Involvement of Experts and HTA Bodies

JY Blay
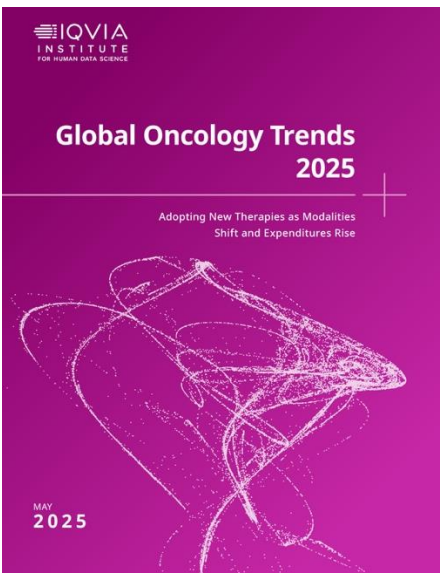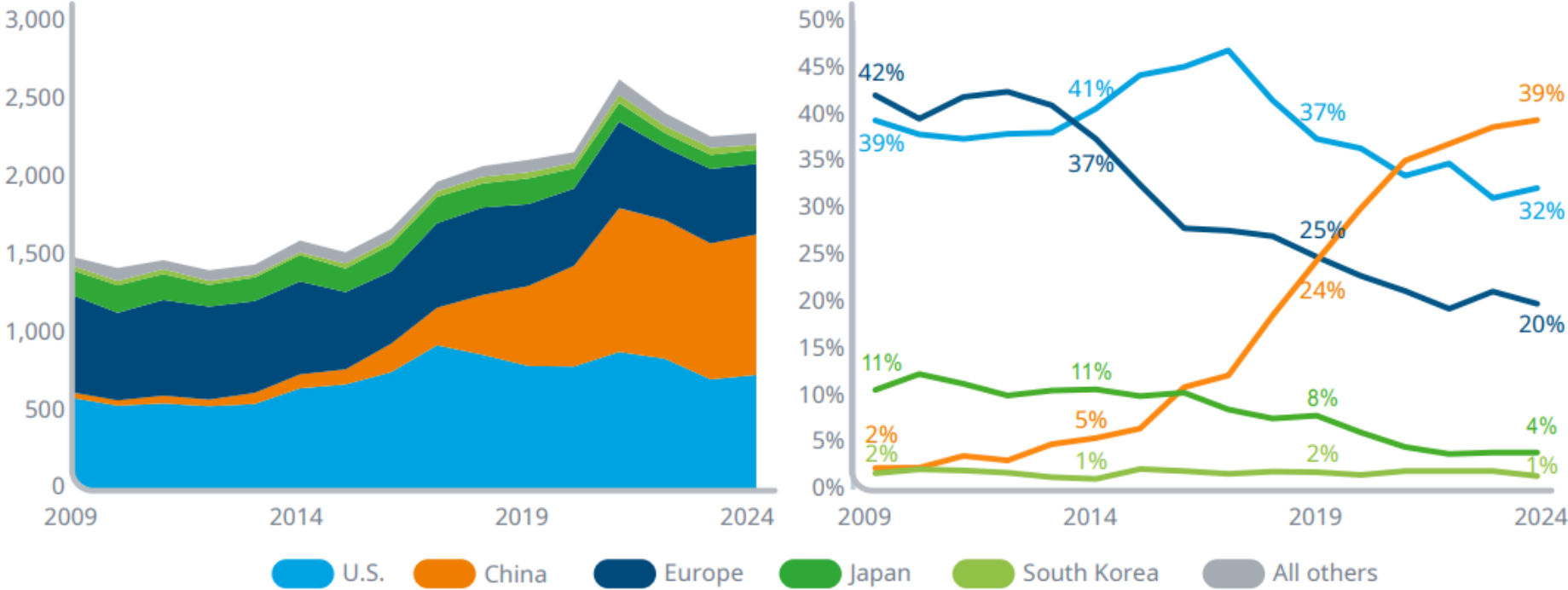
Global Oncology Trends 2025

Adopting New Therapies as Modalities Shift and Expenditures Rise

MAY 2025

Exhibit 3: Number and share of oncology trial starts by company headquarters location, 2009–2024

Legend: U.S. | China | Europe | Japan | South Korea | All others

ESMO-MCBS
ESMO-Magnitude of Clinical Benefit Scale

ANNALS OF ONCOLOGY DRIVING INNOVATION IN ONCOLOGY

SPECIAL ARTICLE

ESMO-Magnitude of Clinical Benefit Scale version 2.0 (ESMO-MCBS v2.0)

N. I. Cherny[1*], S. F. Oosting[2], U. Dafni[3,4], N. J. Latino[5], M. Galotti[5], P. Zygoura[4,6], G. Dimopoulou[4], T. Amaral[7], J. Barriuso[8], A. Calles[9], B. Kiesewetter[10], C. Gomez-Roca[11], B. Gyawali[12], M. Piccart[13], A. Passaro[14], F. Roitberg[15], N. Tarazona[16], D. Trapani[17,18], G. Curigliano[17,18], R. Wester[19], G. Zarkavelis[20], C. Zielinski[21] & E. G. E. de Vries[2]

01. **There are 6 forms**

Evaluation form 1a: for RCTs evaluating new approaches to adjuvant therapy or new potentially curative therapies.

Evaluation form 1b: for single arm de-escalation studies in the adjuvant setting.

Evaluation form 2a: for therapies that are not likely to be curative with primary endpoint of OS with separate forms:
- IF median OS with the standard treatment is <12 months
- IF median OS with the standard treatment is ≥12 - <24 months
- IF median OS with the standard treatment is ≥24 - <36 months
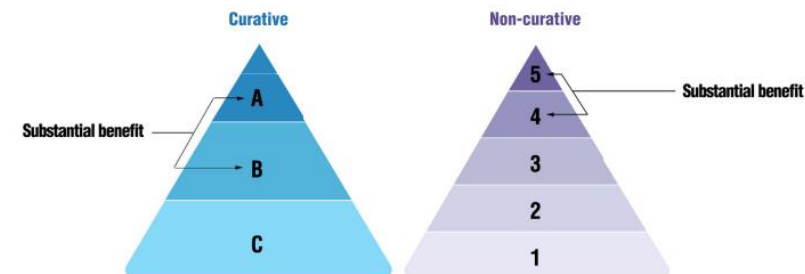- IF median OS with the standard treatment is ≥36 months (or not reached with ≥36 months follow-up)

Evaluation form 2b: for therapies that are not likely to be curative with primary endpoint PFS with separate forms:
- IF median PFS with standard treatment is <6 months
- IF median PFS with standard treatment is ≥6 - <12 months
- IF median PFS with standard treatment is ≥12 months

Evaluation form 2c: for therapies that are not likely to be curative with primary endpoint other than OS or PFS or non-inferiority studies.

Evaluation form 3: for single-arm studies in "orphan diseases" and for diseases with "high unmet need" when

Conclusions: The amendments incorporated into ESMO-MCBS v2.0 change the scores of 13.6% of evaluated studies (10.5% downgraded, 3.1% upgraded) and add toxicity annotations to 45.5% of the studies in the curative setting, and improve its discriminatory capacity and utility.

The highest possible grades of the ESMO-MCBS are A in the curative setting, and 5 for non-curative indications (for the primary endpoint OS). Grades of A and B in the curative setting and 5 and 4 in the non-curative setting indicate substantial clinical benefit. New therapies demonstrating substantial clinical benefit justify rapid consideration for reimbursement.
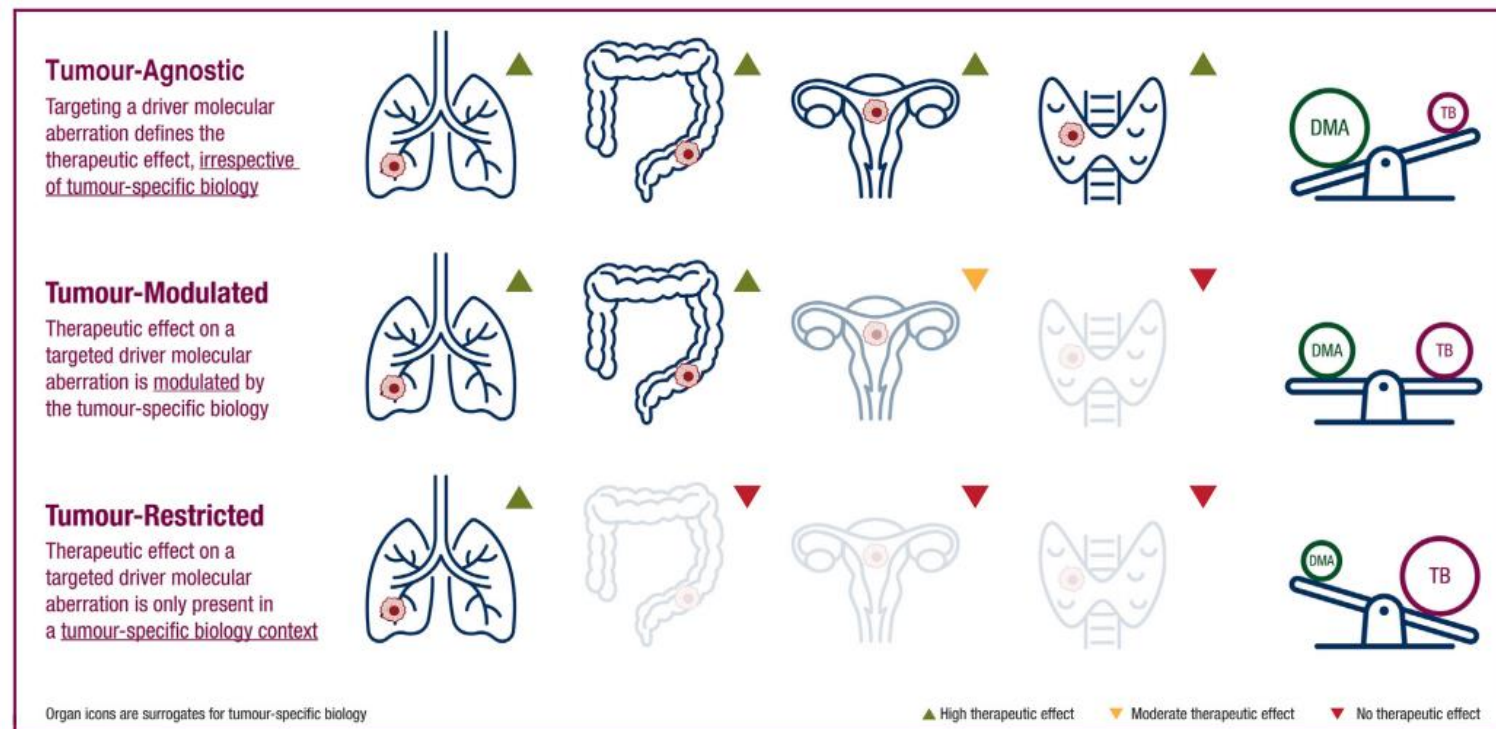

ESMO-MCBS, European Society for Medical Oncology-Magnitude of Clinical Benefit Scale; ORR, overall response rate; OS, overall survival; PFS, progression-free survival; RTCs, randomised-controlled trials.

Optimal and validated tools for clinical decision making & evaluation by Agencies and Payers

Cutting-edge tools for evaluation by Agencies and Payers

SPECIAL ARTICLE

The ESMO Tumour-Agnostic Classifier and Screener (ETAC-S): a tool for assessing tumour-agnostic potential of molecularly guided therapies and for steering drug development

C. B. Westphalen[1,2*†], D. Martins-Branco[3†], J. R. Beal[4], C. Cardone[5], N. Coleman[6,7,8], A. M. Schram[9,10], S. Halabi[11,12], S. Michiels[13,14], C. Yap[15], F. André[16,17,18], F. Bibeau[19], G. Curigliano[20,21], E. Garralda[22], S. Kummar[23], R. Kurzrock[24], S. Limaye[25], S. Loges[26,27], A. Marabelle[28], C. Marchió[29,30], J. Mateo[22], J. Rodon[31], T. Spanic[32], G. Pentheroudakis[3‡] & V. Subbiah[33‡]

Figure 4. Proposed ESMO Tumour-Agnostic Classifier (ETAC) taxonomy: tumour-agnostic (e.g. TRK inhibitors in tumours harbouring *NTRK* gene fusions), tumour-modulated (e.g. PARP inhibitors in tumours harbouring *BRCA*1/2 mutation/homologous recombination deficiency), or tumour-restricted (e.g. PI3K inhibitors in *PIK3CA*-mutated breast cancer).

ESMO Global Consortium Study on the availability, out-of-pocket costs, and accessibility of cancer medicines: 2023 update

N. I. Cherny[1]*[†], D. Trapani[2,3][†], M. Galotti[4], M. Saar[5,6], G. Bricalli[4], F. Roitberg[7,8], B. Gyawali[9], G. Curigliano[2,3], J.-Y. Blay[10], K. Meier[6,11], N. J. Latino[4] & E. G. E. de Vries[12]

Results: Data were collected by 317 reporters and 231 peer reviewers across 126 countries. The study revealed that patients in most high-income countries can access cancer medications without significant out-of-pocket expenditure, including novel treatments with high ESMO-Magnitude of Clinical Benefit Scale (ESMO-MCBS) scores. Conversely, in lower-middle and low-income countries, 40% of traditional chemotherapy agents deemed essential in the WHO EML are only available at full cost to patients.

New & expensive IO/TT

ESMO

ANNALS OF ONCOLOGY — DRIVING INNOVATION IN ONCOLOGY

SPECIAL ARTICLE

ESMO Global Consortium Study on the availability, out-of-pocket costs, and accessibility of cancer medicines: 2023 update

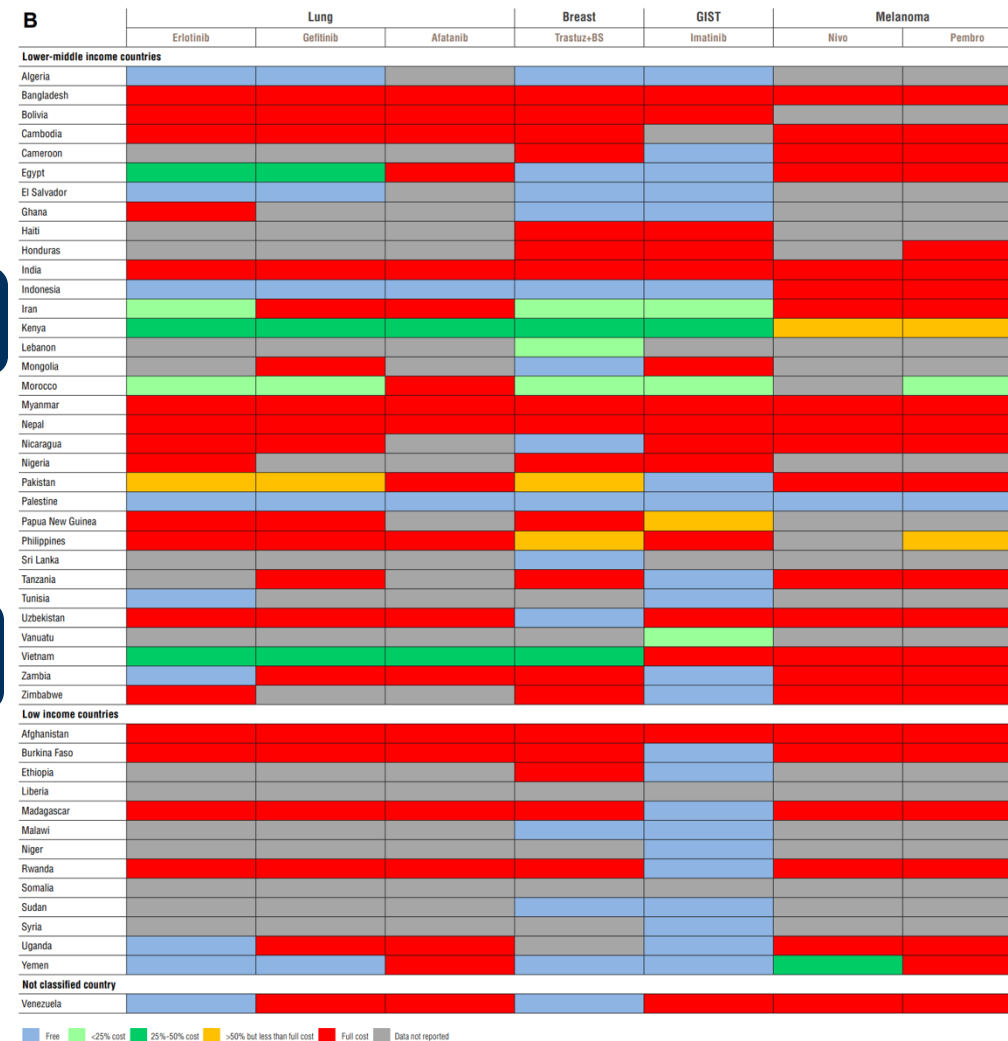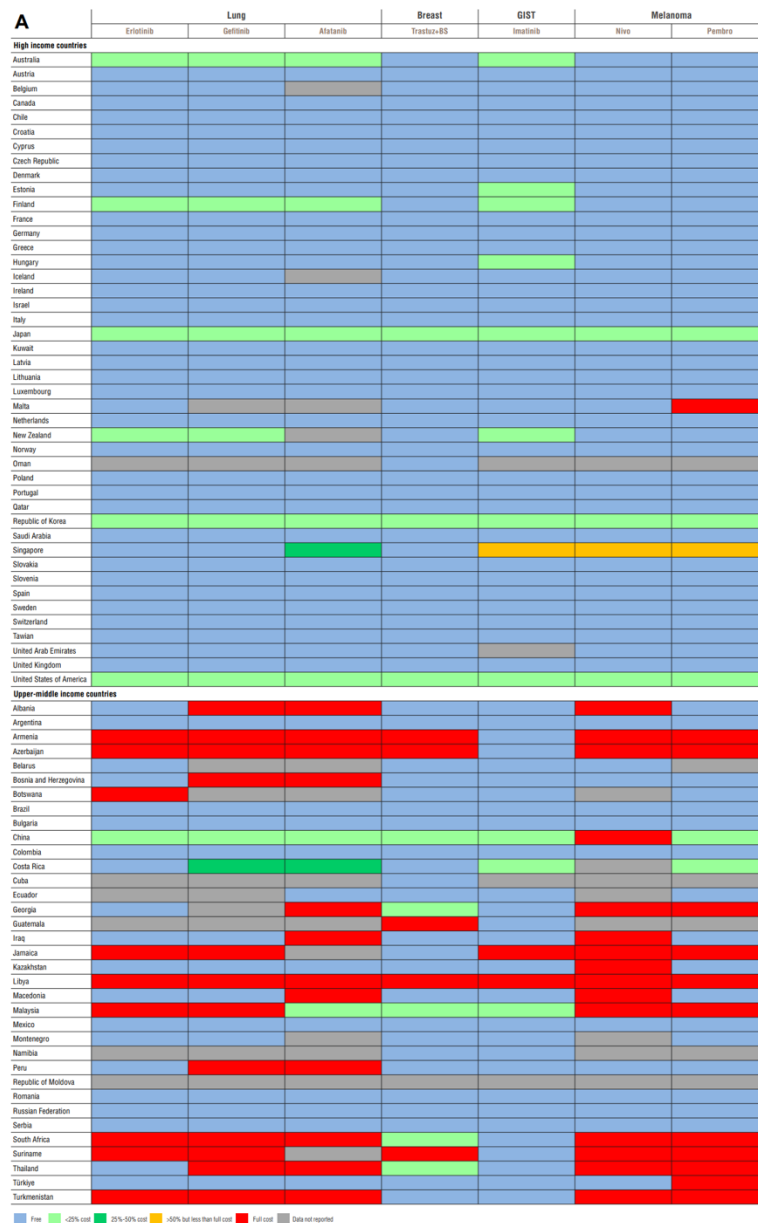N. I. Cherny[1*†], D. Trapani[2,3†], M. Galotti[4], M. Saar[5,6], G. Bricalli[4], F. Roitberg[7,8], B. Gyawali[9], G. Curigliano[2,3], J.-Y. Blay[10], K. Meier[6,11], N. J. Latino[4] & E. G. E. de Vries[12]
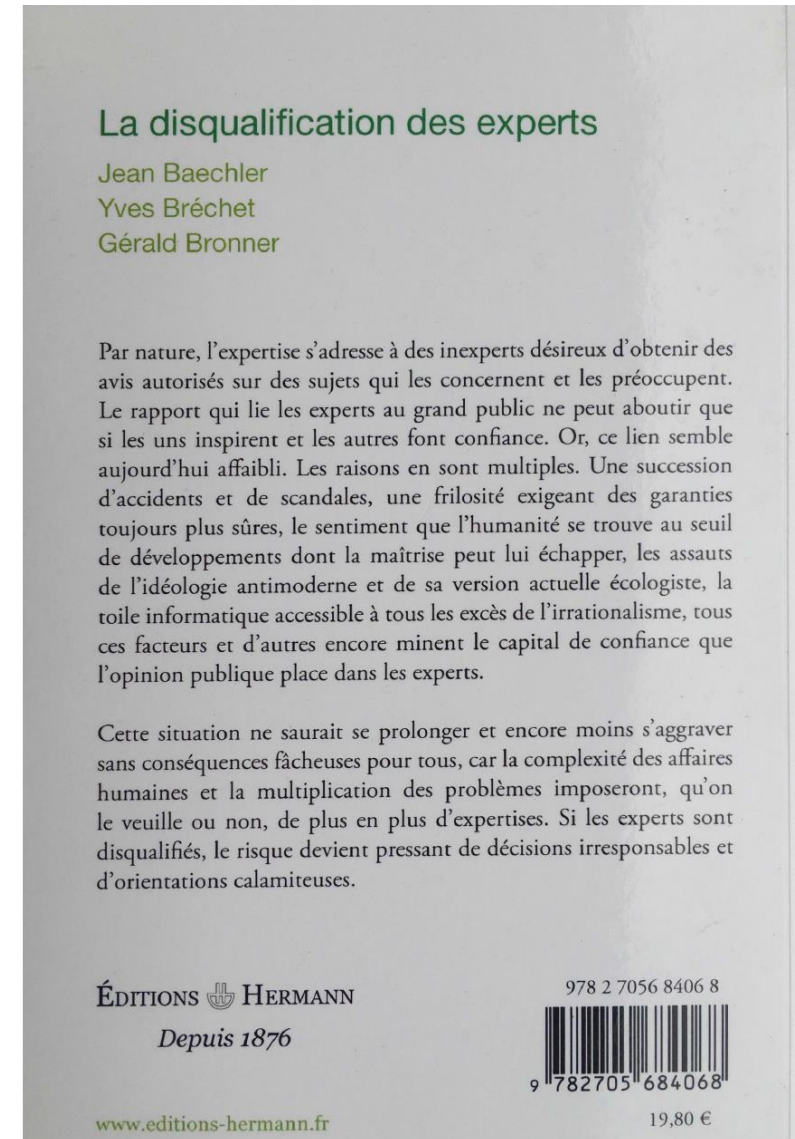
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Health technology assessment: national, EU and global

FRAMEWORK FOR JOINT HTA COOPERATION

» Joint clinical assessments (JCAs).

» Joint scientific consultations (JSCs).

» Identification of emerging health technologies.

» Common procedures and methodologies across the EU.

KEY PRINCIPLES OF THE HTA REGULATION

» Only on clinical domains of the assessment: No economic assessment or any conclusion on pricing and reimbursement.

» Driven by EU HTA bodies who remain responsible for drawing conclusions on added value for their health systems.

» High quality, timeliness and transparency.

» Use of joint work in national HTA processes.

» Input from independent experts.

» Stakeholder engagement and inclusiveness.

» Progressive implementation.

TIMELINE FOR MEDICINES

» 12 January 2025: New oncology medicines and advanced therapy medicinal products will be assessed at EU level.

» 13 January 2028: Orphan medicinal products to be added to the joint work.

» 13 January 2030: All new medicines will come under the scope of the regulation.

Patients and doctors expect
Fast, high quality process, BY the experts of the field

The disqualification of experts

La disqualification des experts

Sous la direction de Jean Baechler

Débat public
Collection de l'Académie
des sciences morales et politiques

hermann
Depuis 1876

La disqualification des experts

Jean Baechler
Yves Bréchet
Gérald Bronner

Par nature, l'expertise s'adresse à des inexperts désireux d'obtenir des avis autorisés sur des sujets qui les concernent et les préoccupent. Le rapport qui lie les experts au grand public ne peut aboutir que si les uns inspirent et les autres font confiance. Or, ce lien semble aujourd'hui affaibli. Les raisons en sont multiples. Une succession d'accidents et de scandales, une frilosité exigeant des garanties toujours plus sûres, le sentiment que l'humanité se trouve au seuil de développements dont la maîtrise peut lui échapper, les assauts de l'idéologie antimoderne et de sa version actuelle écologiste, la toile informatique accessible à tous les excès de l'irrationalisme, tous ces facteurs et d'autres encore minent le capital de confiance que l'opinion publique place dans les experts.

Cette situation ne saurait se prolonger et encore moins s'aggraver sans conséquences fâcheuses pour tous, car la complexité des affaires humaines et la multiplication des problèmes imposeront, qu'on le veuille ou non, de plus en plus d'expertises. Si les experts sont disqualifiés, le risque devient pressant de décisions irresponsables et d'orientations calamiteuses.

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FDA NEWS RELEASE

FDA Announces Completion of First AI-Assisted Scientific Review Pilot and Aggressive Agency-Wide AI Rollout Timeline

⊖ More Press Announcements

For Immediate Release: May 08, 2025

In a historic first for the agency, FDA Commissioner Martin A. Makary, M.D., M.P.H., today announced an aggressive timeline to scale use of artificial intelligence (AI) internally across all FDA centers by June 30, 2025, following the completion of a new generative AI pilot for scientific reviewers.

"I was blown away by the success of our first AI-assisted scientific review pilot. We need to value our scientists' time and reduce the amount of non-productive busywork that has historically consumed much of the review process. The agency-wide deployment of these capabilities holds tremendous promise in accelerating the review time for new therapies," said Dr. Makary.

The generative AI tools allow FDA scientists and subject-matter experts to spend less time on tedious, repetitive tasks that often slow down the review process.

"This is a game-changer technology that has enabled me to perform scientific review tasks in minutes that used to take three days," said Jinzhong (Jin) Liu, Deputy Director, Office of Drug Evaluation Sciences, Office of New Drugs in FDA's Center for Drug Evaluation and Research (CDER).

To reflect the urgency of this effort, Dr. Makary has directed all FDA centers to begin deployment immediately, with the goal of full integration by the end of June. Work will continue to expand use cases, improve functionality and adapt to the evolving needs of each center after June 30. By that date, all centers will be operating on a common, secure generative AI system integrated with FDA's internal data platforms.

"There have been years of talk about AI capabilities in frameworks, conferences and panels but we cannot afford to keep talking. It is time to take action. The opportunity to reduce tasks that once took days to just minutes is too important to delay," said Dr. Makary.

Next Steps

Looking ahead, the FDA plans to expand generative AI capabilities—across all centers using a secure, unified platform. Future enhancements will focus on improving usability, expanding document integration, and tailoring outputs to center-specific needs, while maintaining strict information security and compliance with FDA policy.

The agency-wide rollout is being coordinated by Jeremy Walsh, the FDA's newly appointed Chief AI Officer and Sridhar Mantha. Walsh previously led enterprise-scale technology deployments across federal health and intelligence agencies and Mantha recently led the Office of Business Informatics in CDER.

The agency will continue to assess performance, gather user feedback and refine features to support the evolving needs of FDA staff and advance its public health mission. Additional details and updates on the initiative will be shared publicly in June.