**Subteam: Benefit-Risk**

**Leader Information**

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**Subteam Overview**

We are a group of individuals from pharma companies, a statistical software company, and a regulatory agency, within the Bayesian Scientific Working Group (BSWG), with a special interest in benefit risk.

The benefit-risk assessment of a new medicinal product or intervention is one of the most complex tasks that sponsors, regulators, payers, physicians, and patients face. Therefore, communicating the trade-off of benefits and risks in a clear and transparent manner, using all available evidence, is critical to ensure that the best decisions are made. Several quantitative methods have been proposed in recent years that try to provide insight into this challenging problem. Bayesian inference, with its coherent approach for integrating different sources of information and uncertainty, along with its links to optimal decision theory, provides a natural framework to perform quantitative assessments of the benefit-risk trade-off.

**Objectives of the subteam:**

1. To understand how best to apply Benefit-Risk Methodologies across the Pharmaceutical Industry
2. To discuss and make recommendations on key methodological issues
3. To share examples of how Benefit-Risk has been used within pharmaceutical companies
4. To share external information including new developments around Benefit-Risk
5. To publish research in journals and books regarding these methodologies.
6. To disseminate information on research and best practices to broader scientific community as well as to engage in education efforts through conferences, workshops and seminars.

**Members**: Maria Costa (GSK), Yannis Jemiai (Cytel), Weili He (Abbvie), Yueqin Zhao (FDA/CDER), Pritibha Singh (Novartis), Carl Di Casoli (Celgene)

**How to join our cause**: Please send email to Carl Di Casoli ([cdicasoli@celgene.com](mailto:cdicasoli@celgene.com) and cdpiano27@bellsouth.net) or any of the members of the subteam.

**Completed Projects:**

**Publication:**

Costa M, He W, Jemiai Y, Zhao Y, Di Casoli C, **The Case for a Bayesian Approach to Benefit-Risk Assessment: Overview and Future Directions, Therapeutic Innovation and Regulatory Science,** Vol 51, Issue 5, 2017**,** journals.sagepub.com/doi/abs/10.1177/2168479017698190

**Publications in Progress:**

Book chapter entitled "Risk Benefit" for the book *Bayesian Methods in Pharmaceutical Research* that will be published by CRC press. Editors: Emmanuel Lesaffre, KULeuven, Belgium, Gianluca Baio, University College, London, UK, Bruno Boulanger, Arlenda, Belgium.

**Second draft submitted on 1 December 2017.**

**Presentations:**

ASA Biopharmaceutical Section Regulatory – Industry Statistics Workshop, 2017 September, Washington, D.C. USA

Bayes Pharma, Leuven, Belgium, 2016 May.

Joint Statistical Meetings (JSM) 2016, 31 July 2016, Chicago, United States

**Ongoing Projects**

1. The next project is a technical article aimed at a statistical audience which provides more granularity on specific approaches and how to apply them, as well as some case studies to share experiences of using them in practice.

Members: Maria Costa (GSK), Yannis Jemiai (Cytel), Weili He (Abbvie), Yueqin Zhao (FDA/CDER), Pritibha Singh (Novartis), Carl Di Casoli (Celgene)

**Resources**

1. Structured approach to benefit-risk assessment in drug regulatory decision-making PDUFA V implementation plan – February 2013. <http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM329758.pdf>

2. European Medicines Agency. Benefit-risk methodology project work package 3 report: Field tests. 2011; London, European Medicines Agency.

3. Colopy MW, Damaraju CV, He W, Jiang Q, Levitan BS, Ruan S, Yuan Z. Benefit-risk evaluation and decision-making: some practical insights. *DIA Ther Innov & Reg Sci.* 2006; 49:425-433.

4. Coplan PM, Noel RA, Levitan BS, Ferguson J, Mussen F. Development of a framework for enhancing the transparency, reproducibility and communication of the benefit-risk balance of medicines. *Clin Pharmacol Ther*. 2011; 89:312-315.

5. Mt-Isa S, Owens M, Robert V, Gebel M, Schacht A, Hirsch I. Structured benefit-risk assessment: a review of key publications and initiatives on frameworks and methodologies. *Pharm Stat*. 2015; 15:324-332.

6. Mt-Isa S, Hallgreen CE, Wang N, et al. on behalf of the IMI-PROTECT Benefit-Risk participants. Balancing benefit and risk of medicines: a systematic review and classification of available methodologies. *Pharmacoepidemiol Drug Saf*. 2014; 23:667-678.