Program Workshop 29 February 2016

Selection Criteria for MAPPs: Exploring and aligning stakeholder needs

Background

When thinking about MAPPs (Medicine Adaptive Pathways to Patients) as a new product development paradigm, one of the first questions is: which products are suitable for a MAPPs pathway, i.e. what are 'selection criteria' for MAPPs?

When discussing these selection criteria it is not sufficient to only define criteria for the regulatory process itself. For a seamless adaptive pathway to be viable, the preconditions and requirements of *all* stakeholders that have a responsibility for decision-making throughout the lifespan of a new medicinal product (including clinical development, regulatory approval, reimbursement and use in clinical practice) should be taken into account. For example, stakeholders such as HTA bodies may have specific preconditions for efficacy/effectiveness and safety of the product before they are willing to accept potentially more uncertainty about the product value at initial launch or they must be willing and able to share (financial) risk. In other words: is there a viable 'entry' and 'exit' strategy? From their perspective, industry may want to have earlier input on approval and reimbursement probability before deciding on an extensive clinical research program or investment in a new manufacturing process.

Only if all preconditions and requirements are sufficiently addressed MAPPs can be a success. Therefore, when entering into the MAPPs process all stakeholders should have agreement on, e.g.:

- The eligibility of the medicine for the adaptive pathway;
- The feasibility to collect relevant information on quality, safety and efficacy/effectiveness in a continuous way during development and real-world use,
- The decision points and timing to assess whether the medicine is still suitable for MAPPs.

During an ADAPT SMART stakeholder workshop on the 29th of February all these elements will be discussed. A briefing paper of the ADAPT SMART working group on selection criteria (D2.03) will be circulated in advance of the meeting.

Participants and location

Number of participants: 40, by invitation only. This number should ensure good interactions in the plenary sessions and room for more in-depth discussions in the break-out sessions.

Location:
Pakhuis De Zwijger
Piet Heinkade 179
1019 HC Amsterdam

Final program

Workshop chair: prof. Bert Leufkens, Chair of the Dutch Medicines Evaluation Board, the Netherlands

Morning session

Theme:
Investigating the feasibility of MAPPs: which conditions should be fulfilled when entering the adaptive pathway concept?

- Short presentations (5 to 10 minutes) with bullet statements. The bullet statements will contain the "nice to have", "need to have" and the "show-stoppers".
- Panel discussion with all stakeholders to clarify positions.
- Presentation of the discussion paper on selection criteria.

Time schedule:

10:30 – 11:00	Registration and coffee
11:00 – 11:05	Opening by Bert Leufkens, chair of the workshop
11:05 – 11:15	MAPPs and ADAPT SMART by Hans-Georg Eichler, ADAPT SMART project leader
11:15 – 12:10	Reflections from stakeholders *Industry:* Luk Maes, BMS, Belgium *Patients:* Yann Le Cam, Eurordis, France *Prescribers:* Rosa Giuliani, S. Camillo-Forlanini Hospital, Rome, Italy *Regulators:* Rob Hemmings, MHRA, United Kingdom *HTA bodies:* Jacoline Bouvy, NICE, United Kingdom *Payers:* Ad Schuurman, ZINL, the Netherlands

12:10 – 12:40 Panel discussion chaired by Bert Leufkens
12:40 – 12:50 Presentation of the discussion paper on selection criteria by Angelika Joos (MSD, on behalf of the ADAPT SMART working group D2.03)

12:50 – 13:30 Buffet lunch

Afternoon session
Theme:
More detailed discussion of the selection criteria proposed in the morning in 2 parallel break out sessions with a synthesis during the plenary session.

The break-out session will be structured around the following questions:

- *What are criteria for choosing a product based on the **target disease and population?** (what 'promise' does it have to fulfil), just addressing an unmet medical need may be too limited for some stakeholders, how can we identify common ground here?).*
- *What **data and evidence** is required for the different stakeholders to make a decision? When is the data available for that decision?*
    - *What are **criteria that the regulatory process has to meet** up until initial approval? E.g.: feasibility to gather sufficient evidence pre-launch.*
    - *What are **criteria that the post-marketing phase has to meet?** E.g. both for companies and payers (what are feasible entry and exit strategies? Do stakeholders have sufficient confidence in that sustainable conditions can be met)?*
- *What other decision rules and mechanisms need to be in place to make the MAPPs pathway acceptable to all stakeholders?(e.g. in the context of the regulatory system, or not yet discussed in the meeting).*

Time schedule:

13:30 – 15:30 Break out sessions
Chairs:
Richard Barker, CASMI, United Kingdom
Finn Børlum Kristensen, EUnetHTA, Denmark
Rapporteurs:
Mathieu Boudes, Eurordis, France
Francesca Cerreta, EMA, United Kingdom

15:30 – 16:00 Tea break

16:00 – 16:50	Feedback from the break out sessions (by rapporteurs) and general discussion (chair Bert Leufkens)
16:50 – 17:00	Main conclusions out of the workshop by Hans-Georg Eichler