BIG DATA FOR BETTER OUTCOMES: SUPPORTING HEALTH CARE SYSTEM TRANSFORMATION IN EUROPE

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Summary: Large amounts of data from multiple sources have led to the opportunity of deriving health benefits through using sophisticated technologies. Regardless of the frequently cited revolution of data-driven health care, promises remain to be fulfilled. The IMI2 BD4BO programme recognises this in representative disease areas, providing a framework to guide research and invite stakeholders to discuss the future of health systems shaped by big data. The projects will impact the research environment through shared definitions and methods to avoid duplication of work, while transforming health care systems in terms of clinical operations, research and development, evidence-based personalised medicine and public health.

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Introduction

The computer age has brought about the rapid generation of large amounts of easily accessible data from variable, quickly developing, digital and non-digital sources, often referred to as "big data". Big data has immense, yet so far hardly utilised potential to improve almost all areas of human life, including health. Whether this potential can be exploited depends on the sophistication of methods and technologies available to process and use (make sense of) big data. Regardless of the frequently cited revolution of data-driven health care decision-making, there are still promises to be fulfilled. This is also true for Europe, where the fragmented legal landscape, and inconsistent public opinion inhibits the standardised collection of data, delaying or even diverting the implementation of data sharing agreements.

In the European Union (EU), the key health policy objectives are the strengthening of health system effectiveness, accessibility, resilience, quality and performance.[1] However, health care systems in Europe face significant challenges due to the high incidence of chronic diseases, ageing populations, rising cost of new drugs and widely varying health outcomes across the region.[2] Amid these challenges, the focused application of big data has the

potential to enable health care systems to effectively transform towards value-based health care.[3]

The Innovative Medicines Initiative 2 (IMI2), Europe's largest public-private initiative (a joint undertaking between the EU and the European Federation of Pharmaceutical Industries and Associations [EFPIA]), recognised this phenomenon through the recently launched Big Data for Better Outcomes (BD4BO) programme, which aims to catalyse and support the evolution towards outcomes-focused, sustainable health care systems in Europe. In order to reach its goal, it seeks to exploit the opportunities offered by big and deep data sources in a few representative disease areas, to put together a methodological framework to guide big data research and to invite a wide range of stakeholders to discuss the future of health systems shaped by big data.

The programme explicitly differentiates itself from previous initiatives through the high level of stakeholder engagement in leveraging existing databases and collaborations to reach its aim. It brings together the key stakeholders, including patients, payers, providers, regulators, academic researchers and health care policy makers that are required to create the synergies in big data policies needed to shift to value-based health care. The effective use of big data resulting from such synergies and the insights gained from projects launched under the BD4BO programme has the potential to transform health care systems in terms of clinical operations, research and development, evidence-based personalised medicine and public health. For example, better access to data may improve comparative effectiveness research, allowing providers to make more clinically relevant decisions and identify cost-effective ways to diagnose and treat patients.[4] Such improvements may enable health care systems to derive value by lowering expenditure and improving patient outcomes.

In more operative terms, the BD4BO programme provides a platform and resources for defining and developing enablers to enhance the transparency of

outcomes. The perception of health care stakeholders on outcomes are different, an issue that can only be tackled with a holistic approach by including as many perspectives as possible, continuously being adapted to the context of the disease, patient population or therapeutic field. The programme addresses the following key enablers: definition of outcome metrics; protocols, processes and tools to access high quality data; methodologies and analytics to drive improvements; digital and other solutions that increase patient engagement in the efforts for better outcomes. Each project launched under the BD4BO programme will make concentrated efforts to advance common outcomes definitions, use of more reliable data and related analytical methods with increased patient involvement.

With the BD4BO programme focusing on filling the gaps in availability of standard sets of outcomes, combining different data sources, identifying best practices, and increasing patients' engagement in their care, project deliverables will allow stakeholders to gain more powerful insights to improve health care. These features of the programme and the cooperation framework of IMI seek to promote an efficient dialogue between projects and with other similar non-IMI initiatives. Upon completion of the programme, the realisation of each disease project's aims will contribute to an organic transformation of research and clinical practice in health care systems.

Focusing on disease, population and therapeutic area

Currently, three disease specific BD4BO projects have been launched within IMI2, focusing on Alzheimer's disease (AD), haematological malignancies and cardiovascular diseases. All of these projects attempt to use the toolbox of big data, but with a focus on somewhat different aspects of the selected diseases in relation to health outcomes.

ROADMAP

The first BD4BO disease specific topic is titled "Real World Outcomes Across the AD Spectrum (ROADS) to Better Care". The first phase of this topic provides an important initial step for identification

and integration of AD-relevant real world datasets that are suitable for answering questions about the natural history, cost-effectiveness, and clinical utility of new and innovative treatment interventions across the entire spectrum of the disease. The proposed pilot project under this topic (titled "ROADMAP") will align outcomes and methods to develop an approach within existing data systems to efficiently enable initiation, maintenance, and evaluation of the right treatment to the right patient at the right time in health care systems. Engagement with health technology assessment (HTA)/national health care bodies, regulators, and patient advocacy groups will ensure that proposals for future prospective data collection efforts are relevant to access and reimbursement questions. The initial results produced by ROADMAP will be critical to ensure that the work proposed in the second phase of the project is realistic in scope, relevant to stakeholder needs, and complementary to ongoing IMI2 and other EU collaborations for better patient outcomes from pre-clinical/early stages of AD through all dementia stages.

HARMONY

The second BD4BO topic is titled "Development of an outcomes-focused data platform to empower policy makers and clinicians to optimise care for patients with haematological malignancies". The proposed project (titled "HARMONY") aims to deliver a series of benefits for patients, health care providers and manufacturers within this disease area. Due to the rarity of the conditions and the diverse health care practice across the EU, current health care systems are challenged with several issues. There is limited data on haematological malignancies that are comparable, making it difficult for policy makers to establish benchmarks such as risk/benefit ratios and payers to accurately make reimbursement decisions on life prolonging treatment options. In addition, the lack of data is forcing clinicians to make decisions based on short-term surrogate data that is often not comparable, which may result in patients not getting the right treatment at the right time. Further, there is a lack of definition and alignment on outcomes that is relevant to all stakeholders within this disease area.

HARMONY aims to use 'big data' to deliver information that will help to improve the care of patients with certain haematological malignancies. Specifically, the project will collect, integrate and analyse anonymous patient data from a number of high quality sources. This will help to define clinical endpoints and outcomes for these diseases that are recognised by all key stakeholders. Meanwhile the project's data sharing platform will facilitate and improve decision-making for policy makers and clinicians alike to help them to give the right treatment to the right patient at the right time. Key to this is the collaboration and firm commitment of industry, HTA, payers and other stakeholder experts plus the input of patients. Harmonising data collection and subsequent data flows will rely on the collaboration between researchers, pharmaceutical companies, and academics, in order to advance structures that already exist and which include expert, pan-European groups. Sources certainly need to be of the highest quality throughout and thorough assessment to identify and utilise optimum data is key if the knowledge currently being gathered is to be put to the best possible use. The project will be supported by a robust communication strategy to inform all stakeholders in and outside the project about developments and results, as well as issues that need to be addressed in order to achieve the project's goals.

BigData@Heart

The most recent BD4BO topic is titled "Increase access and use of high quality data to improve clinical outcomes in heart failure (HF), atrial fibrillation (AF), and acute coronary syndrome (ACS) patients". The expected impact of the proposed project (titled *"BigData@Heart"*) is better and safer treatment paradigms for patients with AF, HF, and ACS. In more direct terms, the project is expected to improve understanding of the risks of serious outcomes in these patients compared to the general population. The existing knowledge should be further improved on how these patients are treated in the real world and what affects outcomes with more efficient surveillance of safety and effectiveness in real world settings. Further expectations include: improving information on the importance

of adherence to treatment, the role of risk factors, comorbidities, genetics and lifestyles; improving awareness of quality of life aspects that are important for patients; evaluation and testing of tools that may be useful for predictive analytics and surrogate markers for cardiovascular outcomes; developing strategies to use these predictive analytic tools and surrogate markers to improve clinical care pathways and support innovative drug development that provide relevant improvement of outcomes that are important for patients.

Prostate cancer

Another further topic on prostate cancer, with the primary objective of increasing the body of evidence to improve prostate cancer outcomes, will aim to identify and broaden the relevant outcome measures: epidemiological, clinical, economic, and patient reported outcomes. This includes screening, diagnosis and predictive factors that may have an impact on these measures (including complications and adverse effects) across all stages of disease through collection and analysis of available data.

Impact on research environment for big data

The BD4BO programme ambition is to invest in four key enablers that will support the evolution towards outcomes-focused and sustainable health care systems. In addition, the BD4BO programme will endeavour to impact on the use of big data in the research environment. Researchers will have the opportunity to benefit from the improved quality of data sets in these specific disease areas, stakeholder agreement of clinical outcomes and endpoints to improve health care services and accelerate the development and availability of innovative medicines, and a wealth of patient reported outcome measures from digital solutions for data mining to develop preventative and personalised approaches to patient care.

The BD4BO has a coordination and support action (CSA) project (titled *DO➔IT*) that will facilitate programme coordination of current and future projects, develop a repository for sharing knowledge and insight for use by health

care stakeholders, lead communication and engagement, and address data privacy issues in the development of informed consent forms for use within clinical, non-clinical and biobanking research. As a European-wide project, the value of the CSA project is that it will harmonise activities and the acceptability of outcome measures, and leverage the breadth of experience in member states from different stakeholders towards sustainable health care systems and patient access to innovative and safer medicines.

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