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SCIENCE MEDICINES HEALTH

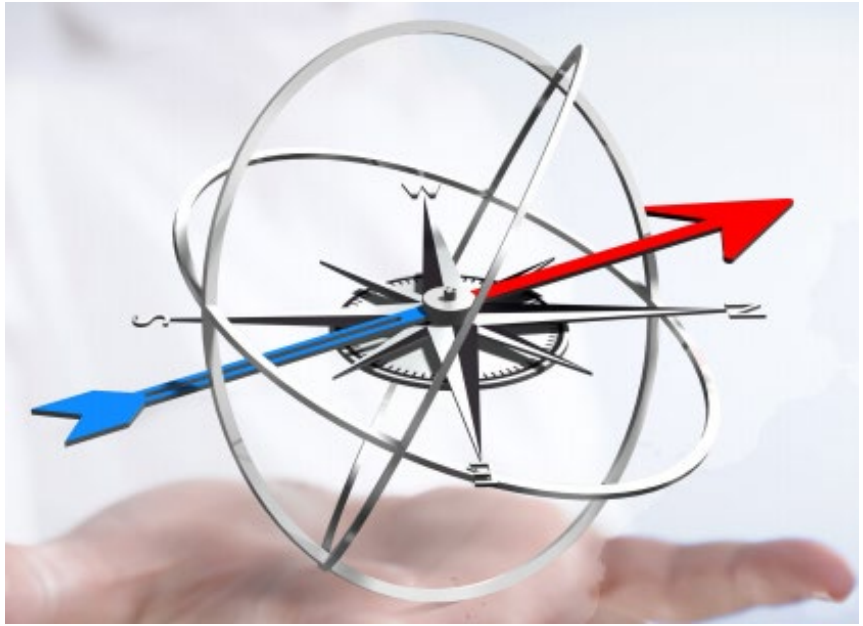
Qualification of novel methodologies

Biomarkers and Companion Diagnostics – The future of precision medicine

MEB Science Day – Utrecht 13 April 2023

Falk Ehmann – European Medicines Agency

The future of Health Care Systems



"We need to be ready to support the development of increasingly complex medicines that more and more deliver healthcare solutions by converging different technologies to promote and protect human and animal health."

EU Strategies and their translation of into tangible outcomes

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HMA
Heads of Medicines Agencies

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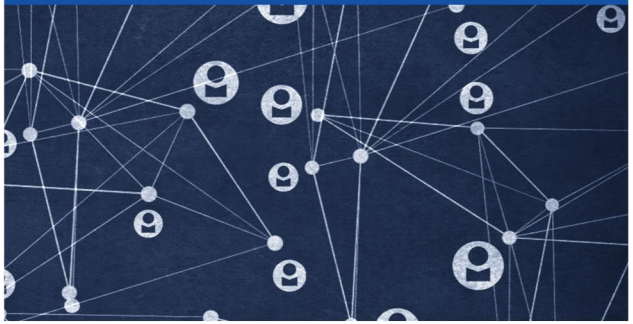
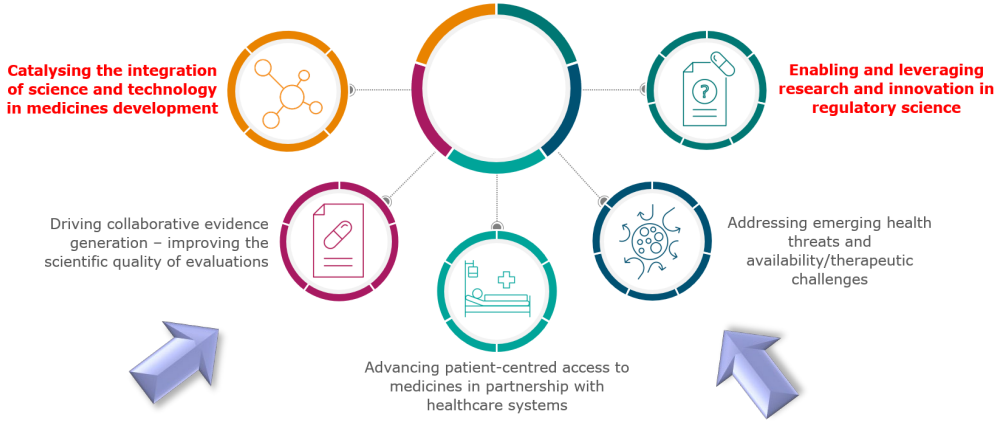
European medicines agencies network strategy to 2025

Protecting public health at a time of rapid change



Goals include:



CBG MEB
MEDICINES EVALUATION BOARD

Towards Tomorrow

A status update on the Medicines Evaluation Board Science Policy 2020 - 2024

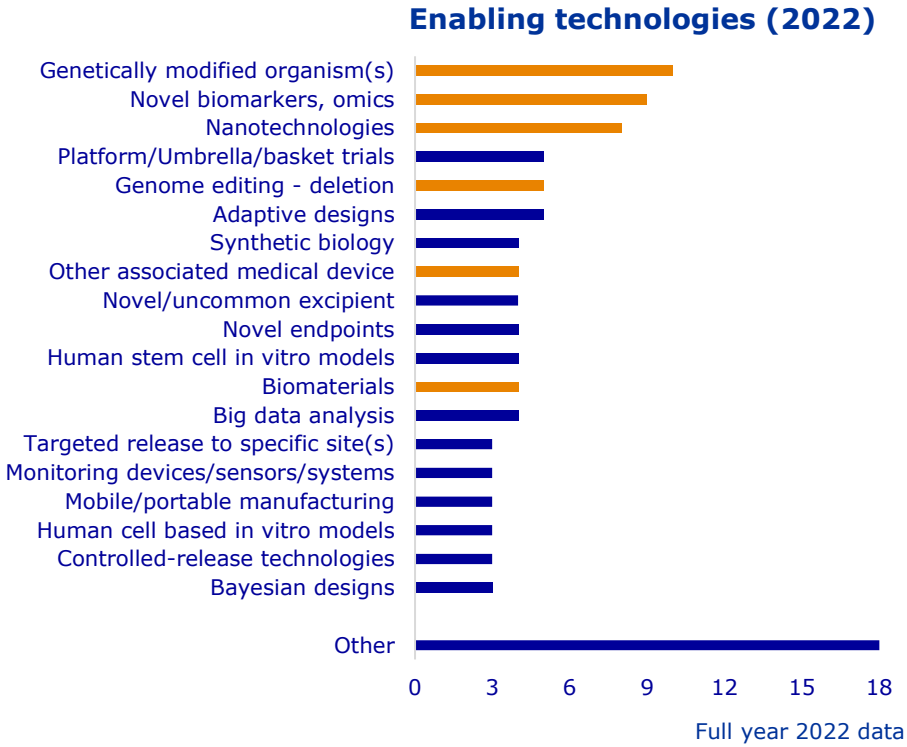
Enabling technologies

Novel and promising technologies that have the potential to enable innovation

- Highest amount of enabling technologies related to new **genetically modified organisms, novel biomarkers** & **omics data**, and **nanotechnologies.**

- Orange: enabling technologies overlapping with EIC pathfinder Health Legacy – technology focus

→ *identification of **trends***



Qualification of novel methodologies…

…a voluntary **pathway** allowing developers of **innovative methods** and **tools** to request a **qualification** by European regulators of these **instruments** for a **specific intended use** in the context of research and development into pharmaceuticals.

What are “Tools, Methods and Instruments”?

Main categories of QonM Advices and Opinions (2021/2022; n=43)

- New Clinical Outcome Assessments
- New Digital Tools and Imaging Methodologies
- New Biomarker Qualification
- New Statistical Methodologies
- New methods for the development of Advanced Therapy Medicinal Products

Acknowledgement: Ana Drmić

Biomarker Qualification analysis

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Review > Clin Pharmacol Ther. 2022 Jul;112(1):69-80. doi: 10.1002/cpt.2554. Epub 2022 Mar 5.

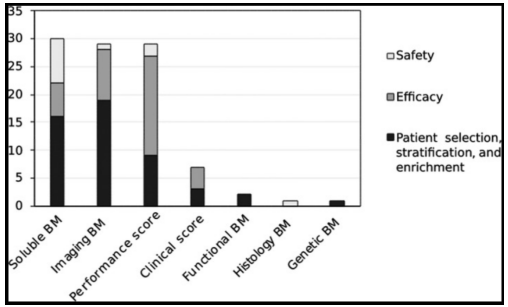
Biomarker Qualification at the European Medicines Agency: A Review of Biomarker Qualification Procedures From 2008 to 2020

Elisabeth Bakker [1], Natalie M Hendrikse [2], Falk Ehmann [2], Daniëlla S van der Meer [1 3], Jordi Llinares Garcia [2], Thorsten Vetter [2], Viktoriia Starokozhko [1 3], Peter G M Mol [1 3 4]

Affiliations + expand

PMID: 35137949 PMCID: PMC9313861 DOI: 10.1002/cpt.2554

Free PMC article



Different types of biomarkers in each CoU category. The stacked bars show the number of procedures in the three CoU categories for each type. BM, biomarker; CoU, context of use.

> Front Med (Lausanne). 2022 Apr 26;9:878942. doi: 10.3389/fmed.2022.878942. eCollection 2022.

Biomarkers in Medicines Development–From Discovery to Regulatory Qualification and Beyond

Natalie M Hendrikse [1], Jordi Llinares Garcia [1], Thorsten Vetter [2], Anthony J Humphreys [1], Falk Ehmann [1]

Affiliations + expand

PMID: 35559349 PMCID: PMC9086587 DOI: 10.3389/fmed.2022.878942

Free PMC article

Supplementary Table 2: ITF meetings with follow up in form of Scientific Advice (SA) or Qualification Advice (QA).

ITF meeting	Description	Follow-up
March 2010	Exploratory-phase qualification of clinical Drug-Induced Liver Injury (DILI) biomarkers (safety, monitoring, diagnostic, prognostic)	QA (2013), FU-QA (2013)
June 2010	General discussion on PD/Response biomarkers for clinical trials CNTO136 programme in Rheumatoid Arthritis.	SA: 3 hits, but no mention of the biomarker
June 2010	Exploratory-phase qualification of clinical Drug-Induced Kidney Injury (DIKI) biomarkers (safety, monitoring, diagnostic, prognostic)	QA (2013), FU-QA (2013), FU-QA (2014), FU-QA (2016)
October 2010	Exploratory-phase qualification of clinical Drug-Induced Vascular Injury (DIVI) biomarkers (safety, monitoring)	QA (2013), FU-QA (2016)
March 2011	Periostin as predictive biomarker to identify patients with severe asthma that may benefit from blockade of IL13 by Lebrikizumab	QA (2012), SA (2015)
March 2011	Predictive biomarker for selection of patients with "Met-positive" non-small cell lung cancer for trials	SA (2011), SA (2012)
September 2012	General discussion on predictive biomarkers for clinical trials in MS	SA (2012)
September 2012	Classification of cystic fibrosis patients by predictive biomarker CTFR mutations/molecular phenotypes	SA (2017)
December 2012	Lung Clearance Index as a surrogate endpoint in cystic fibrosis trials	AS (2017)
October 2014	General discussion on biomarkers for clinical trials in type 1 diabetes	QA (2019): not on biomarkers, but on trial protocol design
October 2015	Genomic Allergen Rapid Detection (GARD): predictive biomarker signature as screening tool in early drug discovery	Applied for QOQA in December 2015, withdrawn
October 2018	Biomarkers for non-alcoholic fatty liver disease and non-alcoholic steatohepatitis (Diagnostic, Prognostic, Monitoring, PD/Response)	QA (2019), QA (2019)

Biomarker and Companion Diagnostics:

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Front. Med., 01 November 2021
Sec. Regulatory Science
Volume 8 - 2021 | https://doi.org/10.3389/fmed.2021.753187

This article is part of the Research Topic
Insights in Regulatory Science: 2021
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Biomarker and Companion Diagnostics—A Review of Medicinal Products Approved by the European Medicines Agency

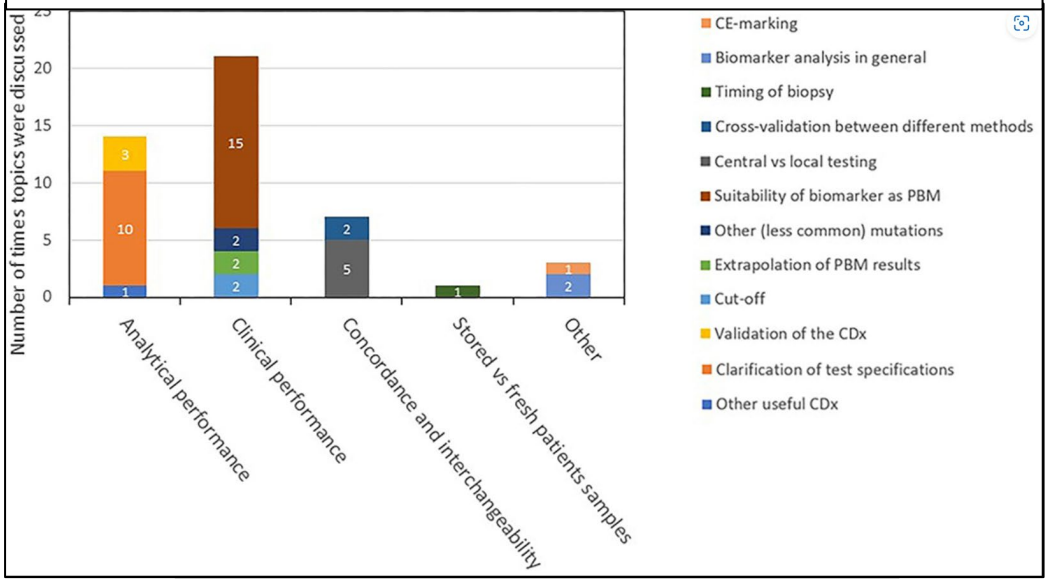
Laura Patricia Orellana García[1,2*], Falk Ehmann[2], Philip A. Hines[1,2,3], Armin Ritzhaupt[2] and Angela Brand[1]

Sec. Regulatory Science
Volume 9 - 2022 | https://doi.org/10.3389/fmed.2022.893028

Evaluation of Companion Diagnostics in Scientific Advice and Drug Marketing Authorization Applications by the European Medicines Agency

Marc Maliepaard[1,2*], Priscilla Nibi[1], Gabrièlla Nibi[1] and Anna M. G. Pasmooij[1]

Description and categorization of CDx found in the *day 120 ARs*. The four topics of CDx analytical performance, clinical performance, concordance and interchangeability, and testing of stored vs. fresh patient samples were further divided into subtopics. Remarks that did not fit to any of the four topics were categorized as "other." AR, assessment report; CDx, companion diagnostic; EPAR, European public assessment report; PBM, predictive biomarker.



EU Innovation Network

Horizon Scanning – Companion Diagnostics (CDx)

Participating EU-IN Members (lead(s) identified in bold)

NL (CBG-MEB), IT, SE, AT, DE, EMA, HPRA

EMA multi-stakeholder workshop:
Qualification of novel methodologies

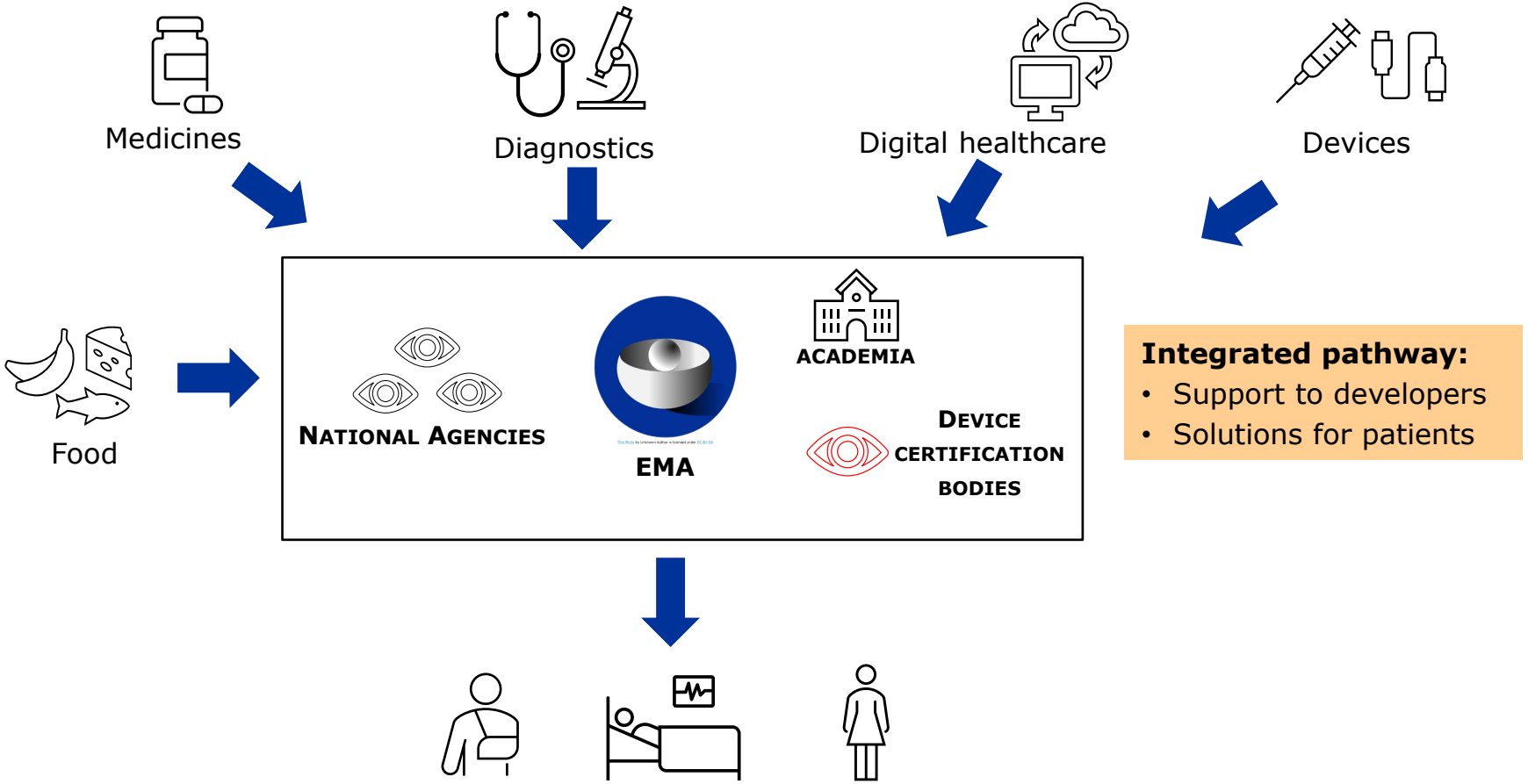
17 – 18 April 2023

Virtual meeting

The aims of the workshop are to:

- How to best support translation of innovation into patient benefit;
- Future proof the qualification of novel methodologies process and its outcomes.

Vision of an integrated pathway for complex health care solutions



Any questions?

Further information

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