**ADSWG-BSWG Expedited Approvals Subteam Details**

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| **Project Name:** Expedited Approvals Subteam  **Date Initiated:** January 2016  **Team Members:**  Zoran Antonijevic, Matt Austin, Robert Beckman, Carl-Fredrik Burman, Robert Campbell, Vladimir Dragalin, Larry Gould, Weili He, Qi Jiang, John Lowey, Eva Miller, David Norris, Martin Posch, Mark Trusheim, Xin Zhao  **Co-Chairs:** Zoran Antonijevic, Robert Campbell, Larry Gould  **Approximate frequency of meetings:** monthly |
| **Problem Statement** Substantial loses in patient health (and lives) occur during the years it currently takes for new therapeutic products to move from initial demonstration of efficacy and safety to the point where they are accessible to patients. Various expedited regulatory approval models or adaptive pathways to approval models have been developed worldwide. E.g., FDA’s breakthrough or accelerated, European MAPPs, Japanese Sakigake in order to address this problem. These new regulatory pathways are associated with challenges, such as (1) assuring satisfactory level of evidence, and (2) impact on various stakeholders. This subteam examines if real-time development program modifications can be made without compromising validity of inferences about therapeutic efficacy and safety. The initial focus is on products aimed at substantially improving outcomes for serious diseases that lack adequate treatment. |
| **Goal Statement**  The goal of the Expedited Approvals subteam is to contribute statistical expertise and develop solutions needed to facilitate MAPPs approaches.  Initial goals are to   * Develop and publish on statistical approaches for evidence generation relevant to Expedited Approvals and other novel development approaches across product life cycles. * Establish and promote the role for Bayesian statistics and Adaptive Design as key drivers of Expedited Approvals * Engage in the subteam patient advocacy, payer, and medical reviewer perspectives * Facilitate visibility and networking among teams and initiatives working on different aspects of efficient and ethical drug development challenge |
| **Project Scope**  Ares for the subteam to consider   * Writing of white papers, and scientific papers proposing/explaining the role of Bayesian statistics and Adaptive Design in Expedited Approvals, including:   + Promote innovative clinical trial designs that allow for more efficient and seamless knowledge generation across the product life cycle;   + Utilize Bayesian statistics and Adaptive Design to enable more frequent decision making (to enable potential adjustments to treatment population, reimbursement, and other aspects of product use)   + Further establish the role of Bayesian statistics and Adaptive Design in precision medicine; i.e., right treatment for the right patient – and in approaches to balance early patient access, public health and societal benefits * Network with other institutions/initiatives involved with Expedited Approvals * Engage with regulators worldwide * Engage with payers and with patient advocacy groups * Engage with our industry colleagues already involved with the initiative |
| **Key Deliverables / Timeline / Progress**   * 1Q2016 – Network expanded to MIT NEWDIGS/Mark Trusheim * 2Q2016 – Two papers with Subteam member authors have been submitted to CPT   + 1. Mark Trusheim, Allison Ackerman Shrier, Zoran Antonijevic, Robert A. Beckman, Robert Campbell, Cong Chen, Keith Flaherty, John Loewy, Denis Lacombe, Subha Madhavan, Joseph O’Connell, Harry Selker, Laura Esserman. Clinical Pharmacology and Therapeutics, in press, 2016.   + 2. Beckman, Robert A, Antonijevic, Zoran, Kalamegham, Rasika, and Chen, Cong. Adaptive Design for a Confirmatory Basket Trial in Multiple Tumor Types Based on a Putative Predictive Biomarker. Clinical Pharmacology and Therapeutics, in press, 2016.   2Q2017 – Complete Strategy Document for further research of statistical properties of Expedited Approvals.  4Q2018 – Complete papers outlined in the strategy document. |
| **Key References**  1. MAPPs initiative led by European Federation of Pharmaceutical Industries and Associations & European Medicines Agency: [http://efpiamapps.eu](http://efpiamapps.eu/)  2. Eichler, HG, *et al*. From adaptive licensing to adaptive pathways: delivering a flexible life-span approach to bring new drugs to patients. *Clin. Pharmacol. Ther*. 2015 Mar;97(3):234-246. PubMed PMID: 25669457  3. Eichler, HG, *et al.* Adaptive licensing: taking the next step in the evolution of drug approval. *Clin. Pharmacol. Ther.* 2012 Mar;91(3):426-37. PubMed PMID: 22336591  4. Innovative Medicines Initiative ADAPT-SMART project: <http://adaptsmart.eu>  **[to be supplemented with team member / team authored papers]** |