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European Confederation of Pharmaceutical Entrepreneurs AISBL

European Medicines Agency (EMA) 1st EU Big Data Stakeholder Forum

Session 2: Stakeholders' priorities

Pharmaceutical industry – EUCOPE

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EUCOPE priorities

Ten BDTF priorities are extremely valuable and **ALL** are very relevant.

i	•**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).** Build the business case with stakeholders and secure funding to establish and maintain a secure EU data platform that supports better decision-making on medicines by informing those decisions with robust evidence from healthcare.	*DARWIN*
ii	•**Establish an EU framework for data quality and representativeness**. Develop guidelines, a strengthened process for data qualification through Scientific Advice, and promote across Member States the uptake of electronic health records, registries, genomics data, and secure data availability.	*Data Quality and representativeness*
v	•**Strengthen EU Network processes for Big Data submissions.** Launch a 'Big Data learnings initiative' where submissions that include Big Data are tracked and outcomes reviewed, with learnings fed into reflection papers and guidelines. Enhance the existing EU PAS register to increase transparency on study methods.	*Guidelines development Roadmap/ Enhance PAS register*
vi	•**Build EU Network capability to analyse Big Data**. Build computing capacity to receive, store, manage and analyse large data sets including patient level data (PLD), establish a network of analytics centres linked to regulatory agencies, and strengthen the Network ability to validate AI algorithms.	*Network capabilities*
ix	•**Collaborate with international initiatives on Big Data.** Support the development of guidelines at international multilateral fora, a data standardisation strategy delivered through standards bodies, and bilateral collaboration and sharing of best practice with international partners.	*International regulatory collaborations*

EUCOPE's focus areas and priorities we believe can bring transformative value to the network

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- **Deliver a sustainable platform to access and analyse healthcare data from across the EU (Data Analysis and Real World Interrogation Network -DARWIN).** Build the business case with stakeholders and secure funding to establish and maintain a secure EU data platform that supports better decision-making on medicines by informing those decisions with robust evidence from healthcare.

Our ask: *Industry should be one of the stakeholders of the DARWIN initiative*

Involved during the design and in demonstration projects to assess the validity, reliability and utility of the system. Ideally, the data and analytic tools included in the DARWIN system should be accessible to all stakeholders in the healthcare ecosystem. This could be done through a coordinating centre (trusted third party) that could facilitate principled research.

- **Supporting the network in the critical first step:** principles for easy and secure accessibility to data
- **Proven experience from industry in data endeavour:** industry has developed advanced analytic capabilities that can be applied to large healthcare data data sets. We have extensive experience of conducting real-world data analyses in order to understand diseases and the benefits and risks of therapeutic interventions.
- **Tangible contribution and input to test system:** disease models and algorithms, experiences and knowledge (e.g. case studies to test system, providing high quality data; common understanding; efficient use of expertise / harmonized risk management programs; sustainability)
- **Risk of "*false start*":** past experience has shown that not involving all relevant key stakeholders will lead to less-than-optimal design and implementation and will exclude valuable expertise that is obtained through multi-disciplinary and multi-stakeholder engagement.
- **Positive examples of cooperative approaches:** Reagan-Udall Foundation's IMED, DAC Pilot, PHUSE, etc.

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- **Establish an EU framework for data quality and representativeness**. Develop guidelines, a strengthened process for data qualification through Scientific Advice, and promote across Member States the uptake of electronic health records, registries, genomics data, and secure data availability.

Our asks: *Advance in multi stakeholder collaboration to design data quality framework and tools*

- **Understanding data relevancy and quality:** a real-world dataset should be evaluated with respect to whether it is fit-for-purpose. For that, key dimensions are data relevancy, data integrity and data quality for a potential regulatory decision within the context of a specific disease state or therapeutic area, (e.g. small orphan conditions versus chronic diseases)

- **Characterizing relevancy and quality:** throughout the dataset creation process with adequate study designs and analytics fit for a study purpose

- **Leverage from industry experience, science and technologies**: Industry and other stakeholders will be conducting multiple independent RWE projects in the coming years – and potential for experience sharing case studies with BDSM e.g. in pilot projects and workshops

- Other collaborative examples: Transcelerate, Duke-Margolis, IMI2

V

- **Strengthen EU Network processes for Big Data submissions.** Launch a 'Big Data learnings initiative' where submissions that include Big Data are tracked and outcomes reviewed, with learnings fed into reflection papers and guidelines. Enhance the existing EU PAS register to increase transparency on study methods.

<u>Our ask</u>: *Involve industry to leverage latest experiences in using RWE in regulatory decision-making*

Experiences with RWE supporting / assessing real-world evidence (RWE) to facilitate regulatory decision-making on COVID-19 treatments and vaccines can be good experiences. The intervention from DKMA / University Utrecht showed the opportunity of the existing registries to effectively establish a wide range of studies on large population.

- **Contribute to key guidance documents:** data characterisation, data analysis, study methods and presentation of protocols and results.
  - ❖ *Guidance on how to assess the reliability and relevance of RWD from medical claims and EHRs, and to design and analyze RW studies with them in support of regulatory decisions*
  - ❖ *Guidance on considerations for designing clinical trials that include pragmatic design elements and that generate evidence of effectiveness for regulatory decisions*
  - ❖ *Guidance on the use of RWD to generate external/synthetic control arms and assess their reliability*
  - ❖ *Guidance on the use and evaluation of AI-based technologies or algorithms in support of care practice or drugs prescriptions*
- **Exchange views on where industry can most effectively contribute:** e.g. case studies, workshops, commenting, 'learnings initiative' leading to workshop in 2021 (orphan case studies on external control arms and associated methodologies and EMA-EU initiative on Registries).
- **Orphan drug development:** RWE (registry or real world data) complementary to clinical trials due to small patient population, heterogenous disease manifestation, lack of clinically robust primary endpoints for whole population and electronic medical record-enabled trials.

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- **Build EU Network capability to analyse Big Data**. Build computing capacity to receive, store, manage and analyse large data sets including patient level data (PLD), establish a network of analytics centres linked to regulatory agencies, and strengthen the Network ability to validate AI algorithms.

<u>Our ask</u>: *Data analytics technologies – preserve mutual trust and data privacy*

- We assume potential role for powerful new players in big data analytics/AI
- Have expertise but operating to different data protection standards
- We recognize the **value of new partners beyond pharmaceutical industry** – but must be fair level play
  - Scientific evaluation versus commercial exploitation of health data
  - Strict adherence to patient data privacy rights
  - Protect company confidential information
  - Trusted third partners
- **Ensure interoperability** and between analytical tools / systems

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•**Collaborate with international initiatives on Big Data.** Support the development of guidelines at international multilateral fora, a data standardisation strategy delivered through standards bodies, and bilateral collaboration and sharing of best practice with international partners.

<u>Our ask</u>: *Industry to be engaged/consulted in the preparation of the standardization roadmap*

In order to make a meaningful contribution to the workshop planned for mid-2021.

- **Scope and limits of international cooperation on Big Data? What are the key stakeholders?**
- **Need for international alignment** on evidentiary standards for RWE to promote global acceptability
  - ICMRA workshop on COVID-19 RWE, WHO global registry on human genome editing
  - Possible future ICH topic?
- **Industry can also constructively contribute** to discussions at international fora on experience with RWE in global development and regulatory submissions (understanding burden of illness, control arms, registries/registry studies) providing case studies with international perspective.

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Key messages

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DARWIN

Establish easy and secure accessibility to data. Pharma Industry should be one of the stakeholders of the DARWIN initiative

ii

Data Quality and representativeness

Advance in multi stakeholder collaboration to design data quality framework and tools

v

Guidelines development Roadmap. Enhance PAS register

Involve industry to leverage latest experiences in using RWE in regulatory decision-making

vi

Network capabilities

Data analytics technologies – Ensure mutual trust and data privacy

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International regulatory collaborations

Industry to be engaged/consulted in the preparation of the standardization roadmap