How Regulators Can Promote Innovation

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(1) Some background  
(2) Mapping of activities  
(3) The EU-Innovation Network  
(4) The STARS project  
(5) STAMP’s repurposing pilot project  
(6) Some conclusions
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Why do medicine development programs fail (or get delayed)?

• When further development proves initial hypotheses wrong (often inevitable)
• Inappropriate development program, wrong studies (usually preventable)

Who takes the risk? Companies, investors, and patients engaged in clinical trials

From Guido Rasi
EMA’s Executive Director
Many EC funded projects focus either on the development of novel or on the optimisation of already registered medicinal products (e.g. novel indications).

However, in a majority of cases, the clinical results produced by researchers in academia do not fulfil regulatory requirements to achieve full impact/reach the patients.

Mainly due to insufficient time and know-how about Regulatory Sciences to develop strategies for successful Scientific Advice and Protocol Assistance procedures.
Where do new medicines originate from in the EU?


Figure 1 | Origin of new medicines in the European Union (2010–2012). a | Originator and the marketing authorization holder for all 94 approved products evaluated, divided according to organization type. b | Direction of product transfers between organization types during development; the size of the lozenges is representative of the proportion of transfers. PPP, public–private partnership; SME, small or medium-sized enterprise. For details of the data and analysis, see Supplementary information S1 (box).
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Idea

Marketing authorisation

Market access
Clinical Trials

Early access programmes

Marketing authorisation

Market access

Off-label Use

Idea
Early access programmes

EU Innovation Network

NCA Offices

ITF

Coordination and Support Action

Clinical Trials

Marketing authorisation

Market access

Off-label Use

Idea

Horizon scanning
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EU-Innovation Network

- 24 Innovation Offices (23 NCAs plus the European Medicines Agency) from 21 Member states
- Co-chaired by the EMA and FIMEA
- Adoption of the mandate in September 2016
- Mission: Facilitate the development of innovative medicines by addressing gaps in early regulatory support to innovation
Mandate

• Make the support more visible and attractive to innovators
• Provide a platform for regulators to share and improve the knowledge flow
• Reinforce dialogue with innovators with a wider EU exposure of identified issues
• Encourage sponsors of promising drug development projects to move into the next appropriate regulatory level
• Actively contribute to and integrate into relevant EU initiatives enabling innovative medicines development and access to patients
(a) Horizontal coordination

EU-IN level

Consortium

NCAs level

(b) Vertical coordination

National groups

Individual institutions
Specific tasks

- Horizon-scanning, case studies for discussion, also candidates for PRIME and centralised scientific advice
- Preparedness for innovation in terms of expertise and regulatory tools
- Collective and harmonised guidance to ease the transition through the regulatory process
- Simultaneous National Scientific Advice
- Attempt for reducing time to MA
CRISPR-Cas9 genome editing

Nano-medicines

Additive manufacturing & 3D Printing

Cell Therapy and other ATMPs

Chimeric antigen receptor (CAR)

Tumor cell

Antigen expressed on the cell surface

Big Data

Microbioma
Dissemination

Agenda—EU-Innovation Network workshop with academia
16 November 2018, 09:00-16:00, meeting room: 2A

EU Innovation Network – For SMEs with ideas on medicines

Welcome to the EU Innovation Network // EU-IN

Supporting innovative medicines’ development and early access
SME info day // 17 November 2017 // EMA // London
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STARS, a spin-off of the EU-IN

Strengthening training of academia in regulatory sciences and supporting regulatory scientific advice
Aims & Objectives

• to improve the direct regulatory impact of results obtained in (early) medical research.

• to reach academic researchers very early in the planning of relevant grant applications

• to strengthen long-term regulatory knowledge in general by reaching clinical scientists during professional training and qualification.
Improving regulatory knowledge, success and direct regulatory impact of academic health research
Regulatory Research

Regulatory Framework

Publicly funded Clinical Research

Academia

Qualification of new methods

Competent authorities
EMA, HMA, HTA

Scientific advice

Regulatory science
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EU ‘Repurposing’ Project Plans Pilot Phase

Executive Summary

Work is underway on a new European procedure. It would mainly be used for active substance authorization holder and the relevant data were g

Cross purposes

“Repurposing” off-patent drugs offers big hopes of new treatments

Repurpose more suited for a different purpose, or convert for us
STAMP: Repurposing working group – whitepaper sections

• Introduction
• Scope
• Key-features of repurposing framework
• Core components
• Regulatory engagement
• Industry engagement
• Incentives – disincentives
• Outline of key components proposed in the framework
• Repurposing schematic
• Summary conclusion/next steps

• Proposal for a pilot
• Proposal for a monitoring board during the pilot
• Annex 1: Useful resources, contacts and information on incentives in the EU

March 2019
• Aim to provide a visible supportive framework to not-for-profit stakeholders who have the data and scientific rationale for a new indication, and who have the aim to see this new use on-label

  – Promotes a process for facilitating data generation in accordance with regulatory standards, described as voluntary steps within the existing regulatory framework

  – Elements of the framework cover only one possible scenario, some key milestones are not regulatory activities

  – Applicable to both EMA and NCA activities, and driven by ‘Champions’

  – A Champion is not a pharmaceutical company, is able to coordinate, transparent, files initial request for scientific advice, provides information to MAH

  – Core components: new indication in areas of public health benefit / Union interests, valid MA exists which is out of protection periods
Repurposing of MP’s out of patent & data protection

6. Regulatory assessment

Approved indication

A champion is not a pharmaceutical company

1. Champion cross checks against the scope criteria

2. Using identified data sources and/or own data, the Champion submits the proposal to enter the pathway to EMA or NCA for a repurposing regulatory scientific advice meeting using the relevant template.

3 A. Regulatory authority gives SA upon request from Champion and as applicable with other relevant stakeholders (NAHs, patient groups, HTA, other). Discussion on the proposals.

3 B. Regulators provide feedback, signposts to relevant information about regulatory routes, Article 57 database etc.

4. The Champion may share SA feedback. The development programme can be taken forward with or without the support of a specific MAH at this stage. The Champion should confirm compliance to Advice when pairing up with MAH.

5. MAH (s) takes forward the data package, constructs a regulatory dossier and submits a variation/extension/marketing authorization application to EMA or relevant NCA (s).

Champion assembles supporting data

Champion proposes new indication

Regulatory guidance by web TC meeting

Champion assembles advised data package

MAH interaction

6. Regulatory assessment
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• New products, technologies, and production models have challenged existing regulatory frameworks at anytime

• Increasing pressure for a safe and timely access to innovative health products has prompted regulators to review their policies and regulatory tools in order to meet this social demand while not lowering bars for approval

• The EU-IN, the STARS project and the STAMP’s repurposing pilot project are examples of tools to promote innovation in the EU
Thank you for your attention

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