Introduction to Adaptive Pathways and ADAPT SMART

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Agenda

• Why Adaptive Pathways?
• Adaptive Pathways concept
• Initiative by EMA: PRIME
• Adaptive Pathways and ADAPT SMART
• Engagement criteria for Adaptive Pathways
• How could an Adaptive Pathway look like?
• State of Play
Regulating medicines: a balancing act

How to manage the “fourth hurdle”
Adaptive Pathways Concept

Definition:
Medicines Adaptive Pathways to Patient (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 sustainable fashion.

MAPPs is a prospectively planned, iterative approach to medicines development and access pathways within the current regulatory framework:

- Make best use of existing tools and methods (e.g. scientific advice, conditional approval, real world data, registries, managed entry agreements, pricing schemes…)
- With multistakeholder engagement
MAPPs characteristics

• Addresses a high unmet medical need
• Provides timely access for the target population in need
• Collaboration between all stakeholders
• Continuous evidence generation during the life-span
• Harnesses real world data and manages risks
• Manages proper utilisation
• Ensures sustainability of the innovation and health care systems
Current pathway

![Diagram showing the current pathway for drug approval with categories for patients treated, no active surveillance, patients in observational studies, registries, and patients in RCTs (or other interventional studies).]

High number of patients treated with no active surveillance

High number of patients over a long period of time

License

Number of patients treated

Time (years)

MAPPs: Adaptive pathway

<table>
<thead>
<tr>
<th>Number of patients treated</th>
<th>Time (years)</th>
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<tbody>
<tr>
<td>Smaller number of a selective patient cohort over a reduced period of time.</td>
<td>Initial license</td>
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<td></td>
<td>“Full” license</td>
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<td>Less number of patients treated without active surveillance.</td>
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<td>Increased number of patients monitored with active surveillance.</td>
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- Patients treated, no active surveillance
- Patients in observational studies, registries
- Patients in RCTs (or other interventional studies)
Initiative by EMA (since March 2016)

• supporting the development of medicines that target an unmet medical need
• fostering early, coordinated and continuous dialogue with stakeholders to facilitate robust data collection and high quality marketing authorisation applications
• speeding up evaluation so that medicines can reach patients earlier
• encouraging developers to focus resources on medicines likely to make a real difference to patients’ lives.
A Public Private Partnership

- Funded by IMI-JU
- 01 June 2015 – 1 Dec. 2017
- 22 EFPIA members
- 2 patients’ organisations
- EMA’s network and 3 HTAs
- Payers as observers
- Total cost € 2 260 000
Engagement Criteria for MAPPS: discussion paper

Framework of questions to be addressed by stakeholders when considering the MAPPS pathway for a given medicinal product:

• Can we define a target population with a high unmet medical need? Does the product hold sufficient promise to address unmet medical need?

• Can a prospective iterative post-(initial) marketing authorisation development plan be proposed, developed, implemented and agreed?

• Are there workable tools to ensure appropriate product utilisation?

• Are there workable ‘strategies’ for payers in case the product under-performs?

• Is there sufficient commitment and resources from relevant stakeholders to ensure successful interaction?

• Which critical aspects for pharmaceutical development would need to addressed?
How could a MAPPs pathway look like?

• **Conceptual framework** within existing EU and national legal frameworks
• **Mandates** of EU and national competent authorities *unchanged*
• Make best use of **existing tools** (e.g. scientific advice, registries, MEA, pricing schemes…)
• **Coordinated dialogue** with relevant stakeholders
• **Iterative development plan** with decision points and stakeholders’ formal engagement
Current Regulatory Framework

- Patients
- Evidence Strategy (Company)
- Scientific Advice (EMA)
- Regulatory Decision (CHMP/Commission)
- Additional Information (Company)
- HTA/Payer Decision (Member State)
- Joint Assessment by EUnetHTA
- Parallel HTA Advice
- Evidence Strategy (Company)
Iterative development plan as vehicle

• An evolving product development strategy, adapted through multi-stakeholder engagement

• The evidence generation plan should address the critical questions to support subsequent pricing and reimbursement decisions at the national level and subsequent indications
### MAPPs – state of play

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<th>MAPPs component parts</th>
<th>Acceptability</th>
<th>Feasibility</th>
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<tr>
<td>• Focus on high unmet need (sub-)population and products likely to have major impact</td>
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<td>• Reduce uncertainty fast; react to incoming data (iterative development; rapid cycle analysis)</td>
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<td>• Pre-plan across whole lifespan, incl. post-market</td>
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<td>• Use entire tool box for knowledge generation (RCT vs RWD)</td>
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<td>• Manage on-market utilisation</td>
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<td>• Leverage multi-stakeholder collaboration</td>
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<td>• Address sustainability to healthcare system</td>
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For more information visit the ADAPT SMART website:  www.adaptsmart.eu

About MAPPs
MAPPs refer to a prospectively planned, iterative approach to medicines development and access pathways within the current regulatory framework that optimises early patient access, public.

About ADAPT SMART
ADAPT SMART provides a novel multi-stakeholder platform to help address common questions about how MAPPs is put into practice in Europe.

Project Deliverables
ADAPT SMART consists of distinct work packages, each with an individual, focused set of deliverables.

Progress Report
The progress report is designed to track the concrete progress made on specific deliverables for each work package.