EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

Health systems, medical products and innovation
Medicines: policy, authorisation and monitoring

STAMP 9/41

STAMP Commission Expert Group
8 June 2018

Subject: Activities related to real world data

Agenda item 4

During the 4th meeting of STAMP in March 2016 the topic of real world data was discussed. At the time it was noted that other initiatives were being taken forward, in particular by the European Medicines Agency (EMA) and the Heads of Medicines Agencies (HMA) and it was agreed that the group would return to the issue at a later stage.[1]

On 25 April 2018 the European Commission published the *Communication on enabling the digital transformation of health and care in the Digital Single Market; empowering citizens and building a healthier society*[2] which identified three priorities:

- Citizens' secure access to their health data, also across borders;
- Better data to advance research disease prevention and personalised health and care;
- Digital tools for citizen empowerment and person-centred care.

The aim of the second priority is to share health resources, for example data, infrastructure, expertise, to allow targeted and faster research, diagnosis and treatment. This priority foresees a pilot on real world data (data collected outside formal clinical trials).

The 9th meeting of STAMP will provide the opportunity to have an overview of the activities that have been taken forward through the initiatives of the EMA, HMA and in research funded by the Commission, including the Innovative Medicines Initiative (IMI). In addition, we would like to understand the further action that will take place through these initiatives and the areas where other EU coordinated action could provide added-value.

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[1] See STAMP webpage https://ec.europa.eu/health/documents/pharmaceutical-committee/stamp_en for the background paper (STAMP 4/4 rev.1), related presentations and the summary record of the meeting.
[2] See: https://ec.europa.eu/digital-single-market/en/european-policy-ehealth For the Commission Communication see: https://ec.europa.eu/digital-single-market/en/news/communication-enabling-digital-transformation-health-and-care-digital-single-market-empowering and the accompanying staff working document: https://ec.europa.eu/digital-single-market/en/news/staff-working-document-enabling-digital-transformation-health-and-care-digital-single-market

In 2014 the EMA set up a cross-committee task force on registries, comprising representatives from EMA scientific committees and working parties and experts from national competent authorities[3]. In September 2015 the **EMA initiative for patient registries** was launched, exploring ways of expanding the use of patient registries and seeking to create an EU-wide framework on patient registries in the field of medicines, facilitating collaboration between:

- registry coordinators, such as physicians' associations, patients' associations, academic institutions or national agencies responsible for overseeing healthcare services;
- potential users of registry data, such as medicines regulators and pharmaceutical companies.

To better understand the challenges and barriers to collaboration between stakeholders, the EMA held a stakeholder workshop in October 2016[4]. In addition, the EMA has hosted stakeholder workshops on patient registries - cystic fibrosis[5], multiple sclerosis[6], chimeric antigen receptor (CAR) T-cell therapy[7] and a fourth workshop on haemophilia registries[8] is planned. In the next steps, the task force will work with stakeholders to facilitate the development of **implementation plans** to support delivery of the recommendations.

In March 2017 the **HMA / EMA Joint Big Data Task Force**[9] was established to explore how regulators can use big data, which includes RWD, to support research, innovation and robust medicines development. In May 2018 the task force organised a stakeholder workshop with a view to identifying solutions for big data challenges[10]. The task force is planning to make recommendations in November 2018. The work of the task force complements ongoing work at the EMA around characterising better the European picture of electronic health databases and considering mechanisms in which datasets may be harmonised to both facilitate and accelerate regulatory access e.g. common data models.

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[3] For information on the EMA activities on patients registries see: http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000658.jsp&murl=menus/regulations/regulations.jsp&mid=WC0b01ac0580961211

[4] Stakeholder workshop: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2016/08/event_detail_001315.jsp&mid=WC0b01ac058004d5c3

[5] Stakeholder workshop on cystic fibrosis registries: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2017/10/event_detail_001522.jsp&mid=WC0b01ac058004d5c3

[6] Stakeholder workshop on multiple sclerosis registries: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2017/10/event_detail_001523.jsp&mid=WC0b01ac058004d5c3

[7] Stakeholder workshop on patient registries in chimeric antigen receptor (CAR) T-cell therapy: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2018/01/event_detail_001569.jsp&mid=WC0b01ac058004d5c3

[8] Stakeholder workshop on haemophilia registries: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2018/03/event_detail_001628.jsp&mid=WC0b01ac058004d5c3

[9] See HMA website: http://www.hma.eu/509.html

[10] HMA / EMA Joint Big Data Task Force stakeholder workshop: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/events/2018/04/event_detail_001630.jsp&mid=WC0b01ac058004d5c3

There has been a great deal of **research** related to real world data and the European Commission has funded many projects. A more recent example is IMPACT HTA[11] which is investigating statistical methods, and tools to combine and use randomised clinical trial and real world data in the economic evaluation of medicines.

The **research funded through the IMI** which have a main focus on real world data include the GETREAL[12] project which was completed last year. Through this project new tools and resources for incorporating real-life data into medicine development have been developed. BD4BO (Big Data for Better Outcomes)[13] has been organised to generate knowledge, data and methodologies needed to support the transition towards more outcomes-focused, sustainable healthcare systems in Europe. The programme focuses on Alzheimer's disease, heart disease, and certain cancers.

Points for consideration of the group:

- What is your experience of collection and use of real world data? Are there any outstanding challenges or issues that are not being addressed through ongoing research activities and other initiatives (e.g. EMA, HMA)?
- Do you see synergies with the needs for real world data from other public actors for different purposes (e.g. monitoring safety, economic evaluation, pricing and reimbursement decisions)?
- What kind of real world data sources (e.g. patient registries, clinical trials, medical health records, health insurance databases) and methodologies do you believe to be most promising?
- Are there specific areas (e.g. therapeutic) on which collaboration at EU level would best focus first, allowing to demonstrate the benefit of the use of real world data for different purposes?

[11] IMPACT HTA (Improved methods and actionable tools for enhancing health technology assessment) https://cordis.europa.eu/project/rcn/213045_en.html
[12] GETREAL: https://www.imi.europa.eu/projects-results/project-factsheets/getreal
[13] BD4BO https://www.imi.europa.eu/projects-results/project-factsheets/bd4bo