Workshop report: Selection Criteria for MAPPs: Exploring and aligning stakeholder needs

(Amsterdam, 29 February 2016)

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IMI ADAPT SMART workshop - Selection Criteria for MAPPs: Exploring and aligning stakeholder needs (Amsterdam, 29 February 2016)
Preliminary conclusions

This document was prepared by André Broekmans and Pieter Stolk (Escher, Lygature) for the participants in the "Innovation for the benefit of the patient" conference organized by the Dutch Ministry of Health on 1-2 March 2016. These conclusions may be modified and do not constitute an official ADAPT SMART publication.

I. Summary

- The IMI ADAPT SMART workshop included representatives from key European stakeholder groups involved in the adaptive pathways discussion (regulatory agencies, HTA bodies, companies, payers, patients and health care professionals). The discussion identified relevant topics, commonalities and areas of divergence.
- MAPPs should balance sustainability of the innovation system and of healthcare systems.
- MAPPs (description below) will not be a new pathway: it is a toolbox that can be used in specific circumstances. MAPPs will rely on current safety, efficacy and quality criteria and therefore must not lower standards for products that enter the market; regulatory demonstration of therapeutic efficacy and positive benefit-risk needs to be done before market entry is considered. Rather, MAPPs aims to generate more *relevant* evidence.
- It is critical to discuss the entry for MAPPs with all relevant stakeholders. The criteria proposed are the trigger for in-depth discussions aimed at co-designing the development plan, timelines, decision points, including reimbursement and prescribing conditions, and prices that can move up or down. These discussions also guide the pre-defined conditions for leaving the pathway.
- Diversity in Europe is a given, therefore, it will be difficult to formulate common principles for early (adaptive) pricing discussions. Nonetheless, where these can take place, they should. Moreover, questions that are relevant for HTA/payers (e.g. on outcomes or comparators) have to be included in the dialogue as soon as possible, even if only indicative information is available at that moment.
- The concept of MAPPs can further benefit from cases and demos that demonstrate the added value and to refine the discussion between stakeholders.
- When communicating about MAPPs, expectations should be managed as the first products that would emerge from an envisioned MAPPs approach are still several years into the future.

II. About MAPPs & ADAPT SMART

- MAPPs is a concept that, based on scientific innovation, seeks to provide timely access to medicines with a potential to address unmet need(s) in specific, well-defined patient populations, in a sustainable way.
    - MAPPs covers development and regulatory approval, HTA value assessment, pricing/reimbursement decisions, access and exit.
    - Marketing authorisation and reimbursement/access decisions are made using a framework within the existing legislation which supports the launch of a therapy, based on early initial evidence.
    - Data generated post-marketing authorisation, including real-world evidence (RWE), should support progressive decrease of benefit-risk uncertainty, and may lead to adjustment(s) in the licensed indication, use and price.
- ADAPT-SMART (Accelerated Development of Appropriate Patient Therapies: a Sustainable, Multi-stakeholder Approach from Research to Treatment-outcomes) is an IMI2 Coordination and Support Action (running to end 2017) investigating and exploring conditions and feasibility of the MAPPs concept within the EU regulatory/legal context. The consortium involves industry, patients, regulators, HTA bodies, and payers as standing observers.

III. Selection criteria

The proposed criteria below were considered to set an appropriately broad framework within the current legal and regulatory systems that allows entering into multi-stakeholder discussions aiming at co-designing the development plan, timelines, decision points, including for reimbursement and prescribing conditions. The level of commitment to complete the process is dependent upon consensus of all stakeholders. Therefore, buy-in of all stakeholders is needed.

Eligibility of the product

- MAPPs should be focused on disease transformative products in areas where there is a high unmet medical need, i.e. intended for a life threatening or severely debilitating condition for which no treatment or no satisfactory treatment exists, with anticipated clinically relevant major improvement and earlier access is important for the patient.
- It is expected that MAPPs would apply where there is a probability to define, deliver and measure quantifiable outcomes.
- Initially, MAPPs could start with products for which there is a clear biological rationale, a well-understood mechanism of action and/or good clinical understanding of the disease.
- It is expected that for products entering MAPPs, Chemistry Manufacturing Controls issues (quality of the product) are manageable.
- MAPPs could be considered to incentivize investment into areas of significant public health need (high burden on society and patients – Priority medicines for Europe and the World, WHO 2013).

Feasibility of collecting iterative evidence

- A clear plan for iterative evidence generation must be defined upfront and as early as possible. Trust in the process is an essential success factor: stakeholders should be confident at entry of the MAPPs pathway that all prospective commitments by all parties will be met.
- The pathways should assure that data and information needs from all stakeholders are met (this includes, for example, the needs of HTA bodies and payers).
- Milestones need to be defined at which evidence generation will be monitored and adjusted. This includes outcomes definition which will be the basis for decisions on reimbursement levels (including exits of the adaptive pathway).
- To what extent tools are available to ‘manage entry’ (both at the level of specific payment/reimbursement schemes and the possibilities for prescribing controls) differs between member states. As part of its work in IMI ADAPT SMART will provide an overview to fuel discussion around future directions.
- Stakeholders should be assured that a satisfactory infrastructure to enable the sustainable collection and analysis of post-marketing evidence is available and a mechanism for cost-sharing is agreed. Setting up disease registries could be an area for early (pre-competitive) collaboration between stakeholders.

IV. Other points to consider

Suitability of the pathway for stakeholders

- It is important to address the resource needs for stakeholder dialogue and to focus the interactions during the lifespan of the product. Sufficient resources have to be made available to allow stakeholders to interact, and from a practical perspective, only a limited number of processes should be ongoing at the same time.
- Smart scheduling of the key meetings/decisions in the process is needed. This could consist of the identification of key time points in order to align with various requirements and procedures, or to identify specific trigger points for decision-making (e.g. based on number of patients treated or studies completed).