Medicines & Healthcare products Regulatory Agency

# Accelerated Approval Processes

## MHRA, UK

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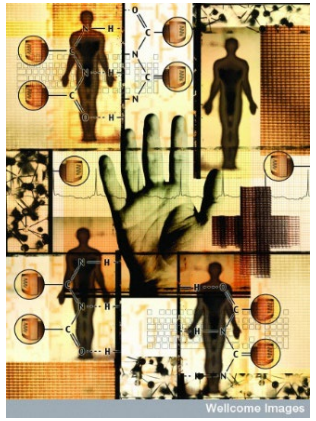
**31st January 2023**

# The MHRA – who we are

**Medicines and Healthcare products Regulatory Agency (MHRA)**

- The Medicines and Healthcare products Regulatory Agency regulates medicines, medical devices and blood components for transfusion in the UK
    - ➢ Combine regulation, standards and data, underpinned by science and research
- Since January 2021, we have been a standalone Regulator following the exit from the European Union
- Aim to be an enabler of medical innovation
- Key goals include development of new processes to bring medical products safely and speedily to market and putting patients first
- https://www.gov.uk/government/organisations/medicines-and-healthcare-products-regulatory-agency

# The Early Access to Medicines Scheme (EAMS)




## Pre-Licensing Access

- EAMS aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation when there is a clear unmet medical need

- Formal oversight and review by the medicines regulator through a two-step process, Step I: the Promising Innovative Medicine (PIM) Designation, Step II: the EAMS Scientific Opinion

- Patients benefit from medicines before they are licensed, and prescribers have greater confidence in the safety and efficacy of prescribing

- New legal basis 2022 aims to ensure that EAMS remains an important route for earlier patient access - opportunity to collect real world data in advance of the licence

- https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams

# Drug licensing – marketing authorisations

**Flexibilities in the licensing system to support earlier patient access**

- Rolling review: intended to streamline development by offering periodic enhanced regulatory interaction, incremental assessment of CTD modules and advice to reduce the risk of failure at the final phase
- Day 150: 150-day assessment timeline for all high-quality marketing authorisation applications (MAAs), aiming at accelerating the availability of medicines
- Conditional MA: To address unmet medical needs of patients, it may be necessary to grant marketing authorisations with less complete data than is normally required - subject to certain specific obligations to be reviewed annually
- Exceptional circumstances: The Applicant must demonstrate that they are unable to provide comprehensive data on the efficacy and safety under normal conditions of use
- https://www.gov.uk/topic/medicines-medical-devices-blood/marketing-authorisations-variations-licensing

# Innovative Licensing and Access Pathway (ILAP)

**Aim to ensure products are developed regulatory and access ready**

- The ambition is to deliver safe, early and financially sustainable patient access to innovative medicines

- Key aspect is the partnership between the MHRA and three UK HTA bodies; NICE, SMC and AWTTC (cost effectiveness); and patient input – closer engagement with NHS system partners

- Alignment of evidence generation, evaluation and access activities throughout the development pathway
  - better integration of regulation and HTA aspects

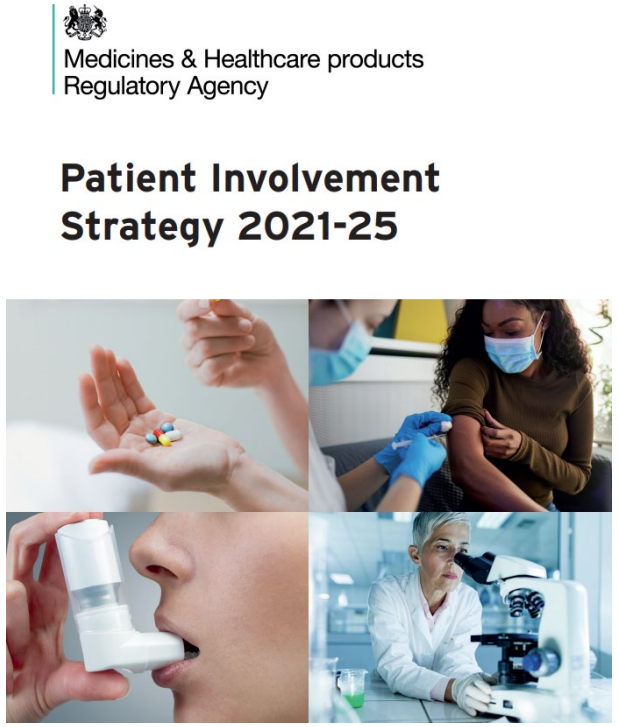
- Innovation Passport designation, Target Development Profile, ILAP toolkit

- https://www.gov.uk/guidance/innovative-licensing-and-access-pathway

# Patient engagement and involvement

**Putting patients first**

- Patient Involvement Strategy 2021-25, Two-year Delivery Plan 2021-2023 ‘Putting patients first – A new era for our agency’
- Ambitious roadmap for change: ensuring that we put patients first
- Define how we will engage and involve the public and patients at every step in our work
- Deliverables on Patient Reported Outcome Measures
- Develop our understanding of patient perceptions of benefit-risk to enhance regulatory decision-making



# Future scope

**Where next?**

- Enhanced patient engagement and involvement
- Wider collaboration with the life sciences ecosystem – end to end system alignment
- Defining better what we mean by ‘innovative’ and ‘unmet medical need’

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