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SCIENCE MEDICINES HEALTH

Where is science, technology and medicines regulation heading to?

Where we are today

10th anniversary of European Medicines Agency (EMA) Healthcare Professionals' (HCPWP) Working Party meeting

Presented by Tony Humphreys on 27 June 2023
Head of Regulatory Science and Innovation Task Force

Innovation is in the DNA of EMA

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*"It is necessary to establish a centralised community authorisation procedure for **technologically advanced medicinal products** in particular those derived from **EU biotech industry**"**

* EC2309/93

Systematic extension of stimulus to innovation

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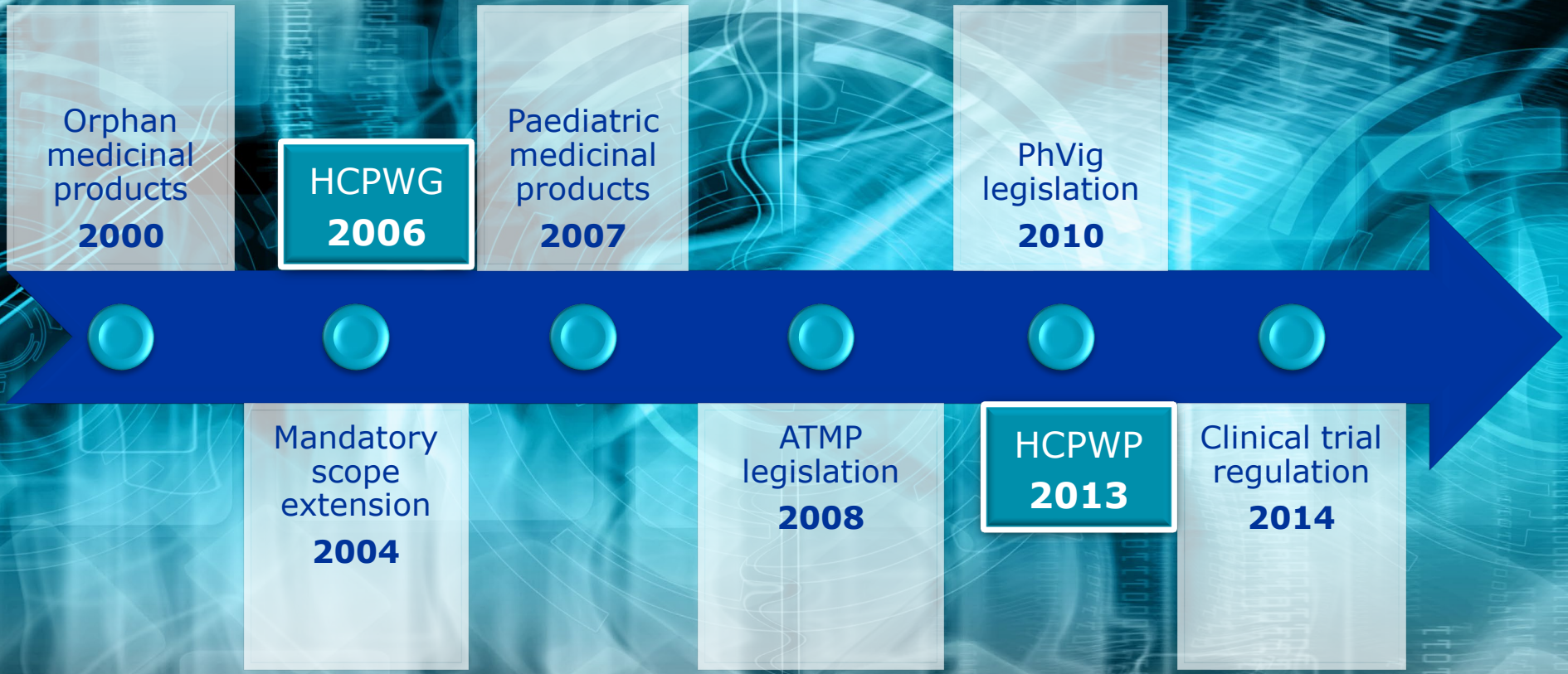

Human medicines – Medicines recommended for approval

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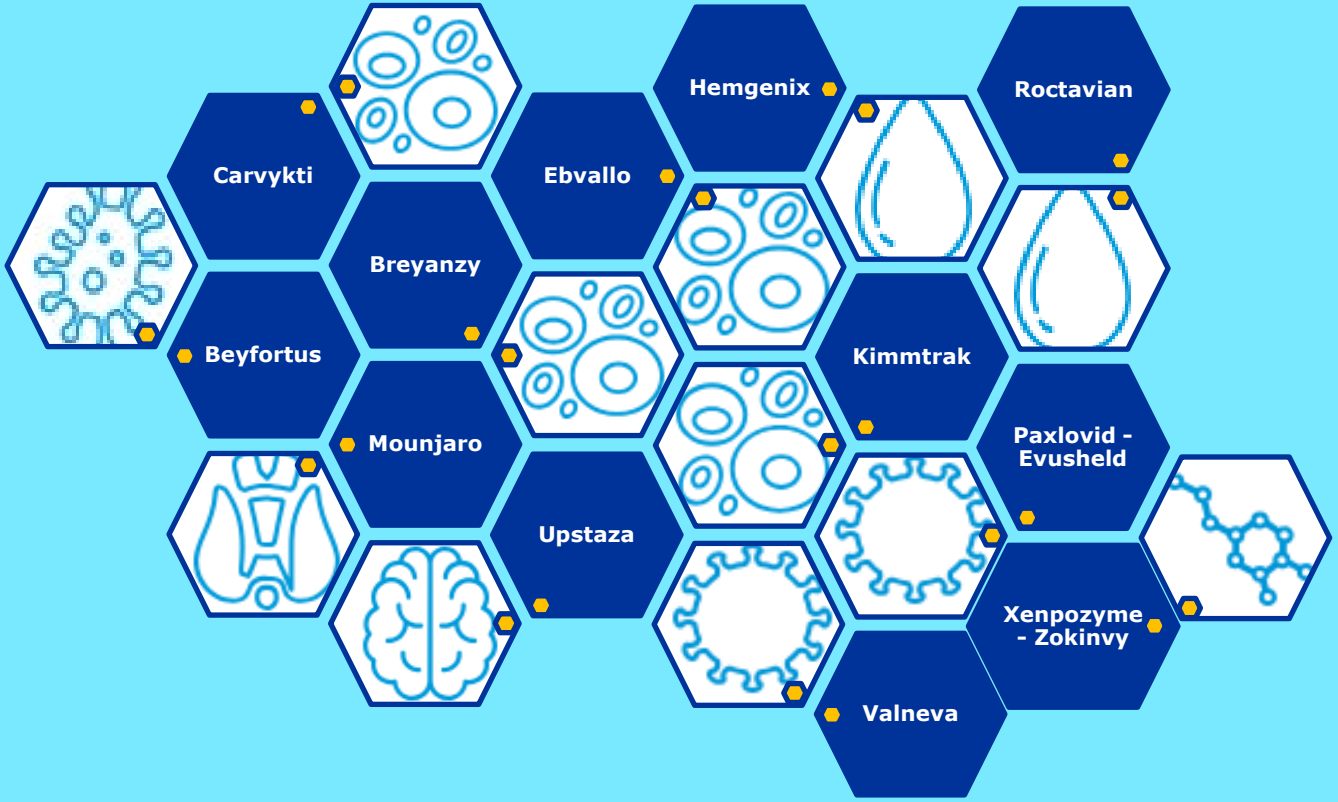
Authorisation of new medicines is essential to advancing public health as they bring new opportunities to treat certain diseases. In 2022, EMA recommended 89 medicines for marketing authorisation, 41 of which had a new active substance. *Here is a selection of medicines approved in 2022 that represent significant progress in their therapeutic areas:*



Early development support

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PRIME – PRIORITY MEDICINES

The enhanced development support aims at helping patients to benefit as early as possible from promising medicines that target an unmet medical need, by optimising the generation of robust data and enabling accelerated assessment.

This year, **eight medicines** with PRIME designation were recommended for approval.

78% of applicants granted a positive opinion for New Active Substance (NAS) medicines received scientific advice or protocol assistance during their product's development phase.

Early engagement with developers allows EMA to clarify what kind of evidence is required to later evaluate a medicine for authorisation.

This encourages the generation of more robust data for regulatory assessment, and thus protects patients from taking part in unnecessary or poorly designed clinical trials.

Digital health innovations

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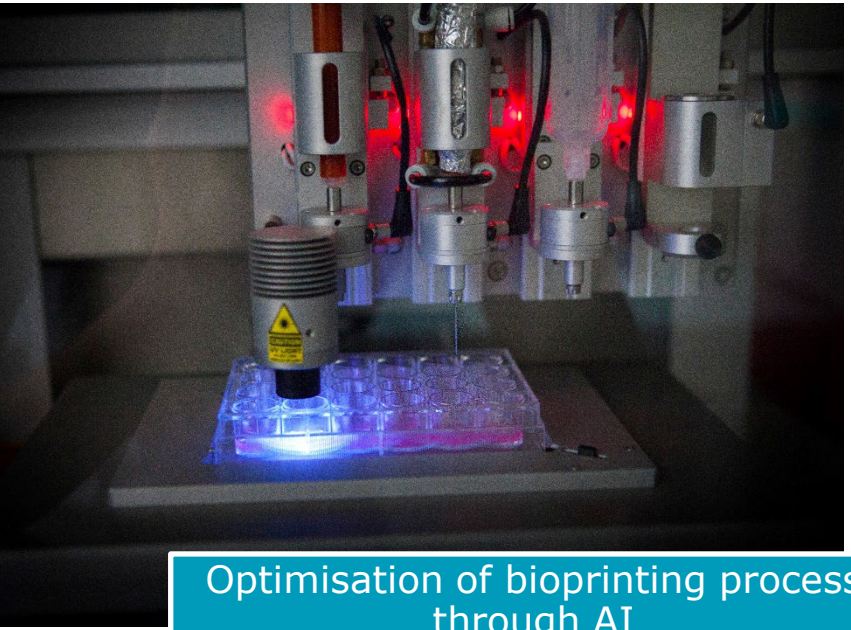
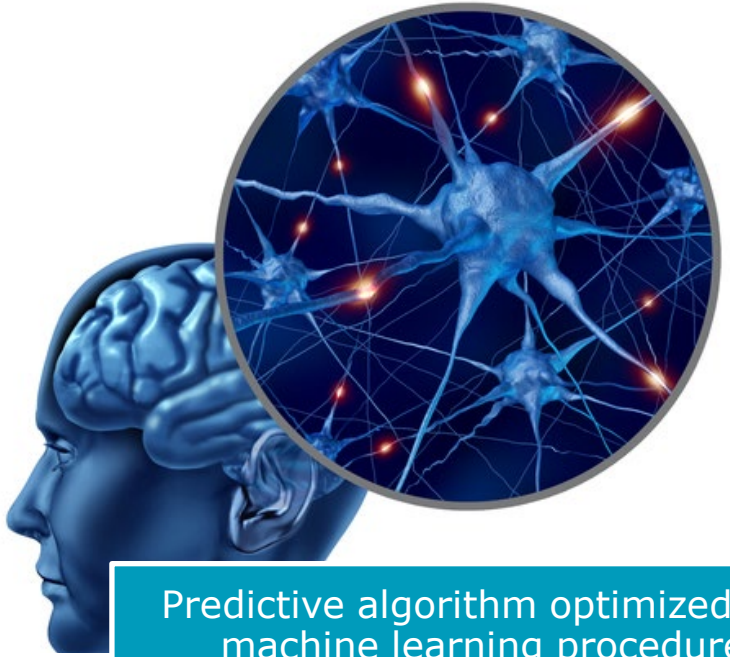
Cellularised bioimplants generated by 3D bioprinting



Optimisation of bioprinting process through AI

Biomarkers for early Alzheimer Disease identification



Predictive algorithm optimized with machine learning procedures

A unique opportunity for an aligned strategic response

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It is vital that R&D of innovative pharmaceuticals continues to be fostered in Europe

Evolving the ecosystem for such innovative developments is a collaborative activity

The critical success factor is a synergistic interplay between academia, developers, regulators and **patients & healthcare professionals**

Our common objective is to enable **patients and healthcare professionals** to have timely access to innovative medicines/treatment

The Covid-19 has been a "super catalyst" for change in the regulatory landscape

EU strategies on fostering an innovative development ecosystem

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Reform of the EU's Pharma Legislation

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Stella Kyriakides
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Today we present once in a generation proposals to ensure medicines are accessible, available and affordable at all times for 🇪🇺 citizens no matter where they live. A Single Market for medicines delivering for citizens and keeps our industry as a 🌎 leader. #EUPharmaStrategy
pic.twitter.com/u5l5xRTtmT

Margaritis Schinas
@MargSchinas

#EUPharmaStrategy is a Single Market for Medicines. Triple A: Access, Affordability, Availability. Triple C: Competitiveness, Compliance, Combatting antimicrobial resistance. An EU #HealthUnion with no 1st and 2nd class citizens, no MS left behind. ec.europa.eu/commission/pre…
pic.twitter.com/BJkV7yqPk3

Development support

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Scientific Advice extended to involve other bodies e.g., Medical Devices authorities/expert panels, HTAs, payers, Substance of Human Origin(SoHO), Clinical Trials Coordination Group (CTCG)

PRIME codified in legislation as "enhanced scientific and regulatory support for priority medicines"

Phased review of complete data packages: only for products that offer an exceptional therapeutic advancement in areas of (High) Unmet Medical Needs

Regulatory sandbox environment to test adapted, waived or deferred requirements for products that provide major advantage to patients

Unmet Medical Need (UMN) and High UMN

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New criteria-based definitions in legislation for UMN and high UMN to have a common understanding of the concept, which drives the granting of incentives/AA/PRIME etc.:

UMN = at least one of its therapeutic indications relates to a life threatening or severely debilitating disease and:

(a) there is no medicinal product authorised, or… despite medicinal products being authorized…, the disease is associated with a remaining high morbidity or mortality;

(b) a meaningful reduction in disease morbidity or mortality for the relevant patient population.

hUMN = **orphan** medicinal product where:

(a) there is no medicinal product authorised … or where, despite medicinal products being authorised …, … the orphan medicinal product, in addition to having a significant benefit, will bring **exceptional therapeutic advancement**;

(b) …a meaningful reduction in disease morbidity or mortality for the relevant patient population.

All ODD products are considered by default addressing an UMN

Access to medicines – incentives for innovation

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- Targeted data exclusivity approach with 8 years of unconditional data protection

- Pre-authorisation support
- Faster authorisation

- Earlier market entry of generic and biosimilar medicines

- Additional indication(s)
- Comparative clinical trials
- (High) Unmet Medical Need
- Market launch

A streamlined regulatory framework

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- **Pre-authorization:**
  support to promising medicines to accelerate development and attract investments + regulatory sandboxes option

- **Faster authorisation**:
  180 days standard review
  150 days accelerated review

- **Regulatory efficiency / Lower regulatory burden:**
  simplified procedures, more agile and flexible expertise, better use of data and digitisation

Patients and HCP becoming full members of CHMP

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Art.148 - Committee for Medicinal Products for Human Use activities

3. The Committee for Medicinal Products for Human Use shall be composed of the following:

   a) one member and one alternate member appointed by each Member State, in accordance with paragraph 6;

   b) four members and one alternate members appointed by the Commission, on the basis of a public call for expressions of interest, after consulting the European Parliament, in order to represent healthcare professionals;

   c) four members and four alternate members appointed by the Commission, on the basis of a public call for expressions of interest, after consulting the European Parliament, in order to represent patient organisations.

CHMP composition – specified expertise

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Art.148 - Committee for Medicinal Products for Human Use activities

6. The members and alternate members of the Committee for Medicinal Products for Human Use shall be appointed on the basis of their relevant expertise in the assessment of medicinal products which should cover all types of medicinal products covered by [revised Directive 2001/83/EC] and this Regulation and which include medicinal products for **rare and paediatric diseases, advance therapy medicinal products, biological and biotechnological products**, in order to guarantee the highest levels of specialist qualifications and a broad spectrum of relevant expertise.

Legal basis for HCPWP

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Art.146 – Scientific Committees – General provisions

8. The scientific committees and any working parties and scientific advisory groups established in accordance with this Article shall in general matters establish contacts, on an advisory basis, with parties concerned with the use of medicinal products for human use, in particular patient and consumer organisations and healthcare professionals' associations.

For that purpose working groups of patient and consumer organisations and healthcare professionals' associations shall be established by the Agency. They shall ensure a fair representation of healthcare professionals, patients and consumers covering a wide range of experience and disease areas, including orphan, paediatric and geriatric diseases and advanced therapy medicinal products, and a broad geographical range.

Rapporteurs appointed by the scientific committees may, on an advisory basis, establish contacts with representatives of patient organisations and healthcare professionals' associations relevant to the therapeutic indication of the medicinal product for human use.

Novel types of applications/authorisations

- Platform Marketing Authorisation
- Combination pack
- Temporary Emergency Marketing Authorisation
- Conditional MA
- Hospital Exemption (HE) for ATMPs

Where is science, technology & medicines regulation heading to?

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Attract pharmaceutical R&D by providing a future-proof, stable legal framework and a favourable regulatory environment

Provide a more targeted incentives framework for innovation with a focus on patient access and addressing unmet medical needs

Address antimicrobial resistance (AMR)

Boost regulatory support for the development of promising medicines

Boost innovation and EU competitiveness through an efficient and simplified regulatory framework

Make medicines more environmentally sustainable

Any questions?

Further information

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