



Research recommendations based on gap analysis for:

Payer requirements

Indirect comparisons (network meta-analysis)

Single arm studies

(Executive summary – document under review by consortium)

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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 is the result of the collective input from working group D1.04 and only reflects the views of the authors.

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Executive summary

ADAPT-SMART work package 1 is titled 'Evidence generation throughout the product life-cycle' and has performed a review of IMI and non-IMI projects resulting in a gap analysis (D1.02). The gap analysis identified areas along the MAPPs pathway where tools and methods for evidence generation essential for enabling a MAPPs approach were lacking or where more tools/methods development was needed. In January 2017, a multi-stakeholder workshop reviewed and discussed the initial results of the gap analysis and reached consensus on three priority topics for which work package 1 needed to formulate more in depth research recommendations for further method, process, and/or tool development. These topics were (1) payer requirements, (2) single-arm studies, and (3) indirect comparison and observational data (network meta-analysis - NMA). Although the three are presented together in this document, the three are considered as separate and distinct pieces of work. Specifically, the (1) payer requirements was considered to be of more process / procedural and harmonisation of approaches gap, whereas (2) single-arm studies and (3) indirect comparison and observation data are seen as research-specific gaps. Other proposals which emerged as part of the second review round of outputs of IMI projects are presented in a separate gap analysis (D1.02).

Topic leads with expertise on the three priority topics were identified and draft recommendations were developed. The draft recommendations were shared with the consortium in order to elicit views on their appropriateness from all project partners. The draft recommendations were also presented to the International Advisory Board for their input and review. This document reports the final research recommendations identified in the context of work package 1. These research recommendations formulate current scientific and policy questions that require more research. The results of that research could further help different stakeholder to implement the MAPPs approach. Payer requirement recommendations identify the need for an in-depth analysis of different pricing and reimbursement policies, how these deal with facilitating early access, uncertainty and product with immature data. Recommendations related to indirect comparisons call for the development of an NMA-plus approach that would allow for the integration of RCT and RWE in a systematic and dynamic way. Recommendations related to single-arm studies encompass the development of a framework for that establishes if and how effectiveness can be established using a single-arm studies.