Repurposing of medicines
the hidden champion of sustainable innovation

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Repurposing/repositioning of medicines

- new therapeutic indication for an already known active substance
- new administration route with the same indication
- new combinations of medicines previously used as separate products for treatments
- new medicine/medical device combinations
Incentives

• new indication for a well-established substance - additional 1 year data exclusivity

• orphan designation - incentives for development of medicines and a 10 year market exclusivity for authorised medicines

• For paediatric studies – supplementary protection certificate extension; data or market protection; plus 2 years market exclusivity for orphans
Challenges

- Development – evidence generation
- Off-label use
- Inclusion of new indications for authorised medicines
- Responsibilities
Safe and Timely Access to Medicines for Patients (STAMP) -

- 2015 – 2019 - repurposing of medicines discussion
- March 2017 - multi-stakeholder meeting
- Sub-group formed – proposal for a framework to support not-for-profit organisations for repurposing
- July 2019 Pharmaceutical Committee endorse pilot project to test framework
- Repurposing Observatory Group – pilot project
Pilot project for repurposing framework for not-for-profit organisations

Repurposing of MP’s out of patent & data protection

6. Regulatory assessment

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

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.

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

2. Champion assembles supporting data

1. Champion cross checks against the scope criteria

0. Champion proposes new indication

A champion is not a pharmaceutical company

Regulatory guidance by web, TC, meeting

MAH interaction

Approved indication

3. Champion assembles advised data package
STARS

Strengthening Training of Academia in Regulatory Science (CSA STARS)

• Comprehensive Inventory of existing support activities
• Common_Strategy to strengthen regulatory sciences
• Core Curriculum and the Comprehensive Curriculum
• 3 pilot projects

www.csa-stars.eu
An in silico-based approach to improve the efficacy and precision of drug REPurpOsing TRIALs

- Screen candidate drugs in mechanistically related disease phenotypes
- Focus on patients with cerebro-cardiovascular indications.
- 10 partners from 4 countries
- € 5.5 million EU funding over 5 years
Saracatinib trial to prevent FOP

- IMI pilot programme in 2017 – **Clinical Compound Bank for Repurposing**
- Repurposing stalled compounds in alternative indications
- Fibrodysplasia ossificans progressive (FOP) is a rare disease in which the muscles and connective tissues (e.g. tendons and ligaments) slowly turn into bone
- Saracatinib (AZD0530) has already been tested for safety in humans in healthy volunteers. Now it will be tested in 16 adults with FOP
- Astra Zeneca and 5 academic partners + patients, FOP experts and regulatory authorities
Exscalate4Cov, now counts **33 partners** and leverages more than 280 Petaflops of computing power delivered by the **five supercomputers** part of its network.

The aim is to **identify molecules** capable of **targeting the coronavirus** and develop a tool effective for countering future pandemics to be consolidated over time.

**By now 6000 molecules have been tested from a huge library of drugs and molecules. Now, 40 of them are in the evaluation phase for preclinical trials.**

#UnitedAgainstCoronavirus #EuropeDay
The European Institute of Innovation & Technology (EIT) is not part of the Specific Programme
Horizon Europe – Health cluster priorities

- Health throughout the Life Course
- Environmental and Social Health Determinants
- Non-communicable and Rare Diseases
- Infectious diseases
- Tools, Technologies and Digital Solutions for Health and Care
- Health Care Systems
Horizon Europe – Work programme 2021 – 2022

• HORIZON-HLTH-2021-DISEASE-04-02: Building a European innovation platform for the repurposing of medicinal products

Main challenges:

- Inadequate patient access to new medicines; unsustainable pricing policies
- Lessons learnt from COVID-19 re. shortages
- Lack of need-driven innovation especially in areas of unmet need
- Quality of medicines and active ingredients, green pharmaceuticals and reap the benefits of digitalisation
- (Vaccines) need for swift reaction / emerging pathogens / safety and efficacy

Proposed solutions:

- **Repurposing** existing medicines
- Support actions on **shortages**
- Boost the development of novel antimicrobials and address **AMR**.
- **Support innovative clinical trials** to speed up development (design and monitoring safety),
- Foster **international convergence** (inspections, ICH, Eur.Pharm) eProduct information
- Foster the development of **environmentally-friendly production and disposal** of medicines;
- Support actions to enhance affordability, (e.g best practice exchanges on pricing, reimbursement)
- (Vaccines) Strengthening post-marketing surveillance, tackling vaccine misinformation
Europe’s Beating Cancer Plan

- **EU platform to improve access to cancer medicines** to support the repurposing of existing medicines

- **EU4Health**: project to use high performance computing for testing of existing molecules and combinations

Timeline: start in 2021 - 2025
Flagships of the pharmaceutical strategy

- Ensure access and affordability of medicines for patients and health systems sustainability
- Enabling sustainable innovation
- Ensuring availability and addressing shortages
- Succeeding on the global level
Flagships of the pharmaceutical strategy

Ensure access and affordability of medicines for patients and health systems sustainability

Unmet needs
- Boost novel antibiotics
- Restrict and optimise the use of antimicrobial medicines (2021)
- Support medicines for children and rare diseases (2022)
- Collaboration on unmet needs evidence generation, Health Technology Assessment (2021)

Accessibility
- Revise the system of incentives and obligations in legislation to support innovation, access and the affordability of medicines (2022)
- Improve access to generic and biosimilar medicines (2022)

Affordability
- Address in legislation the market effects impacting on affordability (2022)
- Develop mutual learning and best-practice exchange on pricing, payment and procurement policies (2021-2024)
Enabling sustainable innovation

Fertile environment
- Optimise the supplementary protection certificates system (2022)
- Legislative proposal on European Health Data Space (2021)
- Interoperable data access infrastructure to facilitate secure cross-border analysis of health data (2021-2025)
- Support public-private and public-public partnerships (2021)

Innovation and digital transformation
- Adapt legislation to cutting-edge products, scientific developments and transformations (2022)
- Enhance dialogue among regulatory and other relevant authorities (2021)
- Take forward the use of high performance computing and artificial intelligence (2021-2022)
- Establish the secure federated access to 10 million genomes (2025)

Flexible regulatory system
- Simplification and streamlining of approval procedures and flexibility for timely adaptation (2022)
- Optimise the lifecycle management of medicines more efficient and adapted to digitalisation (2021-2023)
Pharmaceutical strategy - considerations

- High performance computing and artificial intelligence
- PRIME eligibility
- Specific regulatory pathway
- Possibility for non-commercial organisations to apply for marketing authorisation
- Incentives
More information

Safe and Timely Access to Medicines for Patients (STAMP):

- Link to proposal for a framework for repurposing:

Horizon Europe:
http://ec.europa.eu/horizon-europe

EU4Health:
EU4Health 2021-2027 – a vision for a healthier European Union | Public Health (europa.eu)

Pharmaceutical strategy:
https://ec.europa.eu/health/human-use/strategy_en