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Pathways to bring research insights into clinical practice

Perspectives from supporting, assessing and overseeing cancer medicines

First CCI4EU Capacity Building Conference, 10-11 October 2024, Warsaw

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An agency of the European Union





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Learning health system: shared ambition but no blueprint

Friedman C, et al. J Am Med Inform Assoc 2015;22:43–50. doi:10.1136/amiajnl-2014-002977, Research and Applications

Toward a science of learning systems: a research agenda for the high-functioning Learning Health System

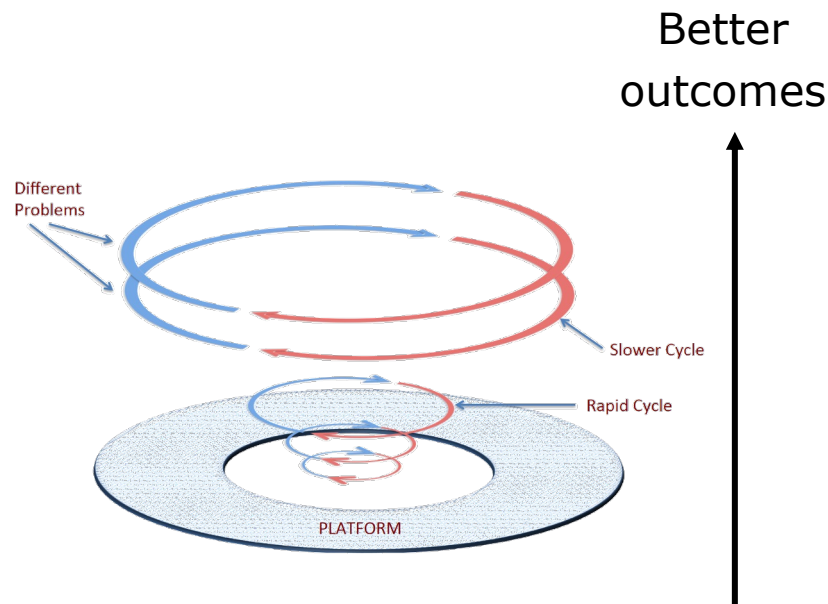
Charles Friedman¹, Joshua Rubin¹, Jeffrey Brown², Melinda Buntin³, Milton Corn⁴, Lynn Etheredge⁵, Carl Gunter⁶, Mark Musen⁷, Richard Platt⁸, William Stead⁹, Kevin Sullivan⁹, Douglas Van Houweling¹

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AMIA
AMERICAN MEDICAL ASSOCIATION
INSTITUTIONAL LEARNING THEORY

OXFORD
UNIVERSITY PRESS

“In a Learning healthcare system, science, informatics, incentives and culture are aligned for continuous improvement and innovation, with best practices seamlessly embedded in the delivery process and new knowledge captured as an integral by-product of the delivery experience”



Progress is difficult, and difficult to measure

Levels of Evidence Supporting American College of Cardiology/American Heart Association and European Society of Cardiology Guidelines, 2008-2018

Alexander C. Fanaroff, MD, MHS¹; Robert M. Califf, MD^{2,3,4}; Stephan Windecker, MD⁵; et al

► Author Affiliations | Article Information

JAMA. 2019;321(11):1069-1080. doi:10.1001/jama.2019.1122



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Comment



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Key Points

Question What proportion of recommendations in current American College of Cardiology/American Heart Association (ACC/AHA) and European Society of Cardiology (ESC) guidelines are supported by evidence from multiple randomized controlled trials (RCTs), and how has this changed over the past 10 years?

Findings In this systematic review of 51 current guideline documents that included 6329 recommendations, 8.5% of recommendations in ACC/AHA guidelines and 14.3% of recommendations in ESC guidelines were classified as level of evidence A (supported by evidence from multiple RCTs), compared with 11.5% of recommendations in a systematic review of ACC/AHA guidelines conducted in 2009.

Meaning Among recommendations in major cardiovascular society guidelines from 2008 to 2018, the proportion supported by evidence from RCTs remains small.

<https://doi.org/10.1001/jama.2019.1122>

Level of scientific evidence supporting NCCN guidelines: Is there evidence of progress?

Authors: Aakash Desai, Ronald S. Go, and Thejaswi Poonacha | [AUTHORS INFO & AFFILIATIONS](#)

Publication: Journal of Clinical Oncology • Volume 37, Number 27, suppl • https://doi.org/10.1200/JCO.2019.37.27_suppl.14

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Abstract

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Background: National Comprehensive Cancer Network (NCCN) guidelines are the most comprehensive and widely used standard for clinical care in oncology by clinicians and payors in the US. The level of scientific evidence in NCCN guidelines has not been studied since its last review in 2010. We describe the categories of evidence and consensus (EC) among the 10 most common cancers in the US as of 2019 and compare them with 2010 guidelines. **Methods:** We obtained the 2019 version of NCCN guidelines. The definitions for various categories of EC used were: Category 1 (high level evidence such as randomized controlled trials with uniform consensus), 2A (lower level of evidence with uniform consensus), 2B (lower level of evidence without a uniform consensus but with no major disagreement) and 3 (any level of evidence but with major disagreement). We compared our results with previously published results from 2010 guidelines. **Results:** Total

distribution of different types of categories of EC are largely similar to 2010. **Conclusions:** Recommendations in 2019 NCCN guidelines are largely derived from lower levels of evidence with uniform expert opinion. Despite the major advances in oncology in the past decade, this is largely unchanged. Our study underscores the urgent need and available opportunities to expand the current evidence base in oncology which forms the platform for clinical practice guidelines.

This is an ASCO Meeting Abstract from the ASCO Quality Care Symposium. This abstract does not include a full text component.

https://doi.org/10.1200/JCO.2019.37.27_suppl.14



Emer Cooke

Extracts from EMA Executive Director's address to the EP Beating Cancer Committee (BECA) Jan 2021

<https://www.europarl.europa.eu/committees/en/public-hearing-mind-the-gap-for-equal-ac/product-details/20210119CHE08124>

Challenges and possible solutions

Clinical trials

- focus on trials that are fit for purpose from a patient perspective
- ensure access to clinical trials and facilitating participation in trials
- majority of trials conducted in only a few countries and centres
- rare cancers will benefit from facilitating access to clinical trials

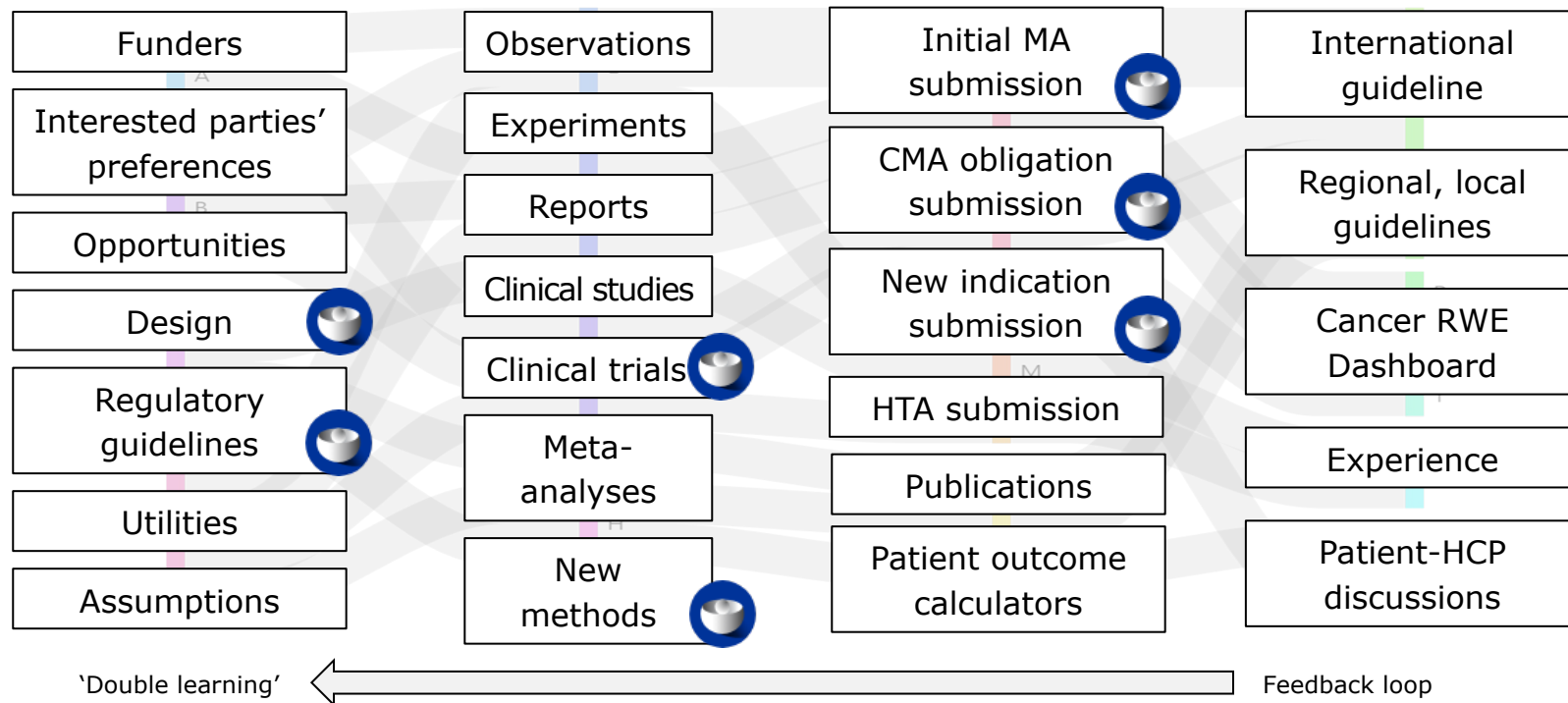
Collaboration with decision-makers

- modernise methods to include patient views in B/R assessment and how we communicate B/R decisions to patients and doctors
- efficient communication to decrease time lag and differences between authorisation decisions by regulators and decisions by HTA
- multi-stakeholder discussions on development plans is a key component

Support to innovation for cancer medicines

- with best designed trials can we support the innovation and breakthroughs that can address cancer patients' needs
- EMA is committed to creating an enabling environment for development, evaluation and access to new and repurposed cancer medicines

Drive Research Results Insights Actions Outcomes



Partners and stakeholders – collaboration management needed beyond primary oncology community

Public-private projects (HE, IMI, IHI) such as GUIDE.MRD, IMAGIO, OPTIMA, HARMONY, SISAQOL-IMI, BIGPICTURE, ...

Tech providers, e.g. EHR (electronic healthcare records), CDSS (clinical decision support systems), case management

Hospital organisations, HTA bodies, payers, devices bodies

EP THCS (Transforming healthcare systems)

Academic methodology and CRO service organisations

Funders and collaboratives (e.g. ATTRACT calls)

Research university associations

EP Rare Diseases

European Partnerships in digital, industry and space

EP PerMed Personalised Medicines

Sustained projects, e.g. BD4BO, c4c-Stichting, EIT Health

ERA4Health (not cancer)

Opportunities with EMA, EU NCAs and beyond

- Systematically use EMA resources for competency and capacity building, e.g.
 - [EPARs](#) | [Clinical data publication](#) | [Trial registers](#) | [HCP infos](#) | [consultations](#) | [workshops](#), [recordings](#)
- Discuss issues with national and EU regulators, e.g. pragmatic and 'academic' trials
 - Project-related issues: [incentivised Scientific advice](#), academia@ema.europa.eu
 - General issues: [EMA Cancer Medicines Forum \(CMF\)](#) | [Oncology European Specialised Expert Community](#) | [Accelerating Trials in EU \(ACT EU\)](#) | [CTR Collaborate Stakeholder meetings](#) | [EMA Regulatory science research needs](#) | European platform for regulatory science research
- Strategically use educational offerings (also ESO; ESMO, EHA programmes)
 - EMA HMA Pilot Educational Program Oncology (10 sessions, 30-45 mins each)
https://www.youtube.com/playlist?list=PL7K5dNgKlawbqMVuBm4yapsI_YilKvv2E
 - FDA <https://www.fda.gov/about-fda/2022-oce-annual-report/professional-education> and <https://www.fda.gov/about-fda/oncology-center-excellence/project-significant-statistics-cancer-trials>

Concluding considerations

- Important to define pathways for research insights into clinical practice
- Important to have a system to shape good research and to get it done well –
What can we do together upstream to strengthen the research?
- Resources available for competency and capacity building for researchers and carers –
What is missing in general, and what more could be for EMA to consider providing?
- Collaborations to be managed with a broad range of stakeholders
- Change management is a possible model for a system approach
- EMA's stake in the change: improving outcomes for patients with cancer!

Thank you for the invitation –
we look forward to hearing from CCI4EU

Further information

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