Accelerated Development of Appropriate Patient Therapies

A Sustainable, Multi-stakeholder Approach for MAPPS - Medicines Adaptive Pathways to Patients
Meet Jane.
Jane was Diagnosed with Advanced Cancer a Year Ago

At first, her tumour responded to treatment but it has now come back, and Jane knows the outlook is bleak.

There is a new experimental treatment being tested. Results from studies and early clinical trials look promising but it is still early days.

Jane’s reaction is swift: “I need to give this a try”
Meet John.
John Has a High Risk for Cancer, Given his Family History

John believes that, if he gets cancer, it is still a decade or two away.

One day, John hears “there is a new experimental cancer treatment, it is a novel concept. Results from studies and early clinical trials look very promising but it is still early days.”

John’s reaction is swift: “I hope this product is available after having been thoroughly tried and tested – I have no appetite for uncertainty.”
Who is right?
We believe that both Jane and John are “right”, given their respective situations, and that Healthcare Decision-Makers have an obligation to both Jane and John.

Medicines Adaptive Pathways to Patients (MAPPs) seeks to foster access to beneficial treatments for the right patient groups at the earliest appropriate time in the product life-span in a suitable fashion.
A Multi-Stakeholder IMI Project led by the European Medicines Agency Investigating MAPPs

- AstraZeneca is deputy project leader; Lygature is project coordinator
- Establish collaborative solutions and address barriers to the implementation of MAPPs
- Bring together patients, regulators, industry, Health Technology Assessment (HTA) bodies, payers, and academics under the umbrella of the IMI
- Provide patients with more appropriate access to innovative medicines
- **Initiate work packages to address existing barriers and test concepts to develop a ‘tool-kit’ to facilitate MAPPs**
A Public Private Partnership

- Funded by IMI-JU
- 01 June 2015 – 1 Dec. 2017
- 23 EFPIA members
- 2 patients’ organisations
- 2 HAs, 2 HTAs
- Payers as observers
- Total cost € 2 260 000

© Copyright IMI ADAPT SMART | This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 115890. This Joint Undertaking receives support from the European Union’s Horizon 2020 research and innovation programme and EFPIA.
Addressing Stakeholder Concerns ...

- **Which medicines** should MAPPs focus on?
- Should MAPPs be something for **special cases**, concentrating on the complexities of demonstrating the **real-life added-value of unique treatments**?
- If we develop **new approaches to data generation**, how can we **prevent a decline in the quality of evidence**?
- Does an **adaptive approval** mean also an **adaptive pricing & reimbursement**?
- How can we ensure that the product will **not be used off-label and the commitments are met**?
... & Facilitating MAPPs

- Produce actionable recommendations to assist with the implementation and adoption of MAPPs
- Find solutions to the challenges of the implementation of MAPPs and foster the aligned understanding of consortium members and their constituents
- Distribute findings, key discoveries and case studies from ongoing or completed MAPPs pilot projects, creating a MAPPs repository of knowledge and opportunities
- Support IMI2 by facilitating the inclusion of MAPPs enablers, tools and methodologies to address its challenges and opportunities
Work Package 1: Evidence generation over the entire product lifecycle

**GOAL:** Determine how the actual performance of the new therapy will dictate its use based on the evidence of its effectiveness and safety over time

**Work Package 1 has two core objectives:**

- Analyse and monitor activities that occur both within and outside of IMI programmes that provide and generate evidence about MAPPs
- Develop a gap analysis of the current and future needs of evidence generation to support MAPPs.

**WP1 Leads**

**Sarah Garner**  
NICE

**Solange Rohou**  
AstraZeneca
Work Package 1: Status of Actions

IN PROGRESS

• Complete a **report of MAPPs research gaps based on the review of mature IMI projects** to inform how best to leverage pre-existing knowledge

• Create a **horizon scanning report of future IMI and non IMI projects** to anticipate the outcomes of future projects to inform MAPPs

• Create **collaborative research proposals** based on gap analysis of what ADAPT SMART needs to accomplish
Work Package 2: Designing a New MAPPs pathway

GOAL: Create a new development pathway where patients, regulators, HTA/payers, practitioners, and industry are aligned

Work Package 2 has the following objectives:
- Create a standardised glossary of terms for MAPPs
- Identify a seamless pathway from pre-clinical research to market access
- Define engagement criteria of product to enter the MAPPs pathways
- Develop tools to ensure appropriate use of products by patients
- Integrate findings from the EMA pilot project and other relevant initiatives to MAPPs


Work Package 2: Status of Actions

**COMPLETED**
- ADAPT SMART Glossary is available for download

**IN PROGRESS**
- Create proposal for pragmatic operational criteria for MAPPs decision(s)
- Select the methods/toolset & data for sample scenarios
- Create briefing documents about the EMA pilots for working groups in the project
- Create a report with recommendations for the tools/systems to be used to guide appropriate use
Work Package 2: Status of Actions

**IN PROGRESS**
- Mapping of the different transition/engagement moments with stakeholders that are part of the MAPPs process.

ADAPT SMART has identified **23 key stakeholder decision points** that MAPPs must successfully address.
Work Package 3: Implications of Decision-making and Sustainability

**GOAL:** Address the opportunities and obstacles to a successful and ethical implementation of adaptive decision making in research, regulatory, and medical practice

**Work Package 3 includes the following in its scope:**

- R&D efficiencies and inefficiencies
- Pricing arrangements, risk-sharing, effective patient access and business cases for sponsors
- Ethical implications for patients and clinicians
- Intellectual property and regulatory data protection
- Legal implications for all decision makers
- Personal data protection

**WP3 Leads**

Richard Barker  
CASMI

Alicia Granados  
Sanofi Genzyme
Work Package 3: Status of Actions

IN PROGRESS

• Create a **matrix** that contrasts key *decision points of current vs. future processes* ➔ available on the AdaptSmart.eu website

• Create an inventory and possible paper on currently **available managed entry agreements** & establish recommendations on the *applicability of managed entry agreements* ➔ podcast available on the AdaptSmart.eu website

• Establish the points to consider on **ethical & legal aspects of adaptive decision making**
Work Package 3: Application of Managed Entry Agreements (MEAs)

- An arrangement between a manufacturer and payer/provider that enables access to (coverage reimbursement of) a health technology, subject to specified conditions.
- Can use a variety of mechanisms to
  - address uncertainty about the performance of technologies
  - manage the adoption of technologies in order to maximize effectively their use, or limit their budget impact
Financial-based MEAs

- Agreements between manufacturer and payers based on observable financial performance
  - Price agreement based on manufacturer’s market share
  - Price-volume agreements
  - Pricing by channel (discount on certain products/channels)
  - Capitation (discounts for specific patients)
  - Free initiation (Patient/dose dependent discount)
  - Portfolio agreement (discounts based on manufacturer’s portfolio)
Outcomes-based MEAs

• Agreements base on **defined outcomes (generally clinical)** or agreements based on the **development of new evidence**. This include
  
  – Patient risk sharing agreement (price decided based on patient subgroups with respect to probability of benefits of treatment)
  
  – Performance-linked reimbursement (e.g. outcome guaranteed)
  
  – Conditional coverage (reimbursement linked to the development of additional evidence)
AdaptSmart.EU – A Resource for MAPPs and Project Status
Some useful documents released on the website


Sign up for the Newsletter

Work Package 1 Update: Evidence generation throughout the lifecycle

By the end of 2016 the WP1 consortium will deliver their first report on the analysis of mature IMI projects developed so far. The report has identified tools and methods developed by these IMI projects, and performed a preliminary gap analysis that should be completed with the analysis of EU non-IMI projects which should start soon, based on a similar research process used for IMI project analysis. At the Annual Meeting hosted by the EMA, a workshop involving WP1 members will be the opportunity to reflect on work done in 2016, review the preliminary gap analysis and agree the 2017 next steps.

Listen to the podcasts

Podcasts

- ADAPT SMART & MAPPs from the Patient’s Perspective
  On 6 September, Nicola Bedlington from The European Patients’ Forum (EPF) and Yann Le Cam from Eurordis, two of Europe’s leading patient representatives, discussed adaptive pathways from the patient perspective.
  
  Listen to the podcast

- MAPPs – Agreeing on Success
- MAPPs can be a promising way of working
- Managed Entry
Accelerated Development of Appropriate Patient Therapies

A Sustainable, Multi-stakeholder Approach for MAPPS - Medicines Adaptive Pathways to Patients