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SCIENCE MEDICINES HEALTH

Upstream convergence and downstream divergence

Perspective of the regulator

Regulatory Science Network Netherlands Annual Workshop 2021

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UPSTREAM REGULATORY CONVERGENCE

Coordinated and structured regulatory framework established at the European level

MA

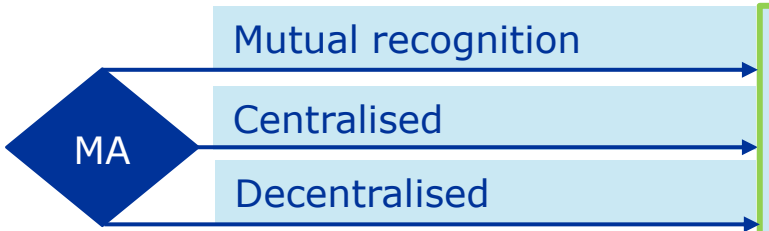
DOWNSTREAM DIVERGENCE

Individual Member States with their own interpretations

Impact on accessibility to patients of medicines & treatments

Are differences inevitable

Are differences undesirable



- Positive Benefit Risk Decision
- Marketing Authorisation
- Common Summary Product Characteristics
- Package Leaflet
- Labelling
- Public Assessment Report
- Coordinated oversight product in clinical use

~All new active substances approved centrally

Established basis for follow-on products, generics & biosimilars

Blue box requirements

- Pricing
- Reimbursement
- Legal Status Supply
- Authentication

Upstream Regulatory Convergence

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Development Phase

Quality / Safety / Efficacy
Evidence generation

European/International guidelines

Scientific Advice		Clinical Trials	
National	Central	National	CT Reg.
+HTA	+HTA		
+Payers	+Payers		

Horizon Scanning
Unmet medical need
PRIME Scheme
Accelerated Assessment

MA

Early Access Tools
- CT's
- Compassionate use
- Managed entry agreements

Downstream Divergence

Individual Member States with their own interpretations

EMA = European Medicine**s** Agency

- Does not govern practice of medicine within healthcare systems
- No obligation on MS to include CAPs in national health systems

HTA + Pricing & Reimbursement Decisions (national, regional)

Company launch plans – wealthier / larger countries come first

Clinical treatment guidelines (national, regional)

- Vaccination strategy special case

"To underpin its mission of protecting human health, EMA must catalyse and enable regulatory science and innovation to be translated into patient access to medicines in evolving healthcare systems."

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Regulatory Science Strategy to 2025 (RSS)

- Invest in PRIME scheme
- Integrate Scientific Advice
- Reinforce patient relevant evidence generation
- Foster innovation in clinical trials
- Promote use of RWE in decision-making
- Contribute to HTA preparedness and downstream decision-making
- Collaborate with payers

European Medicines Agencies Network Strategy to 2025– Access & Availability (EMAN)

- Optimise the path from development, evaluation through to access for innovative and beneficial medicines through collaboration between medicines regulators and other decision makers in the areas of:
  - Evidence planning, including post-licensing evidence
  - Engagement in review of evidence and methodologies, respecting remits of the various players
  - Collaboration on horizon scanning
- As a result of this work, medicines that address unmet medical needs should have broader and earlier access coverage.

EC Pharmaceutical Strategy for Europe

- Delivering for patients: fulfilling unmet medical needs and ensuring accessibility and affordability of medicines
  - Prioritising unmet medical needs
  - Ensuring patients' access to medicines
  - Ensuring affordability of medicines for patients and health systems' financial and fiscal sustainability

Prioritising unmet medical needs - antimicrobial resistance

Pilot innovative approaches to EU R&D and public procurement for novel antimicrobials and their alternatives aiming to provide pull incentives

Promote investment and coordinate research, development, manufacturing, deployment and use for novel antibiotics as part of the new HERA

Consider in the review of the pharmaceutical legislation to introduce measures to restrict and optimise the use of antimicrobial medicines. Explore new types of incentives for innovative antimicrobials

Prioritising unmet medical needs

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- Revise the legislation on medicines for children and rare diseases to improve the therapeutic landscape (e.g. in paediatric cancer) through more tailored incentives
- Facilitate collaboration on unmet needs and evidence generation in joint meetings of existing committees/networks of regulators, health technology assessment (HTA) bodies and payers, involving key actors in the development, authorisation and access to medicines for a lifecycle approach and improved availability and affordability
- Work with the European Parliament and the Council towards the adoption of the Regulation on health technology assessment
- Incorporate the European Medicines Agency (EMA) priority medicines scheme (PRIME) in the regulatory framework to provide enhanced support so as to accelerate product development and authorisation in areas of unmet needs
- Enable parallel scientific advice on clinical study design for medicines by HTA bodies and the EMA, as provided for by the proposed HTA Regulation

Ensuring patients' access to medicines

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Propose to revise the system of incentives and obligations in the pharmaceutical legislation taking into account the relationship with intellectual property rights, to support innovation, access and the affordability of medicines across the EU

Review the pharmaceutical legislation to address market competition considerations and thus improve access to generic and biosimilar medicines, including interchangeability and the 'Bolar' exemption

Initiate a pilot together with the EMA and Member States, with the engagement of future marketing authorisation holders, to understand the root causes of deferred market launches

Encourage buyers from the health sector to cooperate in view of implementing innovative procurement approaches for the purchases of medicine or medical devices, in the framework of the Big Buyers initiative

Ensuring affordability of medicines for patients and health systems' financial and fiscal sustainability

Revise the pharmaceutical legislation addressing aspects that impede the competitive functioning of the markets and to take account of market effects impacting on affordability

Develop cooperation in a group of competent authorities, based on mutual learning and best-practice exchange on pricing, payment and procurement policies, to improve the affordability and cost-effectiveness of medicines and health system's sustainability, including on cancer treatment

Engage with Members States in implementing non-legislative measures to improve transparency, such as guidelines on principles and costing methods for establishing the R&D costs of medicines

Final reflection

However beautiful the strategy you should occasionally look at the results ……

Any questions?

Further information

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