



# Adapt Smart & Adaptive Pathways

**STAMP Meeting**

**14 March 2017**

**Brussels**

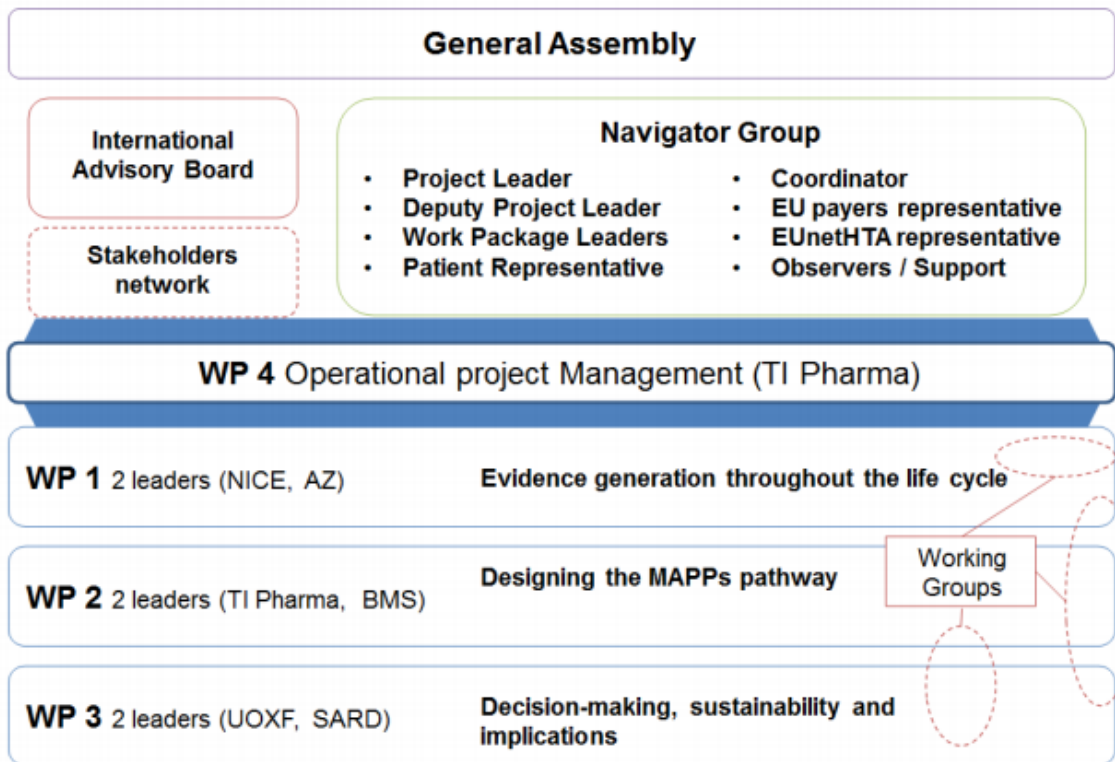
# Agenda

- MAPPs and Adapt Smart
- Engagement criteria for MAPPs: discussion paper
- How could a MAPPs pathway look like?
- Appropriate use by targeted patient populations

# MAPPs and ADAPT SMART

- 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, a 'lifespan approach'
  - Make best use of **existing tools and methods** (e.g., conditional approval, scientific advice, RWD, registries...)
  - With multistakeholder engagement
- The IMI ADAPT SMART consortium has established a platform to **facilitate** and **accelerate the availability of MAPPs** to all healthcare stakeholders → science, policy, political implications

# Governance



## The Navigator Group

### Patients

Eurordis / EPF

Yann le Cam / Nicola Bedlington

### Regulators – EMA

Hans-Georg Eichler, Tomas Salmonson

### Academia

CASMI / Lygature

Richard Barker / André Broekmans &

Pieter Stolk

### Industry/EFPIA

AstraZeneca / Sanofi-Genzyme

Solange Rohou / Alicia Granados

Madga Chlebus

### HTAs

NICE / AIFA-EUnetHTA

Sarah Garner / Simona Montilla

### Payers (observers)

MEDEV

Ad Schuurman & Anna Bucsics

# Achievements (NOT results!)

- Agreement on scope and goal (from highly divergent starting positions)
- Engagement of (some) EU payers as observers
- Ex-EU geographical spread (e.g. Canada, Australia, Japan: +; USA: +/-)
- Wide dissemination of the MAPPs idea

# Issues & concerns to address

## General comment

1. MAPPs not well understood, lack of transparency

## MAPPs-specific comments

1. Need and unmet need?
2. Lowering the evidence standards
3. RCT and Real World Data (RWD)
4. Promises, compliance, exits
5. On-market utilisation

# Main deliverables so far

- Engagement Criteria – [discussion paper](#)
- Report on Seamless Pathway and Decision Points: *to be published*
- Report on Managed Entry Agreements: *to be published*
- Report on MAPPs research gaps based on review of mature IMI projects: *to be published*
- [Glossary of terms](#): 143 terms defined

# Engagement Criteria for MAPPs: discussion paper

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

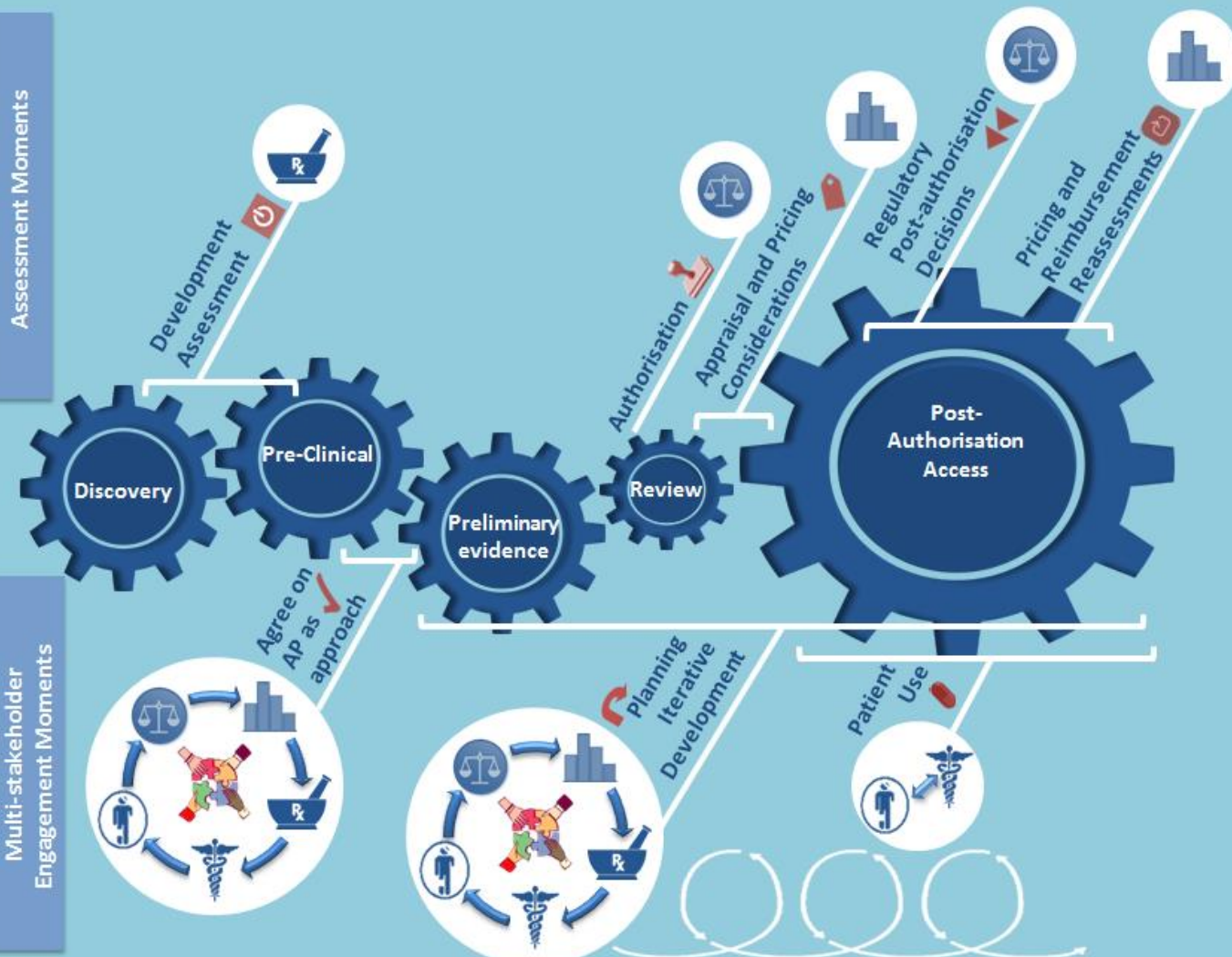
- *Can we define a target population with a high unmet medical need? Does the product hold sufficient promise to address unmet medical need?*
- *Can a prospective iterative post-(initial) marketing authorisation development plan be proposed, developed, implemented and agreed?*
- *Are there workable tools 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 be 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...)
- **Coordinated dialogue** with relevant stakeholders
- **Iterative development plan** with **decision points**, with **stakeholders' formal engagement**

Assessment Moments



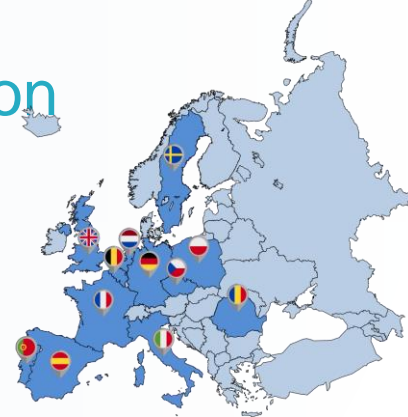
Involved Stakeholders

-  Patient Representatives
-  Health-Care Professionals
-  EU Regulatory Network
-  Pricing and Reimbursement Authorities
-  Medicine Developers
-  Project Manager

# Example: iterative development plan

- 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

# Appropriate use by the targeted patient population



- A survey was developed to:
  - Assess **availability** of tools/systems in the EU to guide appropriate use
  - Identify **consequences** for non-compliance
  - Estimate the **impact** on guiding appropriate use and the difficulty to **implement** these tools/systems
- Survey was sent to 12 EU Member States through EFPIA member companies
- Identify knowledge gaps and create a proposal for further study and recommendations to the national health systems, EMA, European Commission, health care providers and patients

# Results of the Survey

- **Availability:**
  - Large concurrence of tools/systems
  - We received no information indicating that tools/systems were missing
- **Consequences:**
  - Differences between MSs, e.g. Germany: *no consequences*; versus Spain/Italy/France: *reimbursement restrictions*
- **Impact:**
  - Restrictions at the level of *assessing/diagnosing* patients and *prescribing* have the highest impact
  - Limited impact is expected from DHPC type communications
- **Implementation:**
  - Implementation of speciality, assessment of demographic characteristics and assessment less challenging compared to other tools/systems (e.g. PSPs)

		Availability	Total impact score	Implementation score (# of respondents)
	Treatment guidelines	100%	3.8	3.1
	Direct Healthcare Professional Communication	100%	3.1	3.5
	Diagnostic test	92%	4.3	3.3
	Assessment of prior history	92%	4.3	1.9
	Assessment of demographic characteristics	67%	3.4	1.7
	Speciality	100%	4.7	1.4
	Certification	25%	4.5*	4.0*
	Site approval	42%	4.0	3.3
	Patient support program	92%	3.9	3.6
	Registry	100%	3.8	3.6

# Conclusion and recommendations derived from the survey

- Limited evidence is available on the impact of tools/systems - Investing in evidence on successful tools/systems can help to identify which strategies are most suitable for a MAPPs context
- This could be addressed by designing a scenario for a specific product and asking Member States how they would manage appropriate use in this specific case



For more information visit the ADAPT SMART website: [www.adaptsmart.eu](http://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.

