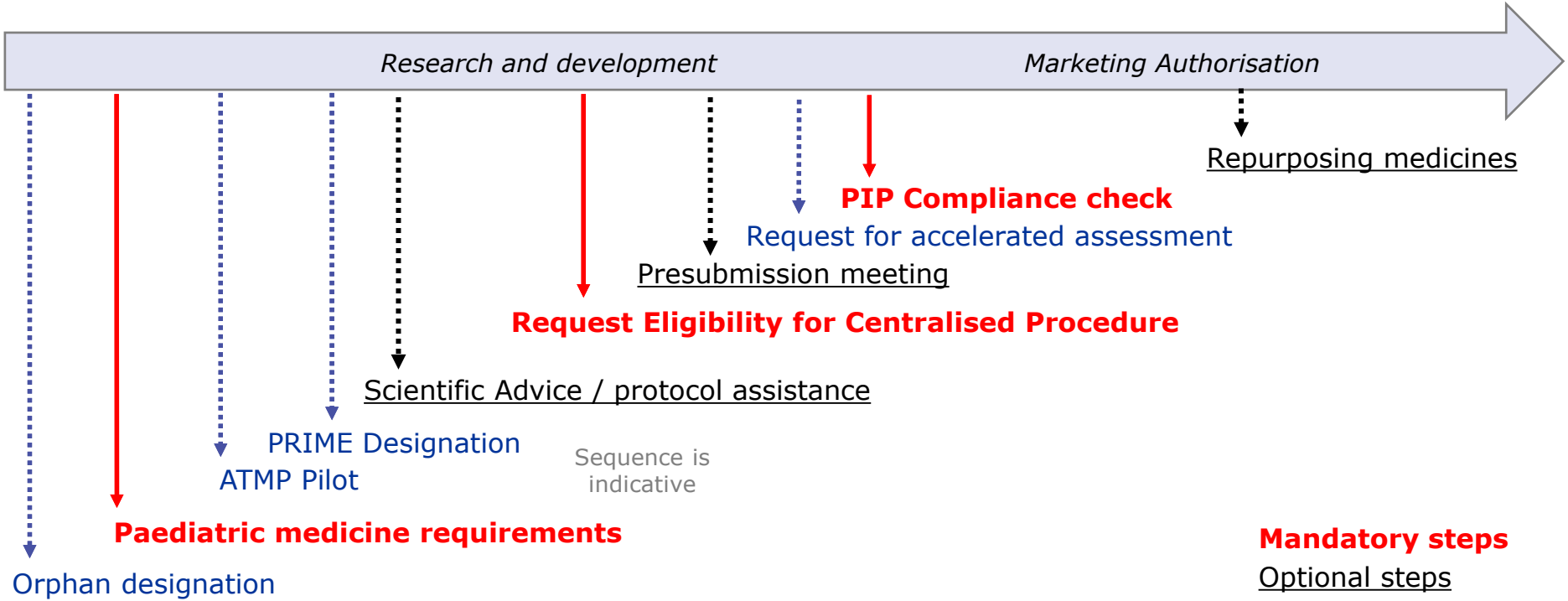
Agenda

- Overview on EU Medicines regulatory programmes that address unmet needs and
- that are of interest to academia,
- Flagging opportunities and obligations for innovators / developers and
- Sharing a perspective of involved EMA staff members

1. PRIME: Priority medicines scheme
2. Orphan medicines
3. Paediatric medicines
4. Advanced therapy medicinal products
5. Repurposing medicines

1 Programmes for academic developers: PRIME, Orphan medicines, Paediatric medicines, Advanced therapy medicines, Repurposing medicines

Development and pre-submission activities for medicines



Mandatory steps

Optional steps

Not applicable to all

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PRIME: Priority medicines scheme

PRIME scheme - Goals & Scope

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Supporting patient access to innovative medicines

Medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation.

- Potential to address to a significant extent an unmet medical need

Reinforce scientific and regulatory advice

- Foster and facilitate early interaction
- Raise awareness of requirements earlier in development

Optimise development for robust data generation

- Focus efficient development
- Promote generation of robust and high-quality data

Enable accelerated assessment

- Promote generation of high-quality data
- Facilitated by knowledge gained throughout development

PRIME scheme – eligibility and enhanced support

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SMEs
Academia

Proof of principle

- Sound pharmacological rationale, convincing scientific concept
- Relevant nonclinical effects of sufficiently large magnitude and duration
- Tolerability in first in man trials

Proof of concept

- Sound pharmacological rationale
- Clinical response (efficacy) and safety data in patients (exploratory trials)
- Magnitude, duration, relevance of outcomes to be judged on a case by case basis → indicating substantial improvement

Any sponsor

Nonclinical | **Phase I** | **Exploratory** | **Confirmatory** | **Evaluation**

- **Dedicated EMA contact point (PRIME Sci Co-ord)**
- **Early CHMP Rapporteur appointment**

Kick-off Meeting

SA 1 (SAWP) | **SA 2 (SAWP)** | **SA n (SAWP)**

Accelerated Assessment confirmation (CHMP)

https://www.ema.europa.eu/en/human-regulatory/research-development/prime-priority-medicines

Orphan medicines

Support for Orphan medicinal products

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Patients suffering from rare diseases deserve access to the same quality of medicinal products as other patients within the European Union (EU)

Without incentives it is unlikely that the marketing of the product would generate sufficient return to justify the necessary investment

The legislative framework on orphan medicinal products aims to stimulate research and development of medicinal products for rare diseases by providing incentives to developers of such products laid down in the orphan legislation Regulation (EC) No 141/2000

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Orphan Drug Designation: criteria and incentives

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RARITY (prevalence)

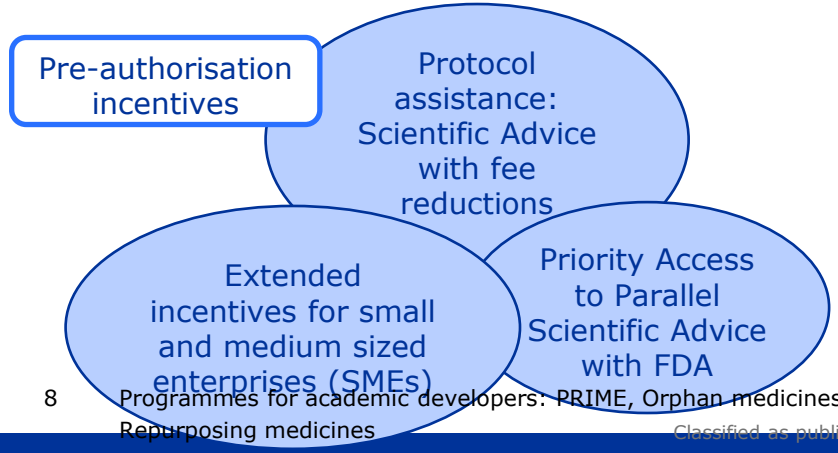
Medical condition affecting not more than 5 in 10,000 persons in the Community

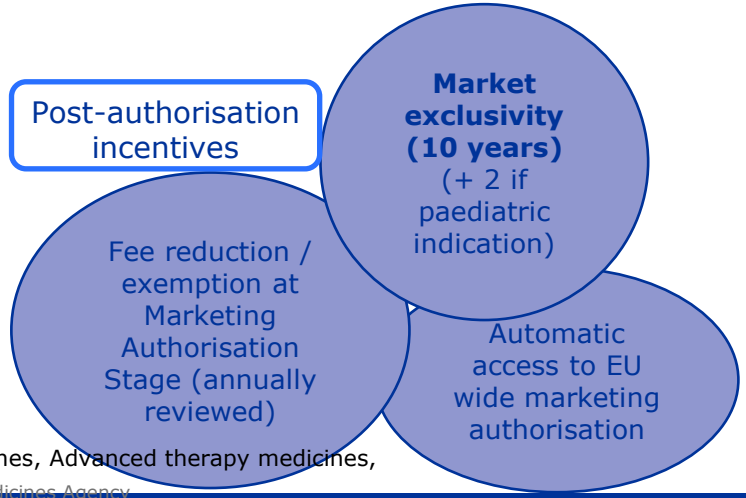
SERIOUSNESS

Life –threatening or chronically debilitating

ALTERNATIVE METHODS

No satisfactory method of diagnosis prevention or treatment of the condition authorised in the Community or, if such method exists, new medicine will be of significant benefit to those affected





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Key activities

Support the **COMP** for Orphan drug designation applications
Maintenance of orphan status at MAA

Protocol assistance (support the Scientific advice office on questions how to show significant benefit)

Engage in, and support activities to develop medicines for patients with an orphan disease (public-private partnerships, research funders, …)

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Medicines for the paediatric population

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Support for Paediatric medicines

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The Paediatric Regulation came into force in the European Union (EU) on 26 January 2007

A system of OBLIGATIONS and REWARDS which main objectives are:

Improve the health of children:

- Increase high quality, ethical **research** into medicines for children
- Increase **availability** of authorised medicines for children
- Increase **information** on medicines

Achieve the above:

- Without unnecessary studies in children
- Without delaying authorization for adults

https://www.ema.europa.eu/en/human-regulatory/overview/paediatric-medicines-overview

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Main activities

Scientific, regulatory and procedural management of procedures

- PIP (Pediatric Investigation Plan) and Modifications of agreed PIPs
- Waivers; Compliance check
- Contributing to generate paediatric information in centralised procedure

Working with stakeholders

- EnprEMA: network of research networks, with investigators and centres with expertise in clinical trials with children
- Engage in, and support activities to develop medicines for children (consortia, networks, funders, …)

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Repurposing authorised medicines

Repurposing authorised medicines

"The Commission supports initiatives to improve academic researchers and not-for-profit stakeholders' regulatory knowledge via scientific and regulatory advice so that the evidence they generate can be seamlessly used to repurpose off-patent medicines for new therapeutic uses. Industry engagement and partnership in this process will be promoted."

"Action: Launch a pilot project with engagement of industry and academia to test a framework for repurposing of off-patent medicines and inform possible regulatory action – 2021"

ONGOING

https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe_en

Repurposing authorised medicines: ongoing activities

Repurposing champions are provided with Scientific advice

https://www.ema.europa.eu/en/news/repurposing-authorised-medicines-pilot-support-not-profit-organisations-academia

Start 2022

RePo4EU
Precision drug REPurpOsing For EUrope and the world

https://repo4.eu/

HORIZON EUROPE

REPURPOSING OF MEDICINES 4ALL The European Platform for Medicine Repurposing

https://remedi4all.org/

Advanced therapy medicinal products

Advanced therapy medicinal products (ATMPs)

ATMPs – three main types:

- **gene therapy medicines**: contain recombinant nucleic acid used in or administered to human beings with a view to regulating, repairing, replacing, adding or deleting a genetic sequence genes that lead to a therapeutic, prophylactic or diagnostic effect.
- **somatic-cell therapy medicines**: contain cells or tissues that have been manipulated to change their biological characteristics, or cells or tissues not intended to be used for the same essential functions in the body. Used to cure, diagnose or prevent diseases.
- **tissue-engineered medicines**: contain cells or tissues that have been modified so they can be used to repair, regenerate or replace human tissue

https://www.ema.europa.eu/en/human-regulatory/research-development/advanced-therapies/support-advanced-therapy-developers

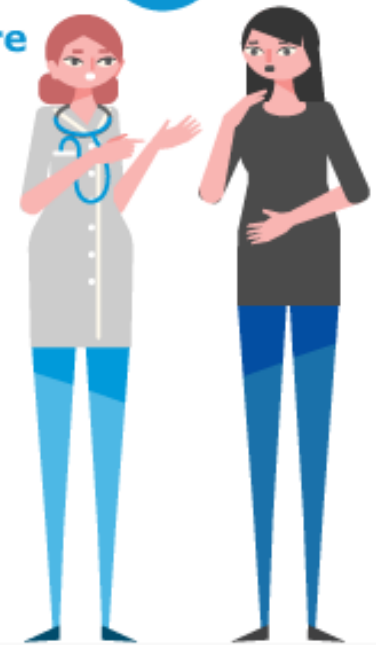
Extensive support and information available

Advice for patients considering treatment with a cell-based therapy

If you are offered cell-based therapy, find out from your healthcare professional if it has been authorised by medicines authorities

- ☑ Ask your healthcare professional to explain the risks and benefits of the cell-based therapy and provide information in writing
- ☑ Ask your healthcare professional how you should report side effects resulting from the treatment
- ☑ Contact your national medicines authority or EMA if you have any questions*
- ☑ If you are considering taking a treatment in a non-EU country, check the regulations in that country

*ema.europa.eu/partners-networks/eu-partners/eu-member-states



ATMP pilot for academia and non-profit organisations

Academic and **non-profit** organisations involved in developing promising advanced therapy medicines (ATMPs) can apply for **EMA's** increased **support** in **meeting regulatory requirements**.

Up to five ATMP developers targeting **unmet clinical needs** will receive dedicated assistance that includes:

- **Guidance** throughout the regulatory process, from manufacturing best practice to clinical development and follow-up planning on efficacy or safety issues
- **Fee reductions** and **waivers**

This EMA pilot aims to assess the level of regulatory support needed to boost the number of advanced therapy medicines that **reach patients** in the European Economic Area.

It was launched in September 2022. Initial results are expected after three to four years.

To apply for this EMA pilot, developers should fill in the **application form** below and send it to advancedtherapies@ema.europa.eu.

Guidance is also available to inform applicants on aspects such as:

- How and when to apply to EMA's pilot
- Selection criteria
- Benefits of taking part
- Expected milestones

https://www.ema.europa.eu/en/human-regulatory/overview/advanced-therapy-medicinal-products-overview#atmp-pilot-for-academia-and-non-profit-organisations-section

Support to medicine development and evaluation

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EMA teams working together for programmes to deliver for patients

Therapeutic Areas (TA)

- Vaccines and therapies for infectious diseases (TA-INF)
- Oncology and radiopharmaceuticals (H-TA-ONC)
- Neurological and psychiatric disorders (H-TA-NEU)
- Endocrine and cardiovascular diseases (H-TA-ECV)
- Immune and inflammatory diseases (H-TA-IMM)
- Advanced therapies and haematological diseases (H-TA-ATH)

Scientific Evidence Generation Dep. (H-EG)

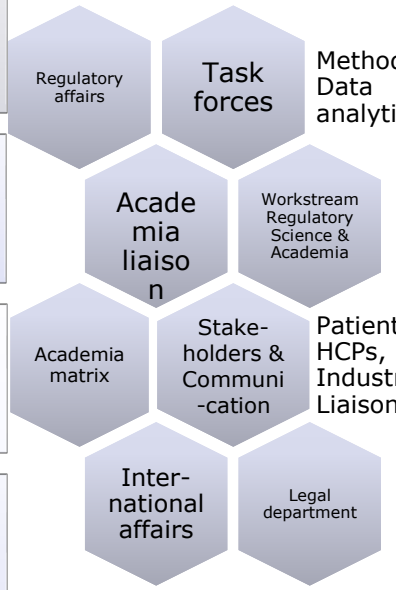
- Scientific advice (H-EG-SCA)
- Paediatric medicines (H-EG-PME)
- Orphan medicines (H-EG-OME)
- Translational sciences (H-EG-TRA)

Quality and Safety of Medicines Dep. (H-QS)

- Pharmaceutical Quality (H-QS-QUA)
- Inspections (H-QS-ISP)
- Pharmacovigilance (H-QS-PHV)
- Referrals (H-QS-REF)

Committees and Quality Assurance Dep. (H-QA)

- Meeting secretariat (H-QA-SEC)
- Procedures (H-QA-PRO)
- Labelling (H-QA-LAB)
- Regulatory affairs (H-QA-REG)
- Expert Panels and Groups (H-QA-EPG)



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Newly authorised medicines in 2022

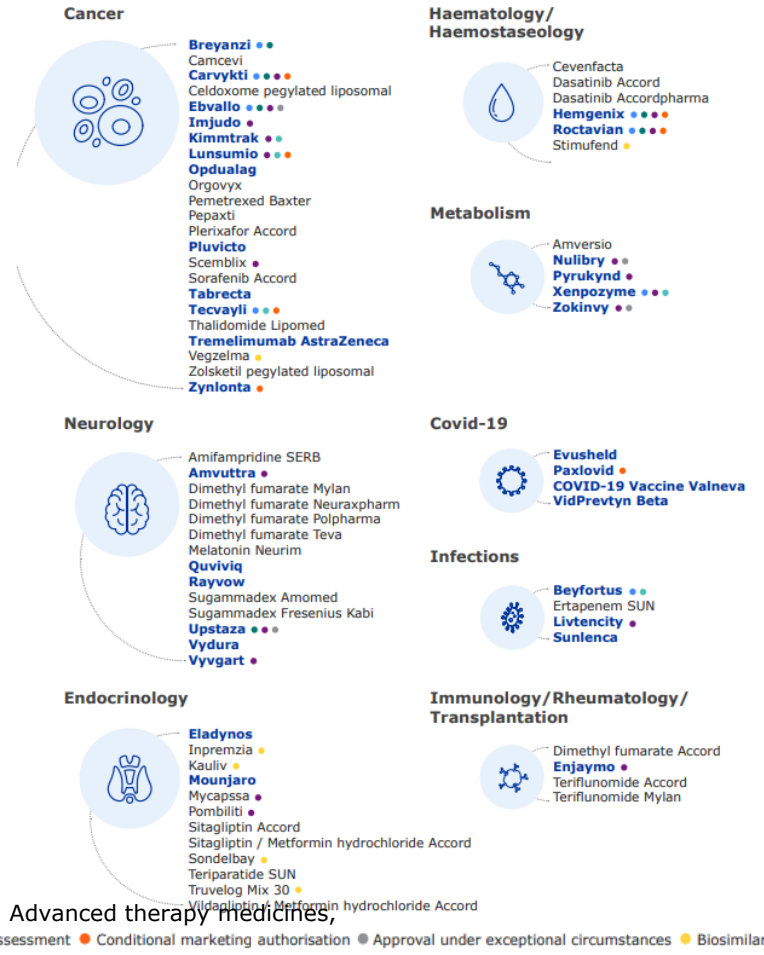
EMA recommended 89 medicines for marketing authorisation

Key figures[1] on the European Medicines Agency's (EMA) recommendations for the authorisation of new medicines in 2022:

89	41	3	16
Positive opinions	New active substances	Negative opinions	Withdrawn applications

PRIME	Advanced therapy medicinal products	Orphan medicines[2]	Accelerated assessments	Conditional marketing authorisations	Approvals under exceptional circumstances	Biosimilars
8	6	21	5	9	5	8

https://www.ema.europa.eu/en/documents/report/human-medicines-highlights-2022_en.pdf



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Thank you for your attention

Any questions?

Further information

Intranet Home Page - EMA intranet (europa.eu) (Human medicines)

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