Preparing for the future – horizon scanning for big data topics

Big Data Stakeholder Forum - 1 December 2022

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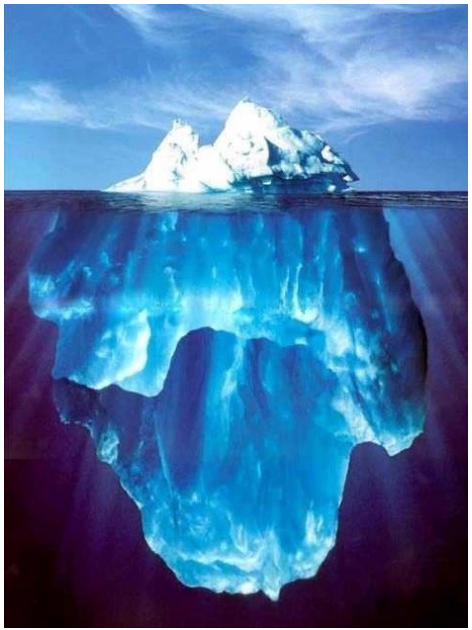
Horizon scanning – looking deeper into the future

Information from regulatory interactions, business forecasts

Systematic examination of information to detect early signs of important developments for medicines and public health



EMA Horizon scanning – overview

Purpose

- Adapt and minimise regulatory bottlenecks
- Foster high-quality development
- Ensure innovations reach patients

- Horizon scanning capabilities established as per EMA Regulatory science strategy 2025 and EMAN Strategy 2025

- Sources – many predefined scientific journals, databases, grey literature are scanned for potential signals
- Signals – technologies, methods or substances that are important regulatory challenges or public health opportunities
- Time horizon – 3 to 10 years before being in a submission for marketing authorisation of a medicinal product
- Outputs – reports with recommendations

EMA Horizon scanning reports 2021/2022

ICMRA: 3D bioprinting

EU-IN: Genome Editing

EU-IN: FMT

EC JRC: Weak signals in science and technology

ICMRA: Artificial Intelligence

First findings for big data from horizon scanning (1/2)

Source information from scanning (paraphrased)	Example regulatory challenge(s) and public health opportunities
1) Deep digital phenotyping - relationships between an individual's real-world "digitosome" and their phenotype	Using an individual's heart rate variability and treatment response to optimise dosing schedules
2) Digital twins - using various data to build virtual digital twins, compare a patient's characteristics with a bank of correlations between characteristics and outcomes	To predict the best treatment strategy, to virtually model that patient and their outcomes with different treatments
3) HCPs need to be able to interpret RWD - RWD is increasingly used and included for example in ARs, SmPCs	Understanding RWD will help HCPs to know how to best use such results with patients, guidelines, research
4) Scaling big data scales uncertainty – conclusions drawn from big data such as RWE are uncertain even when increasing the size of the data	Weights ascribed to RWE should scale according to uncertainty and bias. Models to express uncertainties. Uncertainties to be considered in decision-making.
5) AI individualizing posology – algorithms are being developed to advise HCPs and patients on dose and posology, using big data, e.g., post-marketing RWE from various regions	Individual advice may conflict with SmPC, agreements for reimbursement and clinical treatment guidelines
6) Sociodemographic bias – a risk when building models using big data. For example, prognoses differ for patients due to sociodemographic confounders or healthcare access	Models and their validation need to take potential differences into account and be wary of selection bias, collider bias etc.

First findings for big data from horizon scanning (2/2)

Source information from scanning (paraphrased)	Example regulatory challenge(s) and public health opportunities
7) Ethics – Big data requires consideration by regulators including ethics committees	Concern are informed consent, privacy & confidentiality, fairness and justice, trust, data ownership, transparency, safety, autonomy and patient empowerment
8) Data privacy – important legal and ethical issue	Patient consent and governance; discrimination *vs* data use; managing breaches
9) Data quality – various frameworks have been developed	Various aspects like data completeness, conformance, verification and validation, as well as *metadata* quality
10) Data quantity – despite availability of big data, it still an issue that models are built on insufficient underlying data	Guidance on data quantity and related aspects
11) Qualification of novel methods – Innovative Science and Technology Approaches for New Drugs (ISTAND) as example of broad scope of support sought by developers	Support pathways for broader set of drug development tools such as include big data and AI development and application

Question for the panelists and audience

How are stakeholders doing horizon scanning for big data in their organisation?

Which big data signals have stakeholders found?

What added value could horizon scanning signals have to inform big data activities?

Thank you

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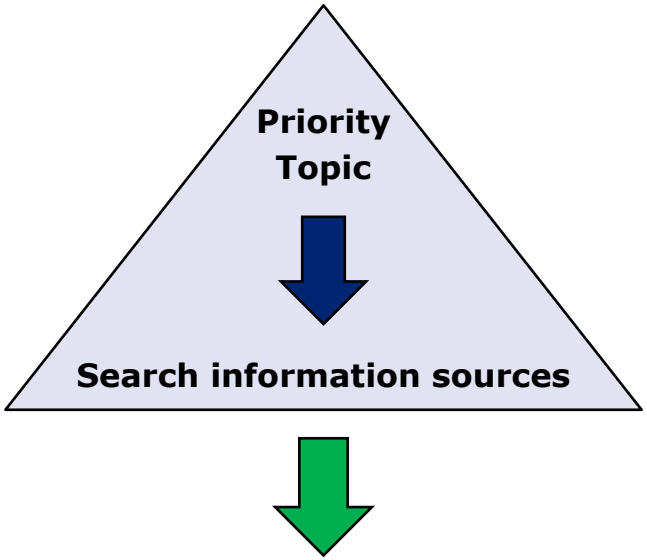
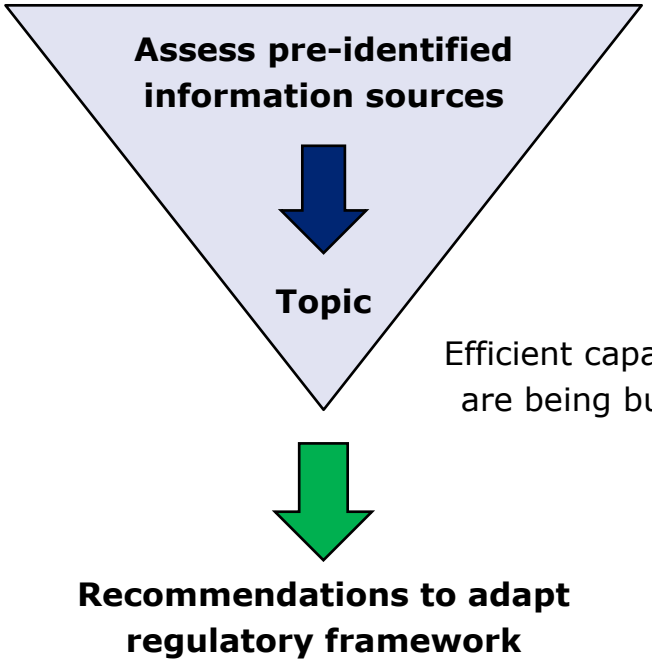
EMA Horizon scanning approaches





Efficient capabilities
are being built up

Outline of horizon scanning report

EMA – facing and enabling innovation

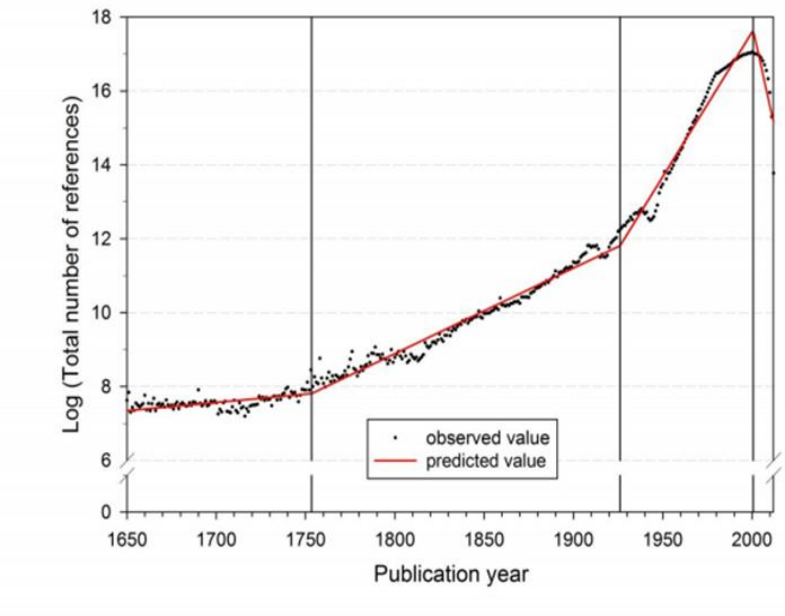
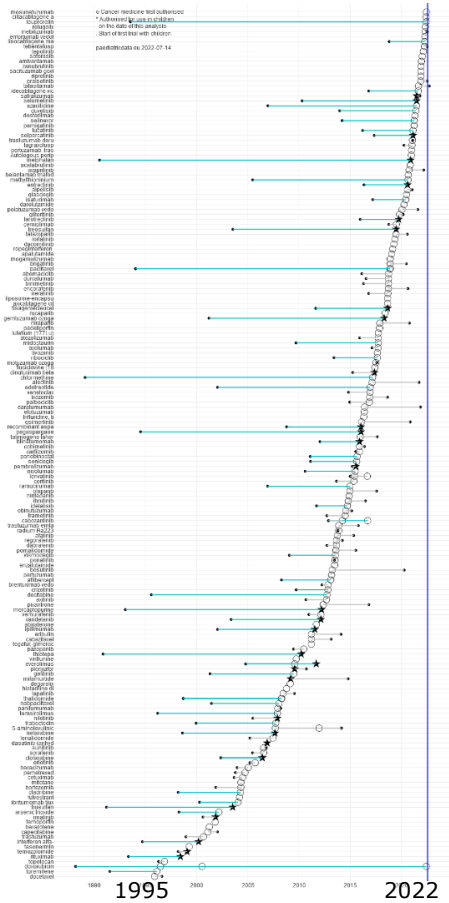


Figure 2. Segmented growth of the annual number of cited references from 1650 to 2012 (citing publications from 1980 to 2012) https://doi.org/10.1002/asi.23329



Novel anti-cancer medicines centrally recommended by EMA

1995 2022