

15 February 2021
EMA/48625/2021

Big Data Steering Group (BDSG): 2020 report

This report provides a summary of the key activities and achievements of the BDSG in 2020. The report documents significant progress in the transformation to more data-driven regulation in line with the [Network Strategy to 2025](#). This progress has been delivered despite the significant challenges posed by the COVID-19 pandemic.

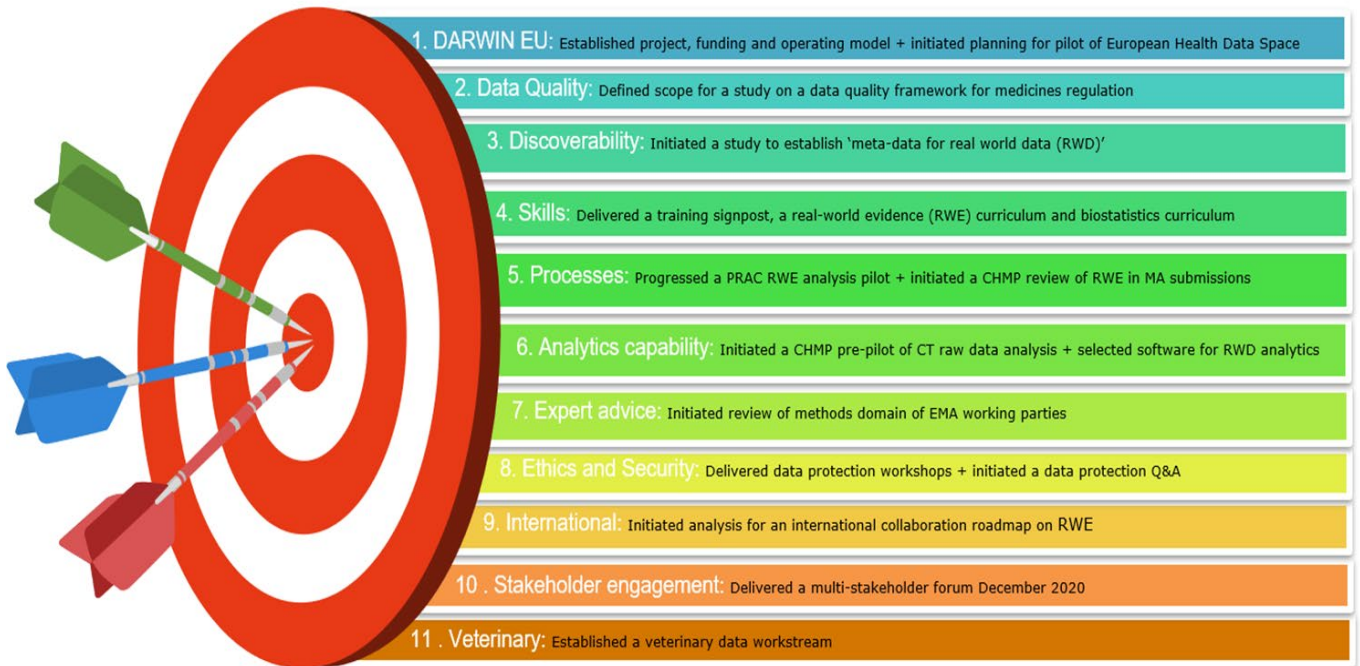
The [Phase II report of the HMA-EMA joint Big Data Task Force](#) (BDTF) and the proposal to establish a joint BDSG (superseding the Big Data Task Force) were endorsed by the Heads of Medicines Agencies (HMA) in November 2019 and EMA Management Board (EMA MB) in December 2019. The BDSG was established to advise HMA and EMA MB on the recommendations of the Big Data Task Force, covering human and veterinary medicines. Annex 1 provides the 10-priority recommendations and Annex 2 the BDSG workplan, against which the 2020 activities and achievements can be compared. The full mandate of BDSG can be found [here](#).

The BDSG kick off meeting took place in May 2020 and the BDSG adopted its [workplan](#) in July 2020, setting its priorities for 2020 and 2021. In 2020 the BDSG met 7 times virtually and the agreed actions have been implemented in line with the workplan.

2020 highlights for the priority recommendations

Figure 1 below provides a representation of the key BDSG highlights presented in the context of the priority recommendations.

Big Data Steering Group: 2020 Report



Description of 2020 Highlights

Recommendation 1: deliver a sustainable platform to access and analyse healthcare data from across the EU (Data Analysis and Real-World Interrogation Network – DARWIN EU)

The Data Analytics and Real-World Interrogation Network (DARWIN-EU) is the first and most ambitious recommendation of the BDTF. Significant progress was achieved in 2020 moving from an aspirational recommendation to an established project supported by relevant EU policy initiatives (EC Digital and Data strategies, European Health Data Space and EU Network Strategy to 2025), a proposal for a more explicit legal basis for access to and analysis of healthcare data in medicines regulation (Health Union proposal) which also included resourcing for critical project activities.

The BDTF recommendations foresaw EMA fees as the source of long-term funding for the operational phase of DARWIN EU. In 2020 EMA worked with the European Commission (EC) services to support the impact assessment for a revised EMA fees regulation to ensure sustainable funding at no direct cost to the public purse.

As DARWIN EU will act as a pathfinder initiative and use case for the proposed European Health Data Space (EHDS), in Q4 2020 EMA actively collaborated with EC to scope and plan a pilot to demonstrate the potential for the EHDS.

The high-level design of the future DARWIN EU network has been presented to the BDSG and the initial regulatory use cases continue to be expanded in consultation with the various stakeholders that will benefit from the DARWIN EU network, including the EMA scientific committees. Informed by consultation with international regulators and a market survey, BDSG has reviewed a delivery and operating model and a high-level project plan. This plan foresees establishment and piloting of DARWIN EU through 2021-2022 with the operational phase starting in 2023.

DARWIN EU will leverage the deliverables from the top 10 Big Data recommendations and the work from the BDSG will be critical to ensure that:

- Real world data is discoverable and of known quality and representativeness;
- The EU Network has knowledge and experience in data science, methods and analytics to use DARWIN EU and support regulatory decision making;
- A Big Data learning initiative allows DARWIN EU to continue to learn and evolve rapidly to be able to address new regulatory needs, including preparing for and responding to future health crises;
- DARWIN EU operates within, complies with and leverages the benefits of the EHDS.

Access to and analysis of real-world data will complement evidence for randomised clinical trials and support the development, authorisation and supervision of medicines for patients.

Recommendation 2: Establish an EU framework for data quality and representativeness

In 2020, BDSG was consulted on a proposal for an external study to analyse existing data quality initiatives, discuss data quality with a wide range of stakeholders and to deliver a draft EU framework for data quality and representativeness. The study will be initiated in 2021.

By understanding data quality, the selection of data and interpretation of study results is informed, and the evidentiary value of studies can be judged.

Recommendation 3: Enable data discoverability

In 2020, BDSG was consulted on a proposed implementation approach for data discoverability. This led to the initiation of an external study to agree 'meta-data for real world data'. This study will establish criteria to include data source in the EU Resource database, provide a definition of metadata and pilot the collection of such metadata.

Agreement on metadata to describe and identify data sets will enable a data discoverability roadmap including a publicly available catalogue of real-world datasets.

Recommendation 4: Develop EU Network skills in Big Data

Significant progress has been made through the EU Network Training Centre on the delivery of training curricula to upgrade the skills available to the EU Regulatory Network.

In 2020, a curriculum on Pharmacoepidemiology/real-world evidence and a second on Biostatistics & Clinical Trial Methodology were finalised and a 'Big Data Training Signpost' tool was made available for

staff in the National Competent Authorities and EMA and published on the EU NTC platform. Work on a third curriculum 'Data Literacy' was also initiated in 2020 and its finalisation is planned for 2021.

The BDSG was also consulted on a survey 'Big Data skills in the network', aiming to validate the scope of the three training curricula, to identify gaps and training needs, and to obtain feedback on the training priority areas within the EU Network. The survey will be conducted in February 2021.

Training will support the development of an expert workforce able to advise on and interpret big data.

Recommendation 5: Strengthen EU Network processes for Big Data submissions

In 2020, the BDSG was consulted on how to further increase the transparency of observational research through the public registration of observational studies facilitated by enhancement of the EU Post-Authorisation Studies Register.

Also in 2020, PRAC piloted rapid analysis of real-world data to support its decision-making. The preliminary pilot results were presented to the BDSG. These showed that rapid analysis is possible with simple assessment questions (e.g. incidence or prevalence of disease, medicine exposure or event frequency), while the availability of data for the medicine and event of interest in the datasets to which EMA currently has access was a key limiting factor that would need to be considered in the next phase (and will be addressed through the establishment of DARWIN EU).

Discussions took place with other EMA scientific committees to scope possible pilots that could be launched in 2021 - 2022. The results of these pilots will inform the improvement of regulatory processes to incorporate real-world data.

In order to develop processes and inform guidance for industry the BDSG was consulted on the interim results of a review of RWE in Marketing Authorisation submissions and line extensions submitted to EMA in 2018 and 2019. This retrospective review aims to identify, classify and characterise RWE submissions and correlate these with the conclusions of the assessment. The final results of the review will be available in 2021 and will inform a discussion with stakeholders on a prospective 'learnings initiative'. A BDSG sponsored workshop with stakeholders on a learnings initiative will be held in 2021.

Finally, the BDSG reviewed the recent EMA experience with medicinal products in combination with digital health / medical devices, that have undergone a CHMP review or were submitted for CHMP Scientific Advice or to the EMA Innovation Task Force, as well as the early learnings with regards to the quality and clinical assessment of such applications. The group supported the need for guidance on the content of submissions and in the questions that are relevant for EMA review (compared to questions for medical device regulators). The support companies developing such products a ['O&A Qualification of methodologies based on digital technology'](#) was published on EMA website.

Learning from data in regulatory submission will inform process improvement, guidance development and the clarification of evidentiary value.

Recommendation 6: Build EU Network capability to analyse Big Data

Information Technology is a critical component of several aspects of the BDSG workplan. In 2020, the BDSG held a first discussion on technology requirements including key principles to guide the

development and implementation of technology underpinning the EU Network capability to analyse Big Data.

EMA acquired rapid analytics software for the analysis of real-world data to support targeted committee assessments. Specialised software for real-world data analysis holds promise to increase the number of analyses that can be performed and to speed up analysis.

Finally, the BDSG was consulted on the approach to work with patient level data and supported the launch of a pre-pilot with CHMP on one product to analyse patient level data from clinical trials to inform future policy, process and technology choices.

Technology is a key enabler to access and analyse big data and its informed selection and use will accelerate transformation to fully data-driven regulation.

Recommendation 7: Modernise the delivery of expert advice

In 2020, under the EMA Management Board mandated review of the Agency's experts working parties and groups, review of the working parties' methods domain was initiated. The review has the objectives to:

- Strive for methodological excellence across the network to ensure best practice in assessment and advice procedures.
- Deliver appropriate guidance documents to support the development and licensure of medicines, based on experience gained assessing products and providing scientific advice on methodology related aspects. The specific focus is on new and emerging clinical study designs and a changing clinical development landscape.
- Raise the awareness, improve the visibility and strengthen the position of methodology in the regulatory procedures and actions, and ensure the appropriate development of junior assessors across the EU network.
- Ensure the EU is recognised globally as a region of excellence in all areas of methodology and to provide a leading voice in international collaboration efforts.

Expert advice including on advanced analytics, real world evidence and 'omics will empower assessment and decision-making by regulatory committees.

Recommendation 8: Ensure data are managed and analysed within a secure and ethical governance framework

To strengthen the secure and ethical governance of healthcare data, the BDSG contributed to workshops on data protection and data governance with stakeholders. Two workshops were held:

- One with EMA's Patients and Consumers Working Party and Healthcare Professionals Working Party.
- One with industry and academia.

Summary reports of both workshops are available on the Big Data website ([link](#)).

Guided by the BDSG and informed by the feedback from stakeholder in 2020, EMA initiated the drafting of a Question and Answer (Q&A) document on data protection in the context of secondary use of healthcare data. This Q&A should be finalized and made public in the second quarter of 2021.

Secure and ethical data governance is an enabler for secondary use of healthcare data and the work of the BDSG seeks to support stakeholders to navigate and comply.

Recommendation 9: Collaborate with international initiatives on Big Data

In 2020, the BDSG was consulted on an outline of a Data Standards initiative which aims to create a data standard's strategy for the EU Regulatory Network. This initiative will create an inventory of all applicable data standards currently in use or being developed, and foster interaction and listen to standards development organisations and stakeholders on needs for data standards. In Q4 2020 EMA contracted a study on a Data Standards Strategy & Roadmap.

In 2020, within the EMA-FDA-Health Canada Cluster on Big Data, a discussion was initiated on opportunities for collaboration on real world data and evidence. This discussion should inform a planned technical meeting with international regulators in 2021.

Convergence with international partners on standards and guidelines will leverage best expertise and will minimise burden on stakeholders.

Recommendation 10: Create an EU Big Data 'stakeholder implementation forum'

The first Big Data Stakeholder Forum was held virtually in December 2020. More than 250 participants took part including representatives of patient, healthcare professional, regulator, pharmaceutical industry, medical device and information technology organisations. The forum aimed to give stakeholders an opportunity to provide feedback regarding the Big Data priority recommendations and provide their perspectives on implementation. During the forum speakers from different stakeholder groups presented what they considered priorities, opportunities and risks, and areas for collaboration and engagement. The summary report of the forum can be accessed [here](#).

Listening to stakeholders and leveraging their work will optimise and maximise transformation to data-driven regulation.

Recommendation 11: Veterinary recommendations

The final BDTF recommendations did not cover veterinary medicines. However, EMA MB and the HMA recommended that veterinary medicines be included in the scope of work of the BDSG. Therefore, in 2020 the BDSG was consulted on the applicability of the recommendations to the veterinary domain and on an analysis of the current veterinary data landscape. BDSG endorsed a proposal for a veterinary stakeholder workshop on data in 2021 and a dedicated veterinary workstream.

Synergies exist in the use of data between the human and veterinary domains that can catalyse our transformation.

Other BDSG activities


The European Commission (EC) has provided regular updates to the BDSG on the various EC initiatives including the European Data Strategy, the European Health Data Space and Pharmaceutical Strategy for Europe.

The BDSG was regularly consulted on the finalisation of the European Medicines Regulatory Network Strategy to 2025 during 2020 BDSG meetings.

Annex 1 - Priority Recommendations of the HMA-EMA joint Big Data Task Force



Annex 2

 THE HMA-EMA JOINT BIG DATA STEERING GROUP WORKPLAN

