



Blog From D2.03 Work Package Leaders: reflections on the workshop's outcomes.

March 2016

The work leading to these results was conducted as part of the ADAPT SMART consortium (Accelerated Development of Appropriate Patient Therapies: a Sustainable, Multi-stakeholder Approach from Research to Treatment-outcomes). For further information please refer to www.adaptsmart.eu. This paper reflects the views of the authors.

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.



On the 29th of February, ADAPT SMART hosted an invitation-only multi-stakeholder workshop around selection criteria for MAPPs in Amsterdam, The Netherlands. Angelika Joos of MSD and Mathieu Boudes of EURORDIS attended the workshop in their capacity as ADAPT SMART Task 2.03 leaders. Here they share an update on the workshop's progress.

As the first event organised by ADAPT SMART, (aside from the kick-off meeting), the workshop on the 29th of February marked an important step in helping to analyse the vision for a MAPPs approach, and to clarify some aspects around Medicine Adaptive Pathways to Patients (MAPPs). MAPPs seek to foster access to beneficial treatments for the right patient population at the earliest appropriate time in the product life span, in a sustainable and affordable fashion. It is important to note that MAPPs does not intend to create a *new* regulatory framework, but instead to make more efficient use of various existing tools of the regulatory/legal and Health Technology Assessment (HTA) and access rules for medicines in the EU.

In the Amsterdam workshop, the discussions with patient representatives, regulators, payers, HTA bodies, prescribers and pharmaceutical companies focused on how optimal entry criteria for the selection of products that could undergo MAPPs in the near future can be identified – such as the target population, product characteristics and considerations from ALL parties involved in medicines development, use and access. The discussions proved useful in establishing a general agreement on main categories of selection criteria for MAPPs, while also providing greater clarity on existing questions and uncertainties around MAPPs. As a consequence the understanding of different perspectives amongst and between stakeholders increased quite a lot.

Understanding what “MAPPs” means

One point that came up in the workshop was the fact that MAPPs is not about implementing a brand new regulatory system – but rather presents a prospective co-designed and planned approach of data generation during development, at the earliest phase possible involving all stakeholders to support access to medicinal products answering a high unmet need. MAPPs also mean that a product' characteristics (such as safety, efficacy, value, etc.) will be reassessed at predefined time points after authorisation while more real world evidence is generated.

In more detail: MAPPs aim to foresee an initial marketing authorisation and reimbursement of a medicinal product in a well-defined patient subgroup. This indication can then be widened to a larger patient population, based on additional evidence gathering where initial data predicting certain product characteristics are complemented and confirmed through the collection of post-authorisation data on the medicinal product's use.

Multiple stakeholder interactions are key during the process. Therefore, MAPPs should focus on transformative products that address high-unmet medical needed in order to proof the approach and collect collaborative learnings while conserving limited resources.

Elements to assist development of the MAPPs process

There remains some confusion about the elements to be discussed for the development of the MAPPs process (something ADAPT SMART's Task 2.5 is looking at) versus the agreement on the selection criteria (Task 2.3) when new assets are entering this process. These selection criteria will mainly be used as high level guiding principles for developers when reviewing their portfolio and proposing specific products for MAPPs.

So what tools are already in place that can help advance MAPPs? Examples discussed during the workshop include:

- Joint or parallel early dialogue or advice from HTA or Regulatory Agencies representatives given during development;
- In the post-approval process, the on-going gathering of evidence through real-life use a supplement to pre-Marketing Authorisation (MA) clinical trials;
- In the MA process, approval can be given in sequential stages – for instance via conditional approval, or post-approval safety and efficacy studies (PASS/PAES);
- The meaningful use and development of data collection systems such as disease registries and electronic health records will be a key element at the core of MAPPs.

Using these existing tools, MAPPs will then not change current standards for evaluation of benefit/risk, but make better use of existing data to grant MA from a quality, safety and efficacy point of view as well as assessing its value for patient access.

Selection Criteria for MAPPs

Different from the tools that can advance development of the MAPPs process are the selection criteria of MAPPs. The discussions around the criteria that could be used to determine inclusion in MAPPs indicated wide agreement among stakeholders—while also acknowledging that broader buy-in from the community will be needed. The discussions during the workshop focused on the following:

- Disease characterisation and matching the definition of unmet medical need;
- Capacity to ensure appropriate stakeholder interaction during product life cycle;
- In-depth knowledge of the R&D environment and the capacity to provide comprehensive data post-authorisation and feasibility of evidence generation;
- Ability to adapt rapidly and efficiently the manufacturing and continued supply of a medicine.

In general, there was a broad agreement of the key categories and the specific data requirements to demonstrate the suitability of a product will be included during the fine-tuning of the discussion paper.

Discussion document forthcoming – your input needed!

The workshop will result in a draft discussion document on the criteria to be used when considering suitability of a MAPPs approach for a given product. This will subsequently be released for public consultation to seek broader input from stakeholders. Questions to be considered include:

- Are the criteria for MAPPs appropriate?
- Have all questions from the view of all stakeholder groups been considered?
- Are the descriptions of the criteria adequate?
- Are any considerations missing?

As part of our on-going work of ADAPT SMART's Work Package 2, we will be regularly reviewing the document over the course of the project. The ADAPT SMART consortium looks forward to collaborating with you on this project, as our efforts to explore and align stakeholder needs regarding the selection criteria for MAPPs are moving ahead. There will also be more parallel discussions in task 2.5 related to the MAPPs process development that may consider important other elements such as “exit strategies” in case predicted product characteristics cannot be fulfilled based on collected data post-approval.