EMA

EUROPEAN MEDICINES AGENCY

EMA Cancer Medicines Forum (CMF) meeting with industry stakeholders on cancer treatment optimisation

Agenda

14 November 2025
Hybrid meeting / EMA, Amsterdam

Advancing Cancer Research Through Shared Data & Samples

Prof. Christine Desmedt
KU Leuven
Leuven Cancer Institute
Leuven, Belgium

Global effort:

53 academic clinicians, researchers & patient advocates

Hans Wildiers
Virginie Adam
Seamus O'Reilly
Josephine Van Cauwenberge
Amal Arahmani
Carlos L. Arteaga
Philippe L. Bedard
Panayota Boussis
Judith Bliss
Etienne Brain
Marc Buyse
Carmela Caballero
David Cameron
Fatima Cardoso
Eva Carrasco
Ana Casas
Boon Chua
Giuseppe Curigliano
Angela De Michele
Laura Esserman
Giuseppe Floris
Matthew P. Goetz
Theodora Goulioti
Benjamin Haibe-Kains
Christine Hodgdon
Michail Ignatiadis
Marleen Kok
Denis Lacombe
Barbro Linderholm
Sherene Loi
Christopher J. Lord
Mairead MacKenzie
Julia Maues
Lydie Meheus
Judy Needham
Heather Parsons
Patrick Neven
Martine Piccart
Lajos Pusztai
Evangelia Razis
Eva Schumacher-Wulf
Shigehira Saji
Gabe Sonke
Tania Spanic
Ian F. Tannock
Andrew Tutt
Ander Urruticoechea
Laura Van 'T Veer
Ines Vaz-Luis,
Gustavo Werutsky
Douglas Yee
Khalil Zaman

Importance

- Molecular analyses of biospecimens collected from study participants are essential for identifying **biomarkers that can tailor treatments to specific subsets of patients** who are most likely to benefit.

- Sharing of data and biospecimens from clinical trials enables **personalized, patient-centric use of cancer therapies** and **accelerates the development of new treatments**.

Increasing role of pharma in cancer research

Original Reports | Health Services and Outcomes

Check for up

Patient Enrollment to Industry-Sponsored Versus Federally-Sponsored Cancer Clinical Trials

Joseph M. Unger, PhD[1]; Hong Xiao, PhD[1]; Riha Vaidya, PhD[1]; and Michael LeBlanc, PhD[1]

DOI https://doi.org/10.1200/JCO.24.00843

ABSTRACT

PURPOSE The conduct of cancer clinical research in the United States is supported by both private and public sponsors. Industry aims to obtain new drug approvals. Federally-sponsored trials examine a broad set of research questions that are not typically addressed by industry; these trials, which are also more commonly conducted in diverse populations, were recently shown to have contributed to gains of 14 million life-years for patients with cancer. Despite the different mandates, the proportion of patients who might participate in industry-sponsored versus federally-sponsored cancer studies is unknown.

METHODS We evaluated trial enrollment patterns from 2008 to 2022 using Clinical-Trials.gov data. The ratio of enrollments attributable to industry versus federal sponsors was estimated. A large set of estimates on the basis of different combinations of study characteristics were generated. Point estimates were determined as the mean of combinations and confidence limits by the IQR. Five-year intervals were examined to smooth annual variation.

RESULTS In total, N = 26,080 studies were examined. The estimated enrollment ratio from 2018 to 2022 for all industry-sponsored versus federally-sponsored trials was 8.1 (IQR, 6.2-9.9). For adult trials, the ratio increased from 4.8 (IQR, 4.4-5.3) during 2008-2012 to 9.6 (IQR, 7.4-11.8) during 2018-2022; for trials in children, the ratio increased from 0.7 (IQR, 0.6-0.7) to 2.3 (IQR, 1.8-2.7). Despite increasing cancer incidence, enrollment counts for federally-sponsored trials were flat over the study period.

CONCLUSION In the United States, there is a growing reliance on industry to conduct cancer clinical research. Underinvestment in federally-sponsored research comes at a cost for both patients and researchers, with lost opportunities for scientific, clinical, and population advances.

ACCOMPANYING CONTENT

Editorial, p. 3887

Appendix

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View Online Article

Analysis of **>23,000 cancer trials** (clinicaltrials.gov)

Enrollment ratios in cancer clinical trials increased over time for all industry-sponsored versus federally-sponsored trials (US):

- 2008-2012= **4.8**
- 2018-2022= **9.6**

Data sharing from clinical trials

- National Institutes of Health (**NIH**): data sharing policy since 2003 for all new grants
- International Committee on Medical Journal Editors (**ICMJE**): clear statement on data sharing
- European Medicines Agency (**EMA**): clinical reports with details on methods and results of trial

BUT **implementation in practice remains limited and challenging**

Sharing of Individual Participant Data (IPD)

JAMA Oncology | Original Investigation

Heterogeneity and Utility of Pharmaceutical Company Sharing of Individual-Participant Data Packages

Ashley M. Hopkins, PhD; Natansh D. Modi, BPharm; Ahmad Y. Abuhelwa, PhD; Ganessan Kichenadasse, MD, PhD; Nicole M. Kuderer, MD; Gary H. Lyman, MD, MPH; Michael D. Wiese, PhD; Ross A. McKinnon, PhD; Frank W. Rockhold, PhD; Aaron Mann, BS; Andrew Rowland, PhD; Michael J. Sorich, PhD

IMPORTANCE The pharmaceutical industry has made substantial investments in developing processes for sharing individual-participant data (IPD) from clinical trials. However, the utility and completeness of shared IPD and supporting documents must be evaluated to ensure the potential for scientific advancements from the data sharing ecosystem can be realized.

OBJECTIVE To assess the utility and completeness of IPD and supporting documents provided from industry-sponsored clinical trials.

DESIGN, SETTING, AND PARTICIPANTS From February 9, 2022, to February 9, 2023, 91 of 203 clinical trials supporting US Food and Drug Administration registrations of anticancer medicines for the treatment of solid tumors from the past decade were confirmed as eligible for IPD request. This quality improvement study performed a retrospective audit of the utility and completeness of the IPD and supporting documents provided from the 91 clinical trials for a planned meta-analysis.

EXPOSURES Request for IPD from 91 clinical oncology trials indicated as eligible for the request.

MAIN OUTCOMES AND MEASURES The utility and completeness of the IPD and supporting documents provided.

RESULTS The IPD packages were obtained from 70 of 91 requested clinical trials (77%). The median time to data provision was 123 (range, 117-352) days. Redactions were observed in 18 of the acquired IPD packages (26%) for outcome data, 11 (16%) for assessment variables, and 19 (27%) for adjustment data. Additionally, 20 IPD packages (29%) lacked a clinical study report, 4 (6%) had incomplete or missing data dictionaries, and 20 (29%) were missing anonymization or redaction description files. Access to IPD from 21 eligible trials (23%) was not granted.

CONCLUSIONS AND RELEVANCE In this quality improvement study, there was substantial variability within the provided IPD packages regarding the completeness of key data variables and supporting documents. To improve the data sharing ecosystem, key areas for enhancement include (1) ensuring that clinical trials are eligible for IPD sharing, (2) making eligible IPD transparently accessible, and (3) ensuring that IPD packages meet a standard of utility and completeness.

Invited Commentary
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Supplemental content

Review of **91 registration clinical oncology trials** from 16 different pharma.

Sharing can happen through different platforms:

- **Vivli**[1] (for 81% of the trials)
- ClinicalStudyDataRequest.com[2] (**CSDR**, 9%)
- Yale University Open Data Access[3] (**YODA**, 6%)
- **Proprietary internal platforms**

Sharing of Individual Participant Data (IPD)

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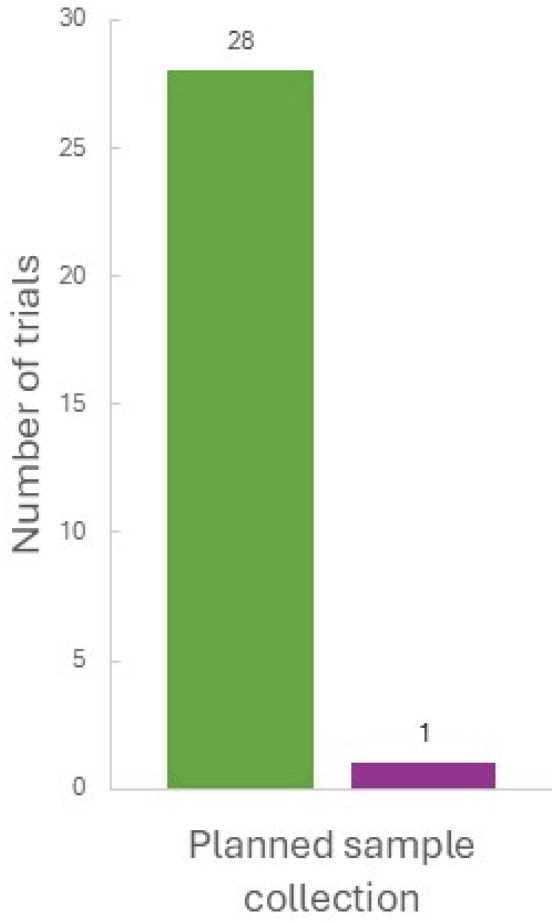
Supplemental content

- IDP packages obtained for 77% trials
- Median time to data provision: 123 days (117-352)
- Heterogeneity in IDP packages:
  - Redaction outcome data (26%)
  - Redaction assessment (16%)
  - Redaction adjustment variables (27%)
  - Lack of study report (29%)
  - Etc

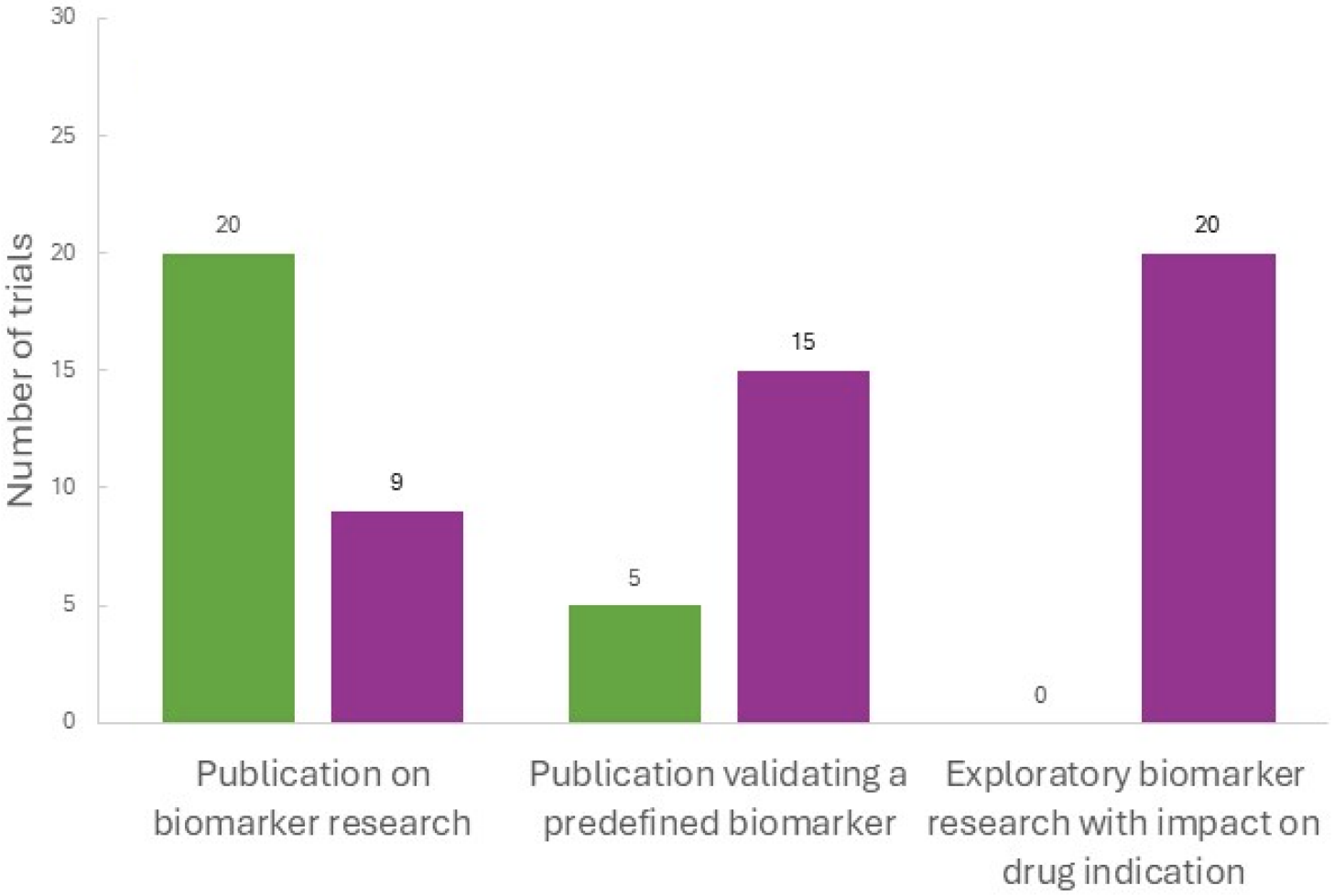
Collection of biospecimen



29 registration trials that led to drug approval in breast cancer (2017 - 2024)

Clinical trial participants are increasingly being asked to provide **tissue and/or body fluid biospecimen** for biomarker research, often also at multiple time points

Predictive biomarkers in registration trials



- New therapies ➔ often '**broad drug label**' because of statistically significant improvements in protocol defined study endpoints, but efficacy is limited to subset of patients.

- Of 29 registration trials:
  - 20 published biomarker study (mostly 1)
  - 5 predefined biomarker study
  - 0 analysis leading to change in indication

Successful examples (not from registration trials)

- **HER2 status** (HER2-0 versus HER-low) in patients treated with CDK4/6 inhibitors in the **PALOMA-2** and **PALOMA-3** trials
- **BMI** in patients with HER2-positive breast cancer across 5 trials (**HERA**, **CLEOPATRA**, **MARIANNE**, **EMILIA** and **TH3RESA**), and BMI in patients with metastatic breast cancer treated with CDK4/6 inhibitors in the MONARCH-2 and -3 trials
- **I-SPY2**: adaptive platform working with different companies
- **PALLAS** trial: partnership between pharma and academia for optimal exploitation of biospecimen and data (BMI and HER2)

Li EbioMedicine 2024, Modi NPJ Breast Cancer 2021, Franzoi JNCI 2021, Wang Curr Breast Cancer Rep 2019, Nader-Marta Breast Can Res 2024, Pfeiler JCO 2023

#1- Trial design and conduct

Co-leadership structures to ensure:

- collection of all the needed data
- prospective biomarker testing beyond standard markers
- planned biomarker analyses for toxicity
- prospective validation in real world data

#2 – Role of patients

- Patients consent to donate data and samples to support correlative science to develop **more tailered treatments** for future patients

- Patients assume that their care team and academic partners have been fully involved in design of the trial and subsequent analyses

- Greater role for **Public and Patient Involvement and Engagement** (**PPIE**) to support effective use of their collected samples and data

- Explicitly mentioning in **ICF** that clinical and biomarker data, as well as biospecimen should be available for biomarker research with academia

#3 – Sharing as requirement for regulatory approval

- Despite data-sharing mechanisms in place, pharma typically retain full control over whicg projects are approved, which data are released, which analyses are done and when.

- Discolure of **IPD** from registrational trials (and others as well) should be encouraged as regulatory requirement.

- **Plan for data sharing** should be proactively and collaboratively developed at time of study design to maje sure everything is considered (pt consent, data formats, legal & ethical requirements, platform to be used, timelines etc).

- **Standardization of IPD packages** needed to also ensure feasibility of post-approval meta-analyses.

#4 – Sharing of biospecimen

- Publications describing translational research involving analysis of these patient samples are scarce and/or much delayed

- **Destruction of biospecimen should be avoided** any time and biospecimen custodianship plan should be developed early, with co-leadership structure.

- Strong need to **standardize biomarker assays and interpretation.**

#5 – Sharing of biological data

- Results of any biomarker analyses that has been conducted **should be shared rapidly** to avoid duplication and wasting of resources → could inform identification and validation of predictive biomarkers and could also inform the **design of new clinical trials**

- **Sharing via recognized platforms** (cBioPortal).

- **Standardization & documentation needed.**

Benefits of Broader Sharing of Data & Samples

- **Boosts Patient Engagement**: Encourages participation in clinical research.
- **Builds Public Trust**: Transparency enhances credibility.
- **Drives Innovation**: Enables research leading to new discoveries.
- **Improves scientific credibility**.
- **Influences Policy**: Better perceptions can lead to favorable pricing models.
- **Optimizes Drug Use**: Focus on subpopulations with meaningful clinical benefit.
- **Accelerates Approvals & Reimbursement**
- **Advances Science**: Academic collaboration reveals new drug targets.
- **Appeals to Investors**: Ethical, socially responsible practices attract funding.

Thank you to the collaborators & thank you for your attention!

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Julia Maues
Lydie Meheus
Judy Needham
Heather Parsons
Patrick Neven
Martine Piccart
Lajos Pusztai
Evangelia Razis
Eva Schumacher-Wulf
Shigehira Saji
Gabe Sonke
Tania Spanic
Ian F. Tannock
Andrew Tutt
Ander Urruticoechea
Laura Van 'T Veer
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Gustavo Werutsky
Douglas Yee
Khalil Zaman