HMA – EMA Joint Big Data taskforce: Phase II report - “Evolving Data-Driven Regulation”

PCWP-HCPWP Joint plenary
3 March 2020

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Outline of presentation

- The Big Data Task Force mandate
- Task force work 2018 -2019
- Key recommendations
- Next steps and collaboration with PCWP-HCPWP

1 "Evolving Data-Driven Regulation"

BDTF – The 'Why'

- IT advances are driving digitisation of large volumes of research and clinical data (Big Data).
- The acceptability of evidence from Big Data for regulatory decision-making is uncertain.
- The EU regulatory network:
  - has limited capacity to access and analyse large, unstructured data sets
  - is not best prepared to guide the use of emerging technologies or to interpret analyses based on big data or novel analytical approaches

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

- Extend the taskforce mandate until end of 2019:
  - regulatory prioritisation and implementation of recommendations

Priority Recommendations from the HMA-EMA joint Big Data Task Force

1. Deliver a sustainable platform to access and analyse healthcare data from across the EU (Data Analysis Real World Interrogation Network (DARWIN)).
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.

Priority Recommendations from the HMA-EMA joint Big Data Task Force

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'.

Priority Recommendation - 1

Deliver a sustainable platform to access and analyse healthcare data from across the EU (Data Analysis Real World Interrogation Network (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. **Year 1 – finalise business case with stakeholders, explore opportunities for funding.**

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.

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.

Year 1 – launch data quality study and review SA process and expertise.

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.

Year 1- agree meta data and start work on ENCePP resources database.

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.

Year 1 - skills survey, curriculum and roll-out training.

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. **Year 1 - launch learning initiative, guidance roadmap and concept papers + start work on EU PAS Register enhancement.**

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. **Year 1- strengthen computing capacity and obtain analytics software, and securely access more EHRs for committee assessments.**

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. **In Year 1: one methods group that combines existing separate groups; enhance pharmacogenomics working party to bereinforced 'omics 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. **Year 1: Q&A on data protection; dialogue on data sharing through the Patients and Consumers and Healthcare Professionals Working Parties, propose mandate of ethics group.**

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. **Year 1: International partners contribute to EU-NTC training, active EU participation at ISO, CDISC, ICH and ICMRA; Standardisation Roadmap; agree a plan with CHMP & PRAC for at least one international study.**

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. **Quick win in Year 1 is establishment of Big Data multi-stakeholder implementation forum.**

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.

Implementation principles

- Collaborate: with external stakeholders
- Require: good practice from the industry we regulate
- Protect: the best parts of our current system e.g. clinical trials

The timing is now:

- 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, industry is embracing and so must we

Big Data Steering Group

Reporting to HMA and EMA MB

Mandate:

- Advise on implementation of key recommendations
- Define and measure success factors
- Communicate within the Network and to stakeholders
- Horizon scan, prioritise regulatory science topics
- Identify funding

Membership

- Co-chairmanship: HMA – EMA
- At least 5 NCAs
- European Commission
- Representative of telematics governance
- Committee chairs / vice chairs
- EMA: IT, Analytics, Communications
- Patient and Healthcare professional reps

What benefit to public health and innovation?

The Network will deliver 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 and
- optimise safe and effective use of medicines through measurement of a products performance on the market.

in Year 1:

- ***Network capacity starts to increase*** *to advise on, assess and analyse Big Data*
- ***Training will be rolled out***
- ***Processes improved***
- ***Guidance will start to be developed***
- ***Patients and HCPs will contribute on data protection and data sharing***

Next steps and collaboration

- March 2020: Big Data Steering Group:
  - Request for nominations for 1 PCWP and 1 HCPWP representative
  - *—Provide PCO and HCP perspective into Big Data Task Force deliverables*
  - *—Call for expression of interest to follow*
- March 2020: Health Policy Agencies Collaboration (HPAC) project: EHR: access, share, expand – Expert Drafting Group
  - Request for nominations for 1 PCWP and 1 HCPWP representative
  - *—Provide PCO and PCP perspective on data protection needs on the secondary use of health data – project deliverable is a set of Q&As*
  - *—Call for expression of interest to follow*
- 2 June 2020: session on data protection
  - *—PCWP/HCPWP input into draft Q&A*
- 23 September 2020: workshop on data sharing/ethics
  - *—Call for expression of interest to follow*

Thank you for listening

Further information

See websites for contact details

Heads of Medicines Agencies www.hma.eu
European Medicines Agency www.ema.europa.eu

The European Medicines Agency is an agency of the European Union