



## **Workshop report: The Impact of Adaptive Pathways and Adaptive Access on Intellectual property and Regulatory exclusivity rights**

(Brussel, 12 April 2017)

*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](http://www.adaptsmart.eu). This paper only reflects the views of the authors.*

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## **1. Background of D3.06 actions**

Activities of the D3.06 working group have been to map both the key *legal* impacts and additional key *perceptual* impacts of adaptive pathways and adaptive access on intellectual property (“IP”) and regulatory exclusivity right (“RER”) periods. The term RER encompasses regulatory data protection (“RDP”) and orphan market exclusivity (“OME”). How these questions affect predominantly medicine developers and regulators have been explored in a series of group workings and extended interviews. This work is complementary to other work streams in assessing any current or future legal blocks to the acceptance and implementation of MAPPs.

## **2. Objectives and Outcomes**

- To gain consensus and validate both legal and perceptual impacts of adaptive pathways and adaptive access on IP and RER periods between all major stakeholders, with a special focus on medicine developers, regulators and clinicians (incl. lawyers).
- To jointly explore potential recommendations to address the impacts on IP and RERs within the current legal framework, address perceptual uncertainties and any impact those recommendations may have, that can support the acceptance of MAPPs.

## **3. Introduction**

On April 12th 2017, the IMI ADAPTSMART consortium hosted a multi-stakeholder workshop. Twenty-two representatives attended, including: medicine developers, independent IP and regulatory lawyers, payers, academics and policy makers.

The workshop was opened by Stuart Faulkner (CASMI) who set out the objectives of the meeting. Andre Broekmans (Lygature) provided an “Overview of MAPPs and ADAPTSMART”, in particular he summarized the rationale for Adaptive Pathways and the details of ADAPTSMART project and consortium. As last part of the introductory session, Ian Hiscock and Alex Meier (Novartis) provided the “Perspective of a company: Intellectual Property & Regulatory Exclusivity Rights – Why do we need it?”

Following the introduction, participants joined two consecutive breakout sessions. The first reviewed the marketing authorization (MA) scenarios that could occur under MAPPs and their implications. The second session discussed a series of recommendations within the current legal framework and options for future work.

### **Assessing the current legal framework**

Three main scenarios had been explored that were considered to be the most likely under MAPPs with respects to impact on IP and RER periods;

- i) Standalone MA,
- ii) MA for product with subsequent approval of additional indications,

iii) MA under conditional approval that:

(a) subsequently gets approved for 'full' MA ("no exit") or

(b) subsequently is revoked/suspended ("exit scenario")

- With regard to RDP, it was clarified with regard to the so-called "8+2+1"-rule that only the 8 years of data exclusivity and the additional 2 years of market protection starting upon initial approval are granted by law "automatically", however, not the additional "+1" year of market protection as this requires (1) additional conditions to be fulfilled (i.e. approval of an additional indication of significant clinical benefit within the first 8 years after initial approval) and (2) pursuant to the Commission's Notice to Applicants a request to EMA to confirm the significant clinical benefit of this additional indication. Irrespective of that, the granting of the +1 year of market protection was generally rare, in particular with regard to conditional marketing authorisations (CMAs). For MAPPs, this could even be more challenging to achieve and therefore far from being guaranteed.
- Under MAPPs, a product developed under a self-standing MA only in one indication, rather than indication expansion, might not offer realistic economic gain. However, under MAPPs other secondary gains (outside of exclusivity periods) could be considered, such as earlier market access, access to multi-stakeholder dialogue, and more coordinated post-MA data generation. Questions remained over how the economic case for MAPPs could be made clear for medicine developers.
- With regard to the use and understanding of CMAs, based on the available information to date no CMA had been suspended/revoked due to safety/efficacy issues. Hence, in case of a greater use of CMAs as an authorisation pathway under MAPPs and this at an earlier time point, it is probable that also a MA suspension/revocation might occur due to non-confirmatory data. Therefore, it was discussed that the impact of such a suspension/revocation will need to be considered as this will impact the economic situation on a product specific case.
- Concerns were raised that an early MA (<5 years after the patent filing) will mean that there can be no compound supplementary protection certificate (SPC) – in other words, the basic compound patent protection will last only 20 years from patent filing and could not be extended by an SPC. This may make it less attractive to invest in necessary clinical trials for the later development of other indications.
- It is, of course, possible that subsequent indications might be the subject of separate patent protection; however, concerns were raised as to the strength of patent protection and/or RER protection in 2<sup>nd</sup> or subsequent indications in the same product. Specifically, obtaining an initial MA earlier than normal in some cases could mean that:
  - i) a SPC was not applicable (i.e. < 5 years after patent filing to MA grant would not permit an SPC),
  - ii) if an SPC is possible, it would lapse if the CMA is withdrawn (there would be considerable legal uncertainty as to whether a new SPC could be granted thereafter,

even if MA is renewed), or

iii) loss of secondary patent protection due to earlier disclosure of data in the framework of EMA's clinical trial data transparency regime.

- With regard to OME protection, it was discussed whether going forward CMAs will continue to be able to benefit from OME. With this regard, concerns were raised whether the recent Notice of the Commission on the application of Articles 3, 5 and 7 of the Orphan Drug Regulation 141/2000 could be interpreted in a way that in the future orphan designation may no more be possible for conditionally authorised products. Others did not share these concerns as the Commission Notice could not be interpreted in such a way, but would just highlight that in order to maintain orphan designation at the point of MA grant the submitted data package has to provide evidence with regard to the required significant benefit over existing treatments, and that this applies irrespective of whether it is a full or a conditional approval.
- Within the current EU framework, the currently available incentives are generally supportive of MAPPs, yet some uncertainties remain, in particular with regard to effective protection of additional, subsequently approved indications. With regard to RDP, the authorization of an additional indication may only lead to a prolongation of RDP by 1 year if certain conditions are met (see above), even if this additional indication is in a different therapeutic area and authorized under a separate, self-standing MA. This current position of the European Commission has been confirmed by the European General Court in its decisions dated 15 September 2015 (*Novartis Europharm v Commission*, T-472/12 and T-67/13). In addition, it was discussed whether additional protection of further developments might be needed, on the other side also that existing exclusivity rights might not always provide effective protection in particular on a Member State specific level and might be undermined.
- The current review of EU exclusivity rights and the practical economics of so-called cross label use (i.e. generic products being approved with a reduced label compared to its reference product due to specific patent protection (or RER) of certain indications whereas these generic products nevertheless are prescribed and reimbursed for this protected indication) could negatively impact MAPPs. Cross label use across Member States could be exacerbated under MAPPs.
- There was strong support for HTA/payer input and perspectives to be taken into consideration early in prospective planning, and that this was seen as a greater economic driver for industry and return on investment compared to IP and RER.
- There was support to map and understand the scope of necessary legal commitments of all stakeholders in MAPPs at the beginning of the process and how that might be implemented as well as the value which might be generated with these upfront commitments.
- There was a strong desire to maintain the current EU legal frameworks for IP and RER – MAPPs could operate effectively within in it. However, if in the future, the EU legislative framework is re-opened for amendment it is vital that MAPPs concept is considered

equally alongside other existing tools to ensure that any changes to exclusivity periods can be optimized prospectively.

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