HARMONY

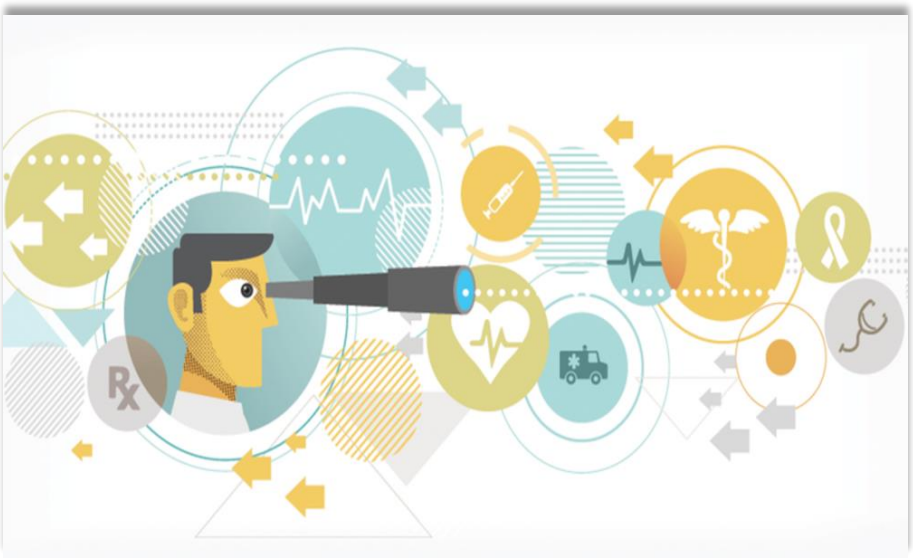
Healthcare Alliance for Resourceful Medicines Offensive against Neoplasms in Hematology

Workshop on the Definition of Meaningful Outcomes for HMs

23 - 24 November 2017, London, UK

Organized by HARMONY Work Package 2 and Work Package 6

Practical Information and Detailed Program



imi innovative medicines initiative

efpia

1. Practical Information

Meeting dates **Thursday 23rd of November and Friday 24th of November 2017**

Venue **Takeda offices**, 61 Aldwych, London

Timeframe **Monday: from 11:00 to 19:00.**
Tuesday: from 8:00 to 13:30.

Objectives

- Brainstorm, exchange and generate ideas regarding a list of outcomes relevant for all stakeholders;
- Initiate a list of outcomes by haematological malignancy and a list of standard core outcomes valid for all haematological malignancy valued by all stakeholders participating in the workshop, for later validation

Expected output of this workshop

- Generate an initial list of outcomes by haematological malignancies valued by all stakeholders participating in the workshop, for later validation;
- Generate plan of work for next 6 months for WP2 and WP6 on definition of meaningful outcomes by disease group;
- Arrange follow up meeting and frequency of communications;
- Longer term objective to develop White Paper by haematological malignancies on the definition of outcomes in 2018 as well as an overarching general outcomes definition White Paper.

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2. Detailed Programme

23 November 2017

11:00 – 11:30 Welcome and introduction

Core Outcomes for HARMONY: Why is it important, How & When we can identify them.

Welcome from Roz Eijgenhuijsen, Site head and VP & European Head, Global Regulatory Affairs.

Introduction from Work package 2 and 6 Leads:

- Aliki Taylor, Director Global Outcomes Research, Takeda, UK
- Lars Bullinger, Professor in Hematology and Oncology, Charité-Universitätsmedizin, Germany
- Jan Geissler, Founder and Managing Director of Patvocates, Germany

11:30 – 13:00 Plenary session

Perspectives on Outcomes for haematological malignancies from HARMONY members and the Stakeholders Forum.

Prof Paula Williamson, University of Liverpool

Learning from the COMET Initiative and the 'core outcome set 'and how we can integrate learnings into HARMONY.

Paula Williamson is Professor of Biostatistics. She is Director of the MRC North West Hub for Trials Methodology Research (HTMR), Director of the Clinical Trials Research Centre (CTRC), and Head of the Department of Biostatistics at the University of Liverpool. Paula chairs the University of Liverpool's Health and Biomedical Informatics Group and is a member of the Farr Institute through HeRC North. Paula co-founded and has led the COMET (Core Outcome Measures in Effectiveness Trials) Initiative since 2010. She was appointed as an NIHR Senior Investigator in 2014, gave the Bradford Hill Lecture in 2017, and is current Chair of the MRC HTMR Network.

Detailed Programme | continued

23 November 2017

Dr Simone Werner, Bayer, Germany

The clinicians, and EFPIA perspective – what are key outcomes for these stakeholders?

Dr. Werner joined the Epidemiology Division of Bayer in summer 2017 as Postdoc. She studied Molecular Medicine at the Universities of Freiburg, Germany and Uppsala, Sweden and did her PhD in Epidemiology at the German Cancer Research Center (DKFZ) in Heidelberg. Her main research focus was on the evaluation of blood- and stool-based biomarkers for early detection and screening for gastrointestinal cancers. In addition to performing statistical analyses with SAS and R, she was involved in the field work for observational studies and assisted in coordinating research with cooperation partners from academia and industry. At Bayer she will work in the field of oncology and cardiovascular diseases in the context of the IMI2 Big Data for Better Outcomes (BD4BO) projects.

Dr Katy Harrison, NICE

Clinical outcomes considered by Regulatory, HTA agencies and payers in their decision- making process.

Katy Harrison is a Scientific adviser at NICE in the Science, Policy and Research Team. Her role involves managing and delivering scientific and technical input in multiple grant funded research projects including IMI HARMONY, DO-IT and ROADMAP. Katy joined NICE in 2010 and has worked across numerous directorates having responsibility for guideline methodology, development and quality assurance covering over 50 clinical and public health topics. Prior to working at NICE, Katy was a Research Fellow in Public Health for several years, focusing on blood borne disease transmission and has over 15 years of experience as a post-doctoral researcher in molecular epidemiology and biomarker evaluation across a number of ontologies.

13:00 – 14:00 Lunch break

Detailed Programme| continued

23 November 2017

14:00 – 14:45 **Invited speaker**

Learning on key outcomes from patients' perspective from the development of the Hematologic Malignancies-Patient Reported Outcomes instrument.

Professor Sam Salek, Cardiff University

Sam Salek is Professor of Pharmacoepidemiology in the School of Life and Medical Sciences, University of Hertfordshire, UK where he leads the Public Health & Patient Safety research group. He is also the Director of the Institute for Medicine Development, Cardiff, UK, and a visiting Professor at the State of Hessen, Germany. Professor Salek is the co-founder (and currently chairs) of the Patient Engagement Special Interest Group of the International Society of Quality of Research, and is the deputy chair of the European Hematology Association Scientific Working Group for Quality of Life and Symptoms. Professor Salek is a fellow of the Royal College of Physicians, the Royal Pharmaceutical Society of Great Britain and Cardiff Medical Society. Increasingly, Professor Salek is shifting his emphasis towards the practical applications of PRO measures in clinical decision-making and policy and patient engagement in research as partners/collaborators.

14:45 – 15:00 **Panel Discussion:**
Professor Paula Williamson, Dr Katy Harrison, Dr Simone Werner and Professor Sam Salek

15:00 – 15:30 **Coffee break**

15:30 – 17:15 **Reflection session**

Objective: Defining a first common set of OUTCOMES across the different haematological malignancies.

All participants are expected to give their own perspective.

17:15 – 17:30 **Break**

17:30 – 19:00 **Food and Drink reception in Takeda offices**

Detailed Programme| continued

24 November 2017

08:00 – 08:30 **Coffee and croissants**

08:30 – 09:45 **Breakout session**

Break-out groups by haematological malignancies to discuss specific outcomes

Expected HM groups: MM, MDS and AML, CLL and NHL, ALL, Child HMs.

09:45 – 10:00 **Coffee Break**

10:00 – 11:15 **Consolidation session**

Conclusions, wrap-up of discussions from breakout sessions

Expected HM groups: MM, MDS and AML, CLL and NHL, ALL, Child HMs.

11:15 – 12:30 **Summary session**

Presentation of Results from Break-Out Sessions

Presentations from individual break-out sessions

Summary Session Final Wrap-up

- What have we learned today on important outcomes from one another?
- What are the next step toward a validation of these sets of outcomes for haematological malignancies?
- Moderator: TBD

12:30 – 13:30 **Lunch**

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About the HARMONY Alliance: Healthcare Alliance for Resourceful Medicines Offensive against Neoplasms in Hematology

The HARMONY Alliance is a European Network of Excellence for Big Data in Hematology, consisting of 51 partners. Our goal is unlocking valuable knowledge on hematologic malignancies (HMs). The HARMONY Alliance is funded through the Innovative Medicines Initiative (IMI), Europe's largest public-private initiative aiming to speed up the development of better and safer medicines for patients. HARMONY has received funding from IMI 2 Joint Undertaking and is listed under grant agreement No. 116026. This Joint Undertaking receives support from the European Union's Horizon 2020 Research and Innovation Program and the European Federation of Pharmaceutical Industries and Associations (EFPIA). IMI supports collaborative research projects and builds networks of industrial and academic experts in order to boost pharmaceutical innovation in Europe.