# Coordination and Support Action: Enabling platform on medicines adaptive pathways to patients

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# Content

- Background information
- CSA project proposal
- References / Abbreviations
- Q&A session

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# The challenges

- The regulatory environment is lagging behind rapidly evolving science
- Conventional R&D models are no longer financially viable
- Medicinal therapy is rapidly moving towards a personalised medicine paradigm
- A more flexible pathway within current pharmaceutical legislation and reimbursement framework is desirable to address patients' needs
  - Some initiatives: NewDIGs, FDA's Breakthrough Program, UK's Early Access to Medicines Scheme, EMA Adaptive Licensing Pilot project (recently renamed 'Adaptive Pathways Pilot Project)

# What is MAPPs – Medicines Adaptive Pathways to Patients?

MAPPs refer to flexible development and access pathways **within the current European regulatory framework** that balances early patient access, public health and societal benefits

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# What is MAPPs – Medicines Adaptive Pathways to Patients?

## How is MAPPs different from current pathways?

- An early authorisation of a product, in a well-defined and targeted patient population with a clear safety and efficacy profile
- The target population is adjusted as additional evidence becomes available
- MAPPs may integrate adaptive clinical trial design, patient centric benefit/risk assessment and continuous re-evaluation as new evidence becomes available
- MAPPs relate to the entire life-cycle of a medicine from early development through licensing to patient access

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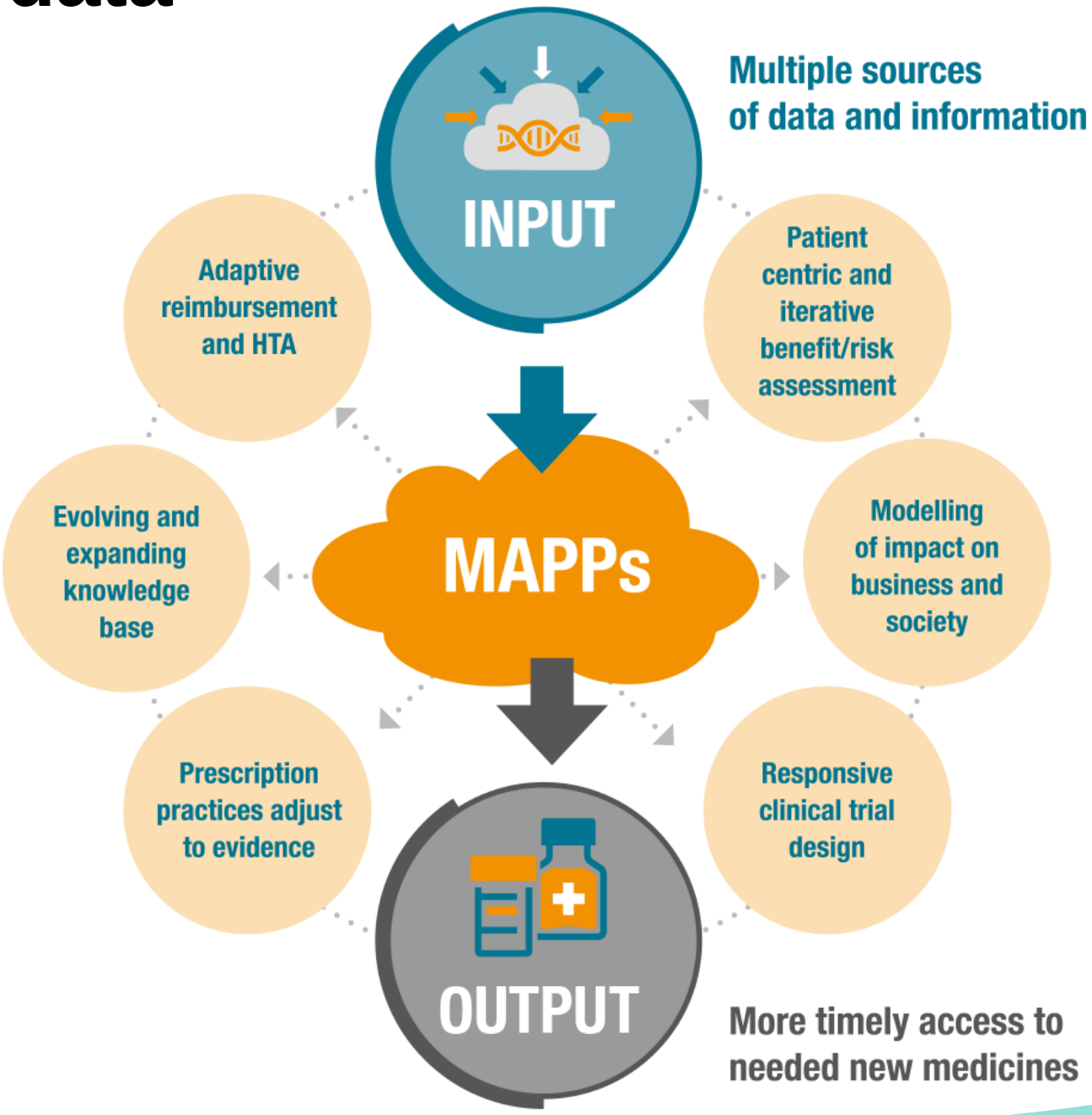
# Evidence data is key

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# To address the challenges

- Support is required to bring together, under a neutral collaborative framework, stakeholders to coordinate scientific activities to progress innovative, pragmatic and viable solutions

➔ **Coordination and support action (CSA) to coordinate effectively the MAPPs activities within IMI2**

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# The Coordination and Support Action: Objectives of the full project

- Build a platform with relevant stakeholders for the coordination of MAPPS-related activities within IMI2
  - **Gap analysis** – lessons learnt from existing IMI projects
  - **Informing research activities** – facilitate the inclusion of tools/methodologies in IMI2 research projects
  - **Knowledge management** – horizon scanning of non IMI activities
- Recommendations should contribute to align understanding of impact of MAPPs versus current paradigm

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# Expected impact on the R&D process

- A comprehensive scientific research plan for the development and exploitation of tools, methodologies, infrastructures
  - to help informing the whole product life-cycle
  - and provide the science-based evidence
  - in order to enable early patient access to innovative prevention and treatment options

# What's in it for you?

- **Academic researchers**
  - High-profile visibility to different stakeholders
  - Attractive funding option
  - Networking opportunity with key researchers and pharmaceutical industry
- **SMEs**
  - Scientific knowledge
  - Increased visibility for collaborations with large companies
- **Patients' organisations**
  - Increase access to information in areas of unmet medical needs
  - Networking opportunities
- **Public Health authorities**
  - Rapid access to effective development tools
  - Early-on influence on drug development
  - Optimised collaboration

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# The EFPIA partners in the CSA consortium

- AZ, BMS, Amgen, Astellas, Bayer, BI, Eli Lilly, GSK/GSK Vaccines, Ipsen, Janssen, Lundbeck, Lysogene, Merck KGaA, MSD, Novartis, Novo Nordisk, Pfizer, Roche, Sanofi, Sanofi/Pasteur, UCB

- Provide expertise in regulatory, HTA/pricing and reimbursement, R&D, clinical development, trials, B/R assessment, legal/IP, medical/health affairs and communication

- Provide support to the platform for communication and dissemination activities

- EFPIA (in kind) contribution:  € 1 130 000

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# Expected contribution of the industry partners

- Analysis of IMI project outputs, and their translation into regulatory and medical outcomes
- Interaction with on-going and future IMI projects and relevant groups involved in IMI projects' definition (e.g. IMI Strategic Governing Groups, EFPIA RDG)
- Monitoring of non-IMI activities relevant to MAPPs
- Liaison with non-IMI initiatives, coordination with various industry fora and across geographic areas, and liaison with other industry sectors
- Preparation of materials/meetings for in/external communication and dissemination of recommendations and conclusions

# Suggested architecture of the project

- Applicants are expected to suggest the architecture for the full proposal to set up the platform

- The proposed platform should address the scope and the expected objectives of the call project

# One suggested architecture of the project

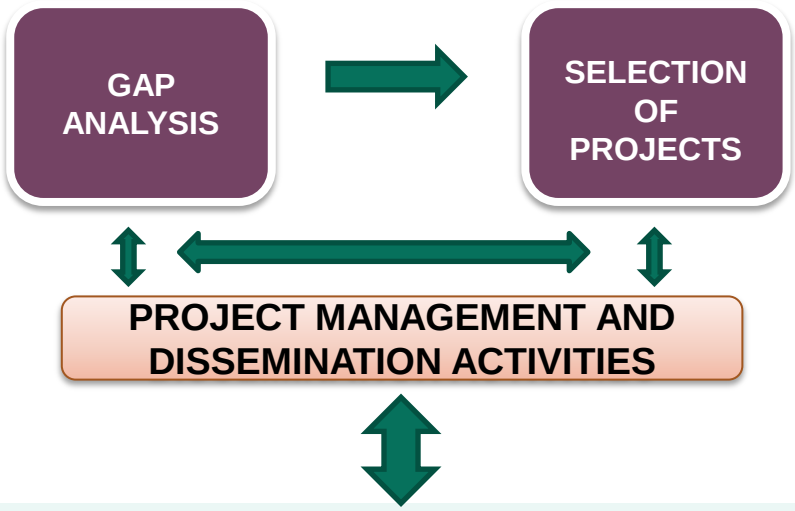
Interactions with on-going and future IMI projects and relevant groups involved in IMI projects' definition (e.g. IMI SGG, EFPIA RDG)

Analysis of IMI projects outputs, to translate these outputs into regulatory and medical outcomes

Monitoring of non-IMI activities relevant to MAPPs

Liaison with non-IMI initiatives, coordination with various industry fora and across geographic areas, and liaison with other industry sectors

## Medicines Adaptive Pathways to Patients




Preparation of materials for in/external communication and dissemination of recommendations and conclusions

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# The applicant consortium

- Multidisciplinary, e.g. regulators, HTA, payers, academia, SMEs, patient organizations, to work in synergy with EFPIA partners
- Adhoc stakeholders where needed

**To address the objectives and make key contributions, this may require:**

- Knowledge/expertise in drug development
- Understanding of R&D pathways and their challenges
- Ability to develop communication strategies on role and challenges of MAPPs to stakeholders and public at large
- Expertise in managing and coordinating complex projects

**Size shall be adequate to secure operational efficiency**

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# Coordination and Support Action

**Duration: 30 month – To be operational promptly**

**Call launched**

1st stage

2nd stage

Information on outcome of the evaluation
- max. 3 months from submission date to 1st stage

Information on outcome of evaluation
- max. 2 months from submission date to 2nd stage

Indicative date to sign off of grant agreements
- max 2 months from date of informing applicants following the 2nd stage evaluation

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# Some references…


- Eichler HG et al. Adaptive Licensing: Taking the Next Step in the Evolution of Drug Approval. *Clinical Pharmacology & Therapeutics* (2012); 91 3, 426–437

  http://www.nature.com/clpt/journal/v91/n3/full/clpt2011345a.html

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  http://www.efpia.eu/uploads/Modules/MCMedias/137329655454 6/IMI2%20Strategic_Research_Agenda_v%208%20July%202013.pdf

- EMA adaptive licensing Pilot

  http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/generalgeneral_content_000601.jsp&mid=WC0b01ac05807d58ce


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# Abbreviations

| | |
|---|---|
| CSA | Coordination and Support Action |
| EFPIA | European Federation of Pharmaceutical Industries and Associations |
| EMA | European Medicines Agency |
| FDA | Food and Drug Administration |
| HTA | Health Technology Assessment |
| IMI | Innovative Medicines Initiative |
| IMI JU | IMI Joint Undertaking |
| MAPPs | Medicines' Adaptive Pathways to Patients |
| NewDIGS | New Drug Development Paradigms initiative |
| RDG | Research Director Group |
| SGG | Strategic Governing Group |
| SME | Small and Medium Enterprises |
| UK | United Kingdom |

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