

## Consultation Paper

### Big Data for Better Outcomes - concept for an IMI2 programme

The objective of this document is to seek stakeholders' input on the scope and desired impact of a proposed IMI2 programme focused on maximising the potential of big data in healthcare (provisional launch date: September 2015):

- Are the objectives and scope relevant and clear?
- What therapeutic areas (or types of products) would benefit most from such an approach?
- What questions related to measuring health outcomes would benefit most from a multi-stakeholder collaboration in IMI?
- Whose participation and what contribution would be key for success?

Feedback on this concept paper will be sought through:

- A dedicated session during the IMI2 [Stakeholder Forum](#) of 15 June 2015
- Written comments and suggestions can also be sent until 25 June 2015 to [health-outcomes@efpia.eu](mailto:health-outcomes@efpia.eu). Individual comments will not be made public, but a FAQ response may be considered.

#### ***1. Background: challenges and opportunities***

Europe's health care systems face significant challenges such as managing ageing populations, rising incidence of chronic diseases and intense budgetary pressures. Furthermore, there is a growing body of evidence demonstrating that health outcomes<sup>1</sup> vary widely, within and across healthcare systems, regions and countries.

In parallel, R&D pipelines are delivering new healthcare solutions, which will contribute to significantly improving management of acute and chronic diseases. While they will inevitably have a short term impact on disease management budgets, they will deliver significant savings for social security and healthcare budgets in medium and long term.

The evolution from the current commodity approach to products and services towards a focus on health care value, including prevention, may help reconcile the tension between short-term budget imperatives and long-term sustainability for patients and societal benefits, and secure continued access to innovative healthcare products and services. It will also prepare the ground for the more extensive availability of new categories of personalised products and services such as personalised medicines and advanced therapies which are not necessarily well served by existing access/delivery schemes.

Increase in availability of large health and population data sets combined with growing analytic capabilities provide opportunities to help health care systems understand patient outcomes achieved, with a view to driving improvement in the value of health care delivered. However, many of today's investments in large datasets and real world evidence are uncoordinated, duplicative, and inefficient due to the lack of agreement on standardized outcomes that matter to patients, appropriate data sources and methods to collect, analyze, report and use/interpret the data.

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<sup>1</sup> The results people care about most when seeking treatment, including functional improvement and the ability to live normal, productive lives (<https://www.ichom.org/>)

## ***2. A programme on Big Data for Better Outcomes***

EFPIA members propose to set up an IMI2 programme dedicated to maximising the potential of big data in the context of European healthcare. The Big Data for Better Outcomes programme would aim to catalyse and support the evolution towards outcomes-focused and sustainable healthcare systems in Europe, exploiting the opportunities offered by big and deep data sources by generating a body of evidence that will inform policy debates.

This umbrella programme will provide a platform and resources for defining and developing together with payers, patients, physicians, regulators, academic researchers, healthcare decision makers, etc. enablers of the outcomes transparency evolution. These are:

- Definition of outcome metrics,
- Protocols, processes and tools to access high quality data,
- Methodologies and analytics to drive improved efficiency, and
- Digital and other solutions that increase patient engagement

### 2.1. Design sets of standard outcomes and demonstrate usefulness (outcomes definition)

Projects will aim at achieving a common agreement across stakeholders on outcomes that matter to patients (as opposed to operational metrics or intermediate results) for medical conditions with high economic and health burden, and the relevance of those outcomes for different usages (e.g. reimbursement, effectiveness assessment, etc.). The focus will be to build on current efforts and fill gaps in availability of standards sets of outcomes.

### 2.2. Increase access to high quality outcomes data

Projects will focus on access to and capture of high quality, comparable outcomes data as well as their integration and analysis. The optimal result would be a data partnership, where users are able to download aggregated data or reports assuming they contribute to the partnership with their own data. A combination of different data sources will enable stakeholders to distil more powerful insights to improve healthcare practice and outcomes. Strong leverage of current IMI projects EMIF<sup>2</sup> and EHR4CR<sup>3</sup> is expected for projects addressing this theme.

### 2.3. Use data to improve the efficiency of healthcare delivery

Projects will address the need to better understand underlying reasons for outcome and cost variation and to develop analytic tools to quickly extract insights from information that would drive clinical improvements and decision support. Potential applications will be focused on identification of best clinical practices, best practices to increase patients' adherence to treatments, and prediction of outcomes based on risk factors, symptoms, genomic data, etc., in order to target treatment to patients who would benefit the most.

### 2.4. Digital and other solutions that increase patient engagement

Projects will address the need to increase patients' engagement in their care to drive better outcomes through data gathered with digital solutions. There are many healthcare digital solutions on the market, but currently patients' uptake is limited, solutions fail to have a holistic view of the patient and there are still gaps to address patients' and physicians' needs.

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<sup>2</sup> European Medical Information Framework

<sup>3</sup> Electronic Health Records for Clinical Research

### 3. Big Data for Better Outcomes: Programme structure

The programme is structured to provide strategic guidance, project management and expert support (legal, ethical, regulatory) across all projects under the scope. Individual projects will address one or more of the enablers. A central coordination structure will support overall consistency, integration and dissemination of results. It will also promote dialogue between projects and with other similar non- IMI initiatives.

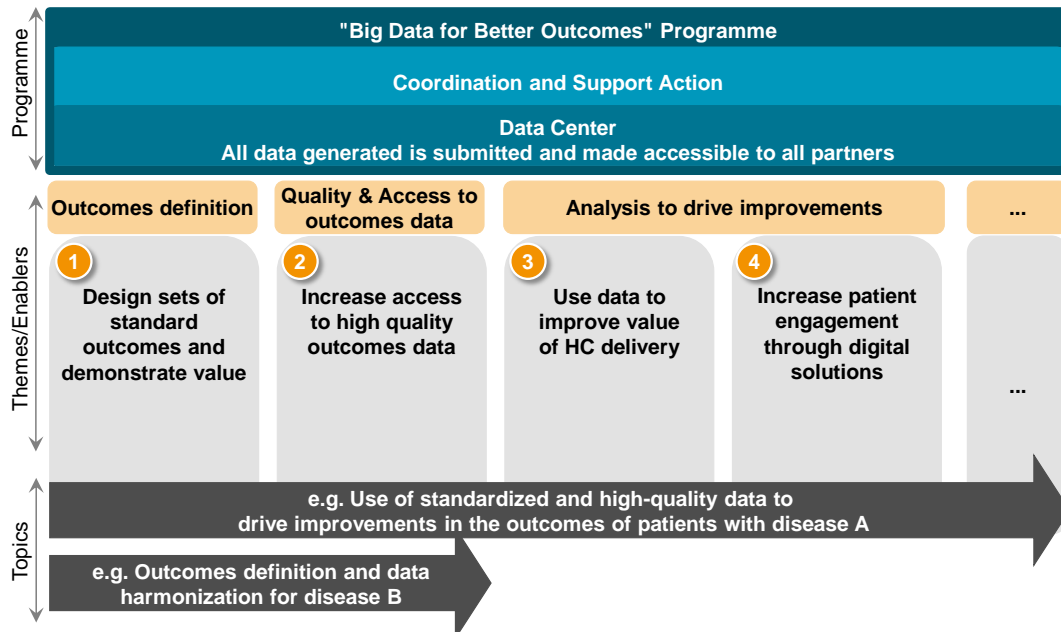


Figure 1: Programme structure, themes/enablers and topics

### 4. Private-public collaboration for Big Data for Better Outcomes programme

A public-private collaboration is an ideal model to achieve improved outcomes by using big data, since it leverages the expertise and capabilities of private companies and public entities in addition to their abilities to jointly drive innovation, transformation and align stakeholders.

The IMI framework makes it possible for any non-industrial, industrial, governmental or non-governmental organisation to engage as partner in the project. Public partners - i.e. all organisations in Europe except companies with turnover above €500 mio are eligible for IMI grants that enable participation in public private consortia. Even though not all organisations are eligible for funding, many organisations can participate in the projects. Various stakeholders can also engage in an advisory capacity in scientific and advisory boards of individual projects. Finally, any organisation can submit an idea for a project using the EFPIA submission form available at: <http://imi.efpia.eu/imi2/create-your-imi2>

### 5. Examples of topics/projects considered under the Big Data for Better Outcomes umbrella

#### 5.1 Alzheimer's disease (AD)

The objective is to increase the understanding of outcomes across the Alzheimer's disease spectrum to improve care. Topic will be launched in 2 phases: phase 1 will set the baseline (outcome definition, data strategy) and phase 2 will cover strategy implementation for improved value.

Key deliverables will include:

- Outcome definition for AD and alignment across healthcare systems stakeholders on relevance of those outcomes for different uses

- Development of a strategy for integration of real world evidence sources for AD, including mapping of existing data sources and identification of gaps
- Analysis and development of analytical tools using retrospective data that will enable improving value delivered: development of transformation algorithms to compare results from existing AD assessments, prediction of late-stage outcomes based on intermediate endpoints and identified drivers of outcome variation, comparison of disease modeling methods, etc.

## 5.2. Hematologic malignancies

The objective is to improve quantity and quality of data that can be used for the evaluation of healthcare value delivered by different treatment options, including innovative advanced therapies, so that each patient can be given the most appropriate treatment.

Key deliverables will include:

- Outcome definition for specific types of hematologic malignancies (ALL, DLBCL, follicular lymphoma) and alignment across healthcare systems stakeholders on relevance of those outcomes for different uses
- Development of an outcomes-focused data platform for hematologic malignancies
- Establishment of a pan-EU framework to value and evaluate health outcomes achieved with cell and gene therapies

## 5.3 Other areas under consideration:

- Disease-specific topics that will address one or several enablers: cardiovascular (heart failure, atrial fibrillation, coronary heart disease / familial hypercholesterolemia), multiple sclerosis, rare diseases, psoriasis, solid-state tumours;
- Enabler topic to address theme 2 (quality and access to outcomes data): development of a financially sustainable European Distributed Data Network which, through the adoption of a common data model across contributing data providers and countries, will enable aggregated analysis to identify opportunities to improve healthcare value (building on existing IMI projects such as EMIF<sup>4</sup> or EHR4CR<sup>5</sup>)

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<sup>4</sup> European Medical Information Framework [www.emif.eu](http://www.emif.eu)

<sup>5</sup> Electronic Health Record for Clinical Research <http://www.ehr4cr.eu/>