HMA-EMA Big Data Task Force: Recommendations and workplan

ENCePP in the Time of Covid – Meeting 20th November 2020

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Outline of presentation

- The Big Data Task Force mandate and reports
- Top 10 key recommendations
- Implementing the recommendations - BDSG workplan and achievements to date
- Why it is important for the ENCePP community

Big Data Task Force – the 'Why'

Challenge: capitalise on the promise of novel new datasets of unknown quality and provenance and still reach a robust position on the benefit risk of a medicine.

Big Data Task Force – the work done

Phase I report: endorsed by HMA & EMA management board end 2018:

- Characterisation of data sources
- Survey of NCAs and industry
- Set of core recommendations
- Annexes including reports from 7 subgroups (now published)

Phase II report

- Published 20 January 2020:
  - regulatory prioritisation and implementation of recommendations

Looking forward

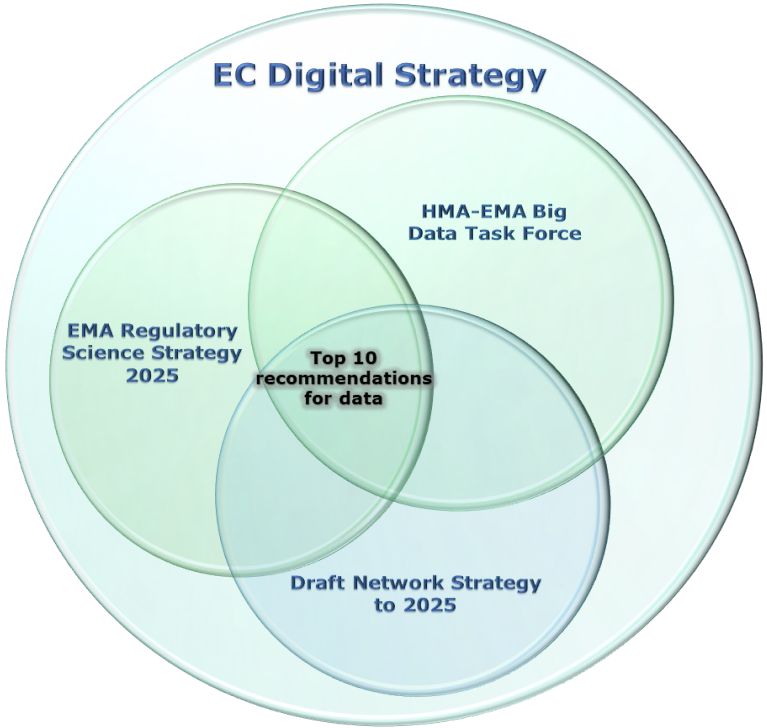
"By delivering the vision of a regulatory system able to integrate Big Data into its assessment and decision making, we can support the development of innovated medicines, deliver life-saving treatments to patients more quickly and optimise the safe and effective use of medicines through measurement of a products performance on the market."

Big Data Task Force final report December 2019

The timing is now:

- Joint HMA EMA Big Data Task Force Top-ten data recommendations
- New Commission supporting digital and "EU health data space"
- EU Network Strategy to 2025 includes data and analytics pillar
- EMA Regulatory Science Strategy to 2025 (stakeholders endorse these actions)

Science and technology are evolving fast - so must we



Big Data Task Force Top-ten data recommendations

1	Deliver a sustainable platform to access and analyse healthcare data from across the EU (Data Analysis and Real World Interrogation Network: DARWIN EU)
2	Establish an EU framework for data quality and representativeness
3	Enable data discoverability
4	Develop EU Network skills in Big Data
5	Strengthen EU Network processes for Big Data submissions
6	Build EU Network capability to analyse Big Data (technology / analytics)
7	Modernise the delivery of expert advice
8	Ensure data are managed and analysed within a secure and ethical governance framework
9	Engage with international initiatives on Big Data
10	Establish an EU Big Data 'stakeholder implementation forum'
11	Veterinary recommendations

Priority Recommendation - 1

Deliver a sustainable platform to access and analyse healthcare data from across the EU (**D**ata **A**nalysis **and** **R**eal **W**orld **I**nterrogation **N**etwork (DARWIN))

Why:

Current EU access to healthcare data is limited to minority of MSs and to GP data.

How:

Leverage stakeholder support and existing projects and collaborate with EC to set up network partnership, secure infrastructure and governance platform. Long-term funding (all or part) could come through change to EMA fees.

Gain:

Access to GP, registry, claims and hospital data from the majority of MSs will increase the power, representativeness and the spectrum of use cases that can be addressed. If DARWIN is supported by stakeholders, NCAs could have secure access to data from across the EU.

Implementation principles

Collaborate: with stakeholders and partners including ENCePP

Require: good practice from the industry we regulate

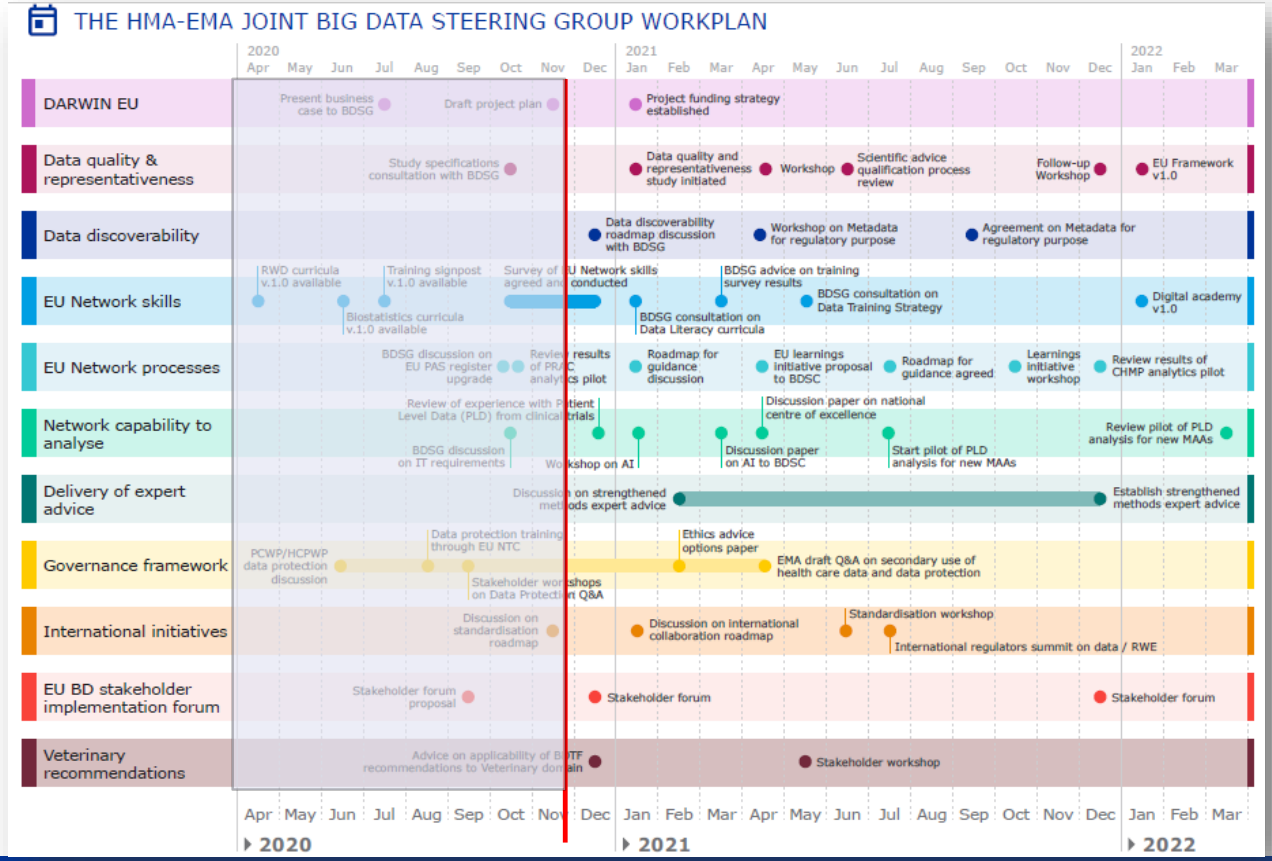
Protect: the best parts of our current system e.g. clinical trials for efficacy

Recognise: the excellence we have in the Network including ENCePP

Seize: opportunities as they arise e.g. revision of regulations, funding opportunities

Internationalise: to deliver more

Implementing the recommendations - BDSG workplan



[Making best use of big data for public health: publication of the Big Data Steering Group workplan for 2020-21]

Big Data work 2021

1. DARWIN	**Details in next presentation | ENCePP consulted on key aspects of data, methods and governance**
2. Data Quality	**Data quality framework – external study launched in 2021 data | ENCePP to be consulted**
3. Discoverability	**Meta data – external study launched December 2020 to agree RWD meta data | ENCePP to be consulted | use meta data to upgrade database of resources (ENCePP upgrade)**
4. Skills	**Big Data Training curricula under development | possible outreach to ENCePP and academia**
5. Processes and transparency	**CHMP + SAWP: pilots initiated | Finalisation of the review of RWE submissions** in marketing authorisation applications | **Database of observational studies (EU PAS upgrade) - project will be scoped 2021**
6. Analytics capability	**Rapid analytics software selected to support EMA committee assessment| Clinical Trials-analysis of Patient Level Data – pilot at CHMP started data | ENCePP to be informed**
7. Expert advice	**Likely new Real World Evidence expert group established in 2021**
8. Ethics and Security	**Data Protection Q&A finalised and published – will be shared with ENCePP**
9. International	**Working on roadmap on international collaboration on RWE –ENCePP – possible opportunities**
10. Stakeholder forum	**First virtual forum 15 Dec 2020: open to ENCePP - will be broadcast**

1st virtual Big data stakeholder forum

- 1st virtual Big data forum to take place on 15 December 2020.
- Objectives:
  - *Inform on implementation of the HMA-EMA Big Data Task Force priority recommendations.*
  - *Understand stakeholders' perspectives.*
  - *Discuss opportunities for stakeholder collaboration and priorities.*

- This Forum will provide a "deep-dive" in the recommendations of the HMA-EMA Big Data Task Force and discuss practical aspects of their implementation. It will also discuss stakeholders' perspectives and opportunities for stakeholder collaboration and priorities. It will therefore usefully complement the session organised during the ENCePP webinar of 20th November and all ENCePP partners are invited to participate.
- To register

Conclusion - the timing is now:

ENCePP community will be key in the successful implementation of the Big data task force Top 10 recommendations.

Thank you

Acknowledgements:

The members of the Big Data Steering Group

BDSG secretariat

HMA secretariat

Commission colleagues

Further information

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Priority Recommendation – 2

Establish an EU framework for data quality and representativeness

Why:

Currently industry and regulators have limited information and knowledge on the quality of different data sources and on their representativeness (patient groups, geography, lab values, pregnancy, lifestyle etc.).

How:

Collaborate with academia to establish a data quality framework relevant to the EU (output is guidance); strengthen Scientific Advice qualification process (and expertise); support Member States to digitalise and securely share data.

Gain:

When giving scientific advice we will know the best data source to recommend to generate evidence for marketing authorisation. When assessing marketing authorisation applications we will be able to judge the evidentiary value of the results.

Strengthened links to national healthcare data sets will help NCAs to know their national data including its quality and relevance to regulation.

Priority Recommendation - 3

Enable data discoverability

Why:

We don’t know what data exist in the MSs and when we do, we don’t know their characteristics.

How:

Agree key (meta) data that describe a data source; include these key data in an enhanced ENCePP resources database as a sign-posting tool (across big data types), promote stakeholders to use FAIR principles.

Gain:

MSs, industry, and academia will have a more comprehensive knowledge of what data sources are available. Supports better drug development (and Scientific Advice) and choice of data source for post-authorisation studies.

Priority Recommendation - 4

Develop EU Network skills in Big Data

Why:

Currently very limited skills and knowledge in the EU Network in key Big Data areas, including: statistics, epidemiology, data science, 'omics, advanced analytics / AI / ML.

How:

Through EU-NTC, map skills in the Network, develop Big Data curriculum, roll-out training (already in development), targeted recruitment, collaboration with academia.

Gain:

EU Network assessors have the knowledge and experience to advise on Big Data sources, on analysis, to conduct analyses in house, to support assessment of MA applications, and to enable the EU Network as reference network for data.

Priority Recommendation - 5

Strengthen EU Network processes for Big Data submissions

Why:

Currently we have limited experience of scientific advice, and of MA application assessments that include Big Data. We do not systematically track and learn from the applications we do have.

How:

Launch Network "Big Data Learnings Initiative" - track and learn from all relevant Big Data applications through the product life-cycle and feed learnings to reflection papers and guidance. Enhance transparency of Big Data studies through the EU Post-Authorisation Studies Register.

Gain:

Each submission received and study posted in the register feeds the knowledge of the EU Network and its assessors. Forms the foundation of guidance for the industry.

Priority Recommendation - 6

Build EU Network capability to analyse Big Data (technology / analytics)

Why:

Currently the Network has limited IT capacity and staff experience to manage and analyse 'raw' data (both patient level data from clinical trials and real world data from health records). Regulators have to rely on what the industry tells them with limited scope to test assumptions and validate.

How:

Build, step by step, through pilots, computing capacity in the cloud. Where possible, leverage existing EU technology initiatives. Improve analytics software capacity for: rapid RWD analysis, Clinical Trials PLD visualisation and analysis, and AI algorithms. MS can support analytics centres.

Gain:

Regulators can receive and analyse 'raw' data to validate claims made by the industry, test AI algorithms and investigate major health issues. Enables better committee decision-making. Establishment of analytics centres of excellence, either within NCAs or academia supports national and EU capacity and leadership in data-driven regulation.

Priority Recommendation - 7

Modernise the delivery of expert advice

Why:

Current methods advice is fragmented (separate biostats, modelling and simulation and extrapolation groups), or absent (no expert fora for real world evidence, advanced analytics or proteomics / metabolomics).

How:

Under the HMA/MB mandate: Build on the existing working party structure to establish a Methodologies Working Party that encompasses biostatistics, modelling and simulation, extrapolation, pharmacokinetics, real world data and epidemiology; an Omics Working Party that builds on and reinforces the existing pharmacogenomics group.

Gain:

Network expertise gaps filled and efficiency of product advice increased. New groups receptive to new agile models of guideline development. New groups support training of Network assessors.

Priority Recommendation - 8

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

Why:

Concerns about data protection and ethical issues around data sharing are major barriers to accessing and analysing data.

How:

Engage with national and EC initiatives on data protection, dialogue and survey patients and HCP views, establish an ethics advisory committee.

Gain:

Clarity for NCAs, EMA, industry and data holders on how to fully comply with data protection while enabling secondary use of healthcare data. Patients' views are heard and the Network has expert ethics advice.

Priority Recommendation - 9

Engage with international initiatives on big data

Why:

International partners including FDA, HC and PMDA are investing heavily in Big Data including pilot projects, IT capability, networks, training and guidance.

How:

Engage with multilateral initiatives and identify opportunities for collaboration including guideline development. Develop a Network standardisation roadmap. Pilot international studies (with FDA and HC). Reach out to international partners for trainings and expert workshops.

Gain:

The Network can learn from the experience of others and leverage their efforts. We avoid duplication of effort and deliver coherent, often harmonised guidance for industry. International studies deliver greater power and allow cross-fertilisation of skills.

Priority Recommendation - 10

Establish two-way communication with stakeholders

Why:

No current platform to discuss Big Data and analytics.

How:

Develop Network communications including road maps and lines to take on Big Data. Communications to articulate the regulatory use cases and requirements for data. Ensure active dialogue with key stakeholders, including, patients, HCPs, industry, HTA bodies, payers, and technology companies.

Gain:

Forum allows to share best practice, to identify common challenges and to scan the horizon. Platform and communication materials increase Network influence over those stakeholders who can deliver change.