EU scientific regulatory support mechanisms and initiatives for innovation in drug development: the EMA perspective

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Acknowledgments

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Agenda

1. European Medicines Agency: introduction
2. The EMA Innovation Task Force
3. The EMA SME office
4. Priority Medicines (PRIME) initiative
5. The European Innovation offices Network
Agenda

European Medicines Agency: introduction

The EMA Innovation Task Force

The EMA SME office

Priority Medicines (PRIME) initiative

The European Innovation offices Network
**EMA in the EU**

*Who do we work for?*

- **over 500 million people living in the European Union**
- **28 member states**
- **27% of global sales of medicines**
- **24 official languages**
What do we do?

- Facilitate development and access to medicines
- Evaluate applications for marketing authorisation
- Monitor the safety of medicines across their life cycle
- Provide information on human and veterinary medicines to healthcare professionals and patients

Protect human and animal health
Who we are

- ~4000 Scientific experts from right across Europe
- 7 Scientific committees
- CHMP
- CVMP
- COMP
- HMPC
- PDCO
- CAT
- PRAC
- 1995 EMA established to evaluate medicines for use in the EU
- 28 Working parties
- ~890 Staff members
- over 1000 marketing authorisations recommended
How are medicines approved?

- Centralised procedure, via EMA;
- National licence, Mutual recognition procedure, Decentralised procedure, via NCAs.

The European medicines regulatory network

~ 50 national regulatory authorities | European Commission | European Medicines Agency
How is EMA organised?

Management Board  Executive Director  EMA staff

+ 28 working parties
+ 8 scientific advisory groups

National competent authorities
~4000 European experts
The European medicines regulatory network

EMA coordinates the European medicines network comprising:

• around 50 national regulatory authorities;
• the European Commission;
• the European Parliament;
• other EU agencies;
• 3,500 experts.

How are medicines approved?

Different authorisation routes: one set of common rules

Centralised procedure (via EMA)   National procedures (via NCAs)
### Which medicines are approved through the centralised procedure?

- Human medicines for the treatment of HIV/AIDS, cancer, diabetes, neurodegenerative diseases, auto-immune, and other immune dysfunctions, and viral diseases
- Medicines derived from biotechnology processes, such as genetic engineering
- Advanced-therapy medicines, such as gene-therapy, somatic cell-therapy or tissue-engineered medicines
- Officially designated ‘orphan medicines’ (medicines used for rare human diseases)
Supporting research and innovation of medicines

**Pre-authorisation**
- Innovation task force (H&V)
- Paediatric investigation plan (PIP) (H)
- Scientific advice (H&V)
- Qualification of novel methodologies (H)
- Advanced therapy medicinal product classification (H)
- Regulatory and administrative assistance for small- and medium-sized enterprises (H&V)
- Orphan designation (including protocol assistance, fee reductions, market exclusivity) (H)

**Post-authorisation**
(When a medicine is available on the market)

**Marketing authorisation application evaluation**
Of 94 novel *authorised* medicinal products:
- Large majority marketed by large or intermediate sized companies.
- SMEs and academia at the origin of innovation.
Advancing public health

Innovation in healthcare brings new opportunities to treat certain diseases and is essential to advancing public health. Noteworthy therapeutic innovations in 2015 included:

**Cancer**
- **Blincyto** - directing the immune system towards cancer cells
- **Farydak** - regulating the activity of genes
- **Imlygic** - using genetically engineered virus to kill cancer cells
- **Opdivo, Nivolumab BMS** and **Keytruda** - increasing the capacity of the immune system

**Cardiovascular**
- **Entresto** - dual action to treat heart failure
- **Repatha and Praluent** - monoclonal antibodies to treat hypercholesterolemia

**Haematology**
- **Praxbind** - targeted neutralisation of the anticoagulant effect of Pradaxa

**Neurology**
- **Wakix** - action on histamine H3 receptors to treat narcolepsy
EU Medicines Agencies Network Strategy to 2020

Includes objectives to:

- Support for patient focused innovation and contribute to a vibrant life science sector in Europe
- Greater collaboration across network to support innovation
- Consider further regulatory incentives for innovation, particularly in certain areas of public health need
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Multidisciplinary platform for preparatory dialogue and orientation on innovative methods, technologies and medicines

- Early dialogue on scientific, legal and regulatory issues
- Address the impact of emerging therapies and technologies on current regulatory system
- Identify the need for specialised expertise at an early stage
- Provide advice on the eligibility to Agency procedures relating to research and development, e.g. borderline products
- Increase awareness and learning in emerging therapies and technologies at the Agency
• Informal exchange of information and provision of guidance early in the development process.

• Discussions led by experts from the Agency's network, working parties and committees

• Complement and reinforce existing formal regulatory procedures, such as ATMP classification and certification, designation of orphan medicinal products and scientific advice.
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The EMA SME Office

SME Office launch
December 2005.

A single interface
"One-stop-shop."

Assistance to SMEs
Regulatory, administrative and procedural support.

Facilitates communication
Of SMEs in veterinary and human pharma sector.

Coordinating & networking
Working closely with EU.

A strategic regulatory toolbox to promote innovation and development of new medicines by SMEs.
1. Validation of SMEs status
2. Regulatory Assistance
3. Fee Incentives
4. Translation Assistance
5. Training and Awareness
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PRIME scheme - Goal & Scope

To foster the development of medicines with major public health interest.

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
- Facilitated by knowledge gained throughout development
- Feedback of relevant SA aspects to CHMP

Building on existing framework;
Eligibility according to existing ‘Accelerated Assessment criteria’
Features of the PRIME scheme

Early access tool, supporting patient access to innovative medicines.

- **Written confirmation of PRIME eligibility** and potential for accelerated assessment;
- **Early CHMP Rapporteur appointment** during development;
- **Kick off meeting** with multidisciplinary expertise from EU network;
- **Enhanced scientific advice** at key development milestones/decision points;
- **EMA dedicated contact point**;
- **Fee incentives** for SMEs and academics on Scientific Advice requests.
Overview of PRIME scheme

- Early identification of therapeutic innovation in unmet medical needs.
- Iterative Scientific advice
- Enhanced regulatory guidance
- Incremental knowledge gain
- Proactive dialogue
- Promote use of existing tools
- MAA review under accelerated assessment.

Nonclinical → Phase I → Exploratory → Confirmatory → Evaluation → Post-authorisation

- Eligibility (CHMP)
- SA 1 (SAWP)
- SA 2 (SAWP)
- SA n (SAWP)
- Accelerated Assessment confirmation (CHMP)
- Early CHMP Rapporteur appointment

SMEs
Academia
Any sponsor
Justification for eligibility to PRIME

For products under development yet to be placed on the EU market

Unmet medical need

- Epidemiological data about the disease
- Description of available diagnostic, prevention and treatment options/standard of care (SOC), their effect and how medical need is not fulfilled

Potential to significantly address the unmet medical need

- Description of observed and predicted effects, clinical relevance, added value and impact
- If applicable, expected improvement over existing treatments

Data required at different stages of development
Entry points PRIME eligibility and required evidence

**Proof of principle**
(For SMEs and academia only)
- 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)
- Substantial improvement
- Magnitude, duration, relevance of outcomes to be judged on a case by case basis
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EU innovation network (EU-IN)

- EMA ITF + 12 National innovation offices/contact points
- Regular teleconferences
- Aims to share information related to innovative drug development and initiate early dialogue with developers
Everybody wants to help Small and Medium-sized Enterprises (SMEs) at FAMHP workshop, 2 May 2016.

Supporting Innovation in the EU NCAs

MHRA Innovation Office

MHRA Innovation Office helps organisations in the field of medical devices or using novel manufacturing processes to progress their products or technologies.

Contents

- Role of the group
- Roles and services
- Core profile of an innovation office
- The core profile of an innovation office

Role of the group

The Innovation Office’s main role is to support the MHRA in its work under the Innovative Medicines Initiative (IMI). The Innovation Office also supports the MHRA in its work under the EU Framework Programme for Research and Innovation (FP).

Roles and services

The Innovation Office offers a range of services to support the MHRA in its work.

Core profile of an innovation office

The core profile of an innovation office includes:

- Target group: Hospitals units, Start-ups, SMEs
- Target products: Chemical medicines, Biological medicines + vaccines, ATMPs, Medical devices
- Focus: Quality / viral safety, Preclinical, Clinical, Regulatory
- Services: A f2f meeting; Simple advice, referral to scientific advice or other competence
- Other information: website, interaction with other stakeholders of innovation
The typical long route of medicines to patients

Chance of reaching access for a product entering the development phase:

- 0.01-0.1% Pharmaceutical + nonclinical (4 – 6 y)
- 5-10% Phase I and II (2 – 4 y)
- 50-60% Confirmatory phase III (2 - 5 y)
- 75-90% Assessment and approval (1 – 2 y)

Access

National innovation office

PRIME

EMA/CHMP scientific advice

ITF

National Scientific Advice

FAMHP workshop, 2 May 2016
For an EU seamless support to Innovation

**ITF, SMEs, ATMPs CAT support, OMP des., re-direct to NCAs products**

**Research Methods**
- Pilot GMP, GLP Non-clinical
- GCP - FIH

**Qualification Phase II/III MAA**

**EU COMMITTEES**
- Scientific Advice, MAA, MAPP (PRIME des.)
- Post-approval

**NCAs local support**
- **EU-IN**, National Scientific Advice, Clinical trials authorization, Compliance

**National provisions for early access**
National Innovation Office: awareness, requirements, issues identification, funding opportunities

EMA ITF: NCA support/experts briefing meetings, H2020/IMI relevant initiatives, SMEs incentives/information/visibility

EU-IN: Innovation scan, issues, precedents, support

Innovation Office Preparatory work:
- Regulatory framework/requirements
- HTA and scientific advice
- Clinical trials / EMA CT Portal
- GMP/GLP/GCP rules/authorisations
- CAT Classification, OMP designation

Pilot GMP
GLP Non-clinical

GCP – FIH

Phase II:
- EU Scientific Advice + HTA
- PRIME, MAPP, EARLY EU and National Access opportunities, Post-authorisation requirements
Collaboration across EU network to support innovation
Thank you for your attention

Further information

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