FONDAZIONE
PER LA RICERCA FARMACOLOGICA
GIANNI BENZI
ONLUS

December 10th, 2021
Virtual meeting

XIV FORESIGHT TRAINING COURSE
The health emergency: regulatory crash and future perspectives

Real-World Evidence Data in a Drug Submission Process: the EMA vs. FDA perspective

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Regulatory Definitions on RWD and RWE

Real World Data (RWD) are data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources

- electronic health records (EHRs)
- medical claims data
- product and disease registries
- patient-generated data, including in-home settings
- data gathered from other sources, such as mobile devices, that can inform on health status

Real World Evidence (RWE) is the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD

Generated using different study designs, including but not limited to randomized trials (e.g., large simple trials, pragmatic trials), externally controlled trials, and observational studies

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EMA on Real World Evidence

3.3.4 Promote use of high-quality real-world data (RWD) in decision-making

Real world data is currently used predominantly in the post-authorisation phase but there are opportunities for further application throughout the medicines lifecycle to help address some of the limitations of clinical trials. The Agency recognises the benefit of using RWD to generate complementary evidence across the product life cycle and is committed to promote the use of high quality RWD in decision-making.

However, it will be important to agree amongst stakeholders where RWD may add value into the assessment process. Given the often heterogeneous nature of the data sources, further work is also needed on the analytical and epidemiological methodologies needed to deliver robust evidence. As noted in some other recommendations, there are additional needs to ensure privacy and security of the data, and governance models must address these.

The actions EMA proposes to promote the use of high-quality RWD in decision making are:

- Create a sustainable, quality assured, flexible framework delivering rapid access to and analysis of representative, longitudinal RWD throughout a product's lifecycle
- Develop a capacity that will enable the Agency to rapidly and securely access and analyse large amounts of healthcare data
- Accelerate the implementation of a learning regulatory system based on electronic health records and other routinely collected clinical care data (including RWD).

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24 September 2020
EMA/502388/2020

Guideline on registry-based studies

Draft

Draft approved by the Cross-Committee Task Force on Registries	May 2020
Draft sent to the EU Regulatory Network for consultation	9 July 2020
Start of public consultation	24 September 2020
End of consultation (deadline for comments)	31 December 2020

Comments should be included in the form published with this draft guideline, and should be sent to EMAregistries@ema.europa.eu by 31 December 2020.

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REVIEW

Marketing Authorization Applications Made to the European Medicines Agency in 2018–2019: What was the Contribution of Real-World Evidence?

Robert Flynn[1,2,†], Kelly Plueschke[1,†], Chantal Quinten[1], Valerie Strassmann[3], Ruben G. Duijnhoven[1,4], Maria Gordillo-Marañón[1,5], Marcia Rueckbeil[1,6], Catherine Cohet[1] and Xavier Kurz[1,*]

Information derived from routinely collected real-world data has for a long time been used to support regulatory decision making on the safety of drugs and has more recently been used to support marketing authorization submissions to regulators. There is a lack of detailed information on the use and types of this real-world evidence (RWE) as submitted to regulators. We used resources held by the European Medicines Agency (EMA) to describe the characteristics of RWE included in new marketing authorization applications (MAAs) and extensions of indication (EOIs) for already authorized products submitted to the EMA in 2018 and 2019. For MAAs, 63 of 158 products (39.9%) contained RWE with a total of 117 studies. For 31.7% of these products, the RWE submitted was derived from data collected before the planned authorization. The most common data sources were registries (60.3%) followed by hospital data (31.7%). RWE was mainly included to support safety (87.3%) and efficacy (49.2%) with cohort studies being the most frequently used study design (88.9%). For EOIs, 28 of 153 products (18.3%) contained RWE with a total of 36 studies. For 57.1% of these products, studies were conducted prior to the EOIs. RWE sources were mainly registries (35.6%) and hospital data (27.0%). RWE was typically used to support safety (82.1%) and efficacy (53.6%). Cohort studies were the most commonly used study design (87.6%). We conclude that there is widespread use of RWE to support evaluation of MAAs and EOIs submitted to the EMA and identify areas where further research is required.

"Real-world evidence" (RWE) has been defined as the information derived from analysis of routinely collected real-world data (RWD) relating to a patient's health status or the delivery of health care from a variety of sources other than traditional clinical trials.[1] The use of RWE to support regulatory decision making is not new. For decades, such data have been used in the postauthorization phase for safety signal evaluation, risk management, and for studies to support life cycle benefit-risk evaluation. A review of postmarketing assessments conducted by the European Medicines Agency (EMA) in 2019, showed that noninterventional studies commonly contributed to the evaluation of referrals related to both products' safety and efficacy.[2] Although randomized clinical trials (RCTs) represent the gold standard for studying drug efficacy because they prevent systematic bias in allocation of treatment,[3] they cannot answer certain questions, for example, effectiveness under normal conditions of use, and may not be practical in some circumstances, for example, in very rare diseases or populations. The rapid pace of change in the scientific and technological landscapes is shifting the regulatory landscape. An increasing number of medicines, such as advanced therapy medicinal products (ATMPs) and orphan products for conditions with significant unmet need, face challenges when aligning with the traditional drug development pathway, where traditional RCTs may be unfeasible, unethical, or less well suited to "precision medicines" that increasingly require analysis on subsets of patients on complex treatment pathways.[1,4,5]

Whereas methodological challenges remain before RWE can become a routine part of decision making across all parts of drug development,[6] RWE can still have a substantial impact on regulatory decision making, for example, by informing on the natural history of disease and standards of care, by contextualizing results of uncontrolled trials when used as comparator groups of patients for single arm trials, or by collecting follow-up data to generate postauthorization evidence on long-term safety and effectiveness of medicinal products.[1]

[1]Data Analytics and Methods Task Force, European Medicines Agency, Amsterdam, The Netherlands; [2]Medicines Monitoring Unit, University of Dundee, Dundee, UK; [3]Pharmacovigilance Office, Quality and Safety of Medicines, Human Medicines Division European Medicines Agency, Amsterdam, The Netherlands; [4]Amsterdam University Medical Centers, University of Amsterdam, Amsterdam, The Netherlands; [5]Institute of Cardiovascular Science, Faculty of Population Health, University College London, London, UK; [6]Department of Medical Statistics, University Hospital Aachen, Aachen, Germany. *Correspondence: Xavier Kurz (Xavier.Kurz@ema.europa.eu)
[†]Contributed equally as joint first authors.
The views expressed in this article are the personal views of the authors and may not be understood or quoted as being made on behalf of or reflecting the position of the European Medicines Agency or one of its committees or working parties.
Received March 1, 2021; accepted October 13, 2021. doi:10.1002/cpt.2461

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We conclude that there is widespread use of RWE to support evaluation of MAAs and EOIs submitted to the EMA and identify areas where further research is required.

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PERSPECTIVES

PERSPECTIVE

Real-World Evidence in EU Medicines Regulation: Enabling Use and Establishing Value

Peter Arlett[1,*], Jesper Kjær[2], Karl Broich[3] and Emer Cooke[1]

We outline our vision that by 2025 the use of real-world evidence will have been enabled and the value will have been established across the spectrum of regulatory use cases. We are working to deliver this vision through collaboration where we leverage the best that different stakeholders can bring. This vision will support the development and use of better medicines for patients.

Real-world data (RWD) and real-world evidence (RWE) are already used in the regulation of the development, authorization, and supervision of medicines in the European Union. Their place in safety monitoring and disease epidemiology are well-established while their evidentiary value for additional use cases, notably for demonstrating efficacy, requires further evaluation.[1] During the coronavirus disease 2019 (COVID-19) pandemic, RWE rapidly provided impactful evidence on drug safety, vaccine safety, and effectiveness and we were reminded of the importance of robust study methods and transparency.[2] Our vision, anchored in the European Medicines Regulatory Network (EMRN) strategy to 2025, is that by 2025 the use of RWE will have been enabled and the value will have been established across the spectrum of regulatory use cases.[3] Delivering this vision will support the development and use of better medicines for patients.

In December 2018, the US Food and Drug Administration (FDA) published its framework for RWE underpinned by three pillars: whether RWD are fit for use, whether the study design can provide adequate evidence, and whether the study conduct meets regulatory requirements.[4] In 2019 in the European Union, we published the OPTIMAL framework for RWE also consisting of three pillars: operational, technical, and methodological.[5] More recently, the EU approach places RWE in the wider context of big data and is guided by the priority recommendations of the Big Data Task Force. These recommendations are being implemented through the Big Data Steering Group and the second multiannual work plan was published in August 2021.[6] **Figure 1** represents the workplan with its 11 workstreams which will deliver our vision for RWE by 2025. The workplan places emphasis on collaboration across stakeholders and with international regulatory partners. This work also needs to be seen in the wider EU policy context, most notably the European Commission's plans for a European Health Data Space.[7]

Acknowledging different frameworks to conceptualize the challenges and opportunities of RWE, we believe the two main priorities for the European Union are to enable its use and establish its value for regulatory decision making. The EMRN is working to deliver on both priorities through a collaborative approach where we leverage the best that different stakeholders can bring, and where those stakeholders can complement the central role of industry in generating evidence.

ENABLING USE

To enable use, we are working on multiple fronts with our stakeholders, including patients, healthcare professionals, industry, regulatory and public health agencies, health technology assessment bodies, payers, and academia. We are initiating work to establish a data quality framework, not just for RWD but for all data used in regulatory decision making. We are striving to improve the discoverability (findability) of RWD through agreement of metadata for RWD and through a public catalogue of RWD sources[8] that builds on the early work of the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP). The ENCePP Guide on Methodological Standards in Pharmacoepidemiology,[9] extensively updated in 2021, is the core of our efforts to drive up the standards of study methods for RWE, and this is complemented by recently published guidance on conducting studies based on patient registries.[10]

The European Medicines Agency (EMA) and some national medicines agencies

[1]European Medicines Agency, Amsterdam, Netherlands; [2]Danish Medicines Agency, Copenhagen, Denmark; [3]BfArM, Bonn, Germany. *Correspondence: Peter Arlett (Peter.Arlett@ema.europa.eu)

Received March 1, 2021; accepted November 1, 2021. doi:10.1002/cpt.2479

1. DARWIN EU
2. Data quality
3. Data discoverability
4. Skills
5. Business processes
6. Analytics capability
7. Expert advice
8. Data governance
9. International collaboration
10. Stakeholder engagement
11. Veterinary data strategy

Figure 1 Big Data Steering Group workplan to 2023. Eleven workstreams to progress the real-world evidence (RWE) vision.[5]

CONCLUSION

Our vision is that by 2025 the use of RWE will have been enabled and its value will have been established across the spectrum of regulatory use cases.

We are committed to working with stakeholders to deliver this vision and in turn to support the development and use of better medicines for patients.

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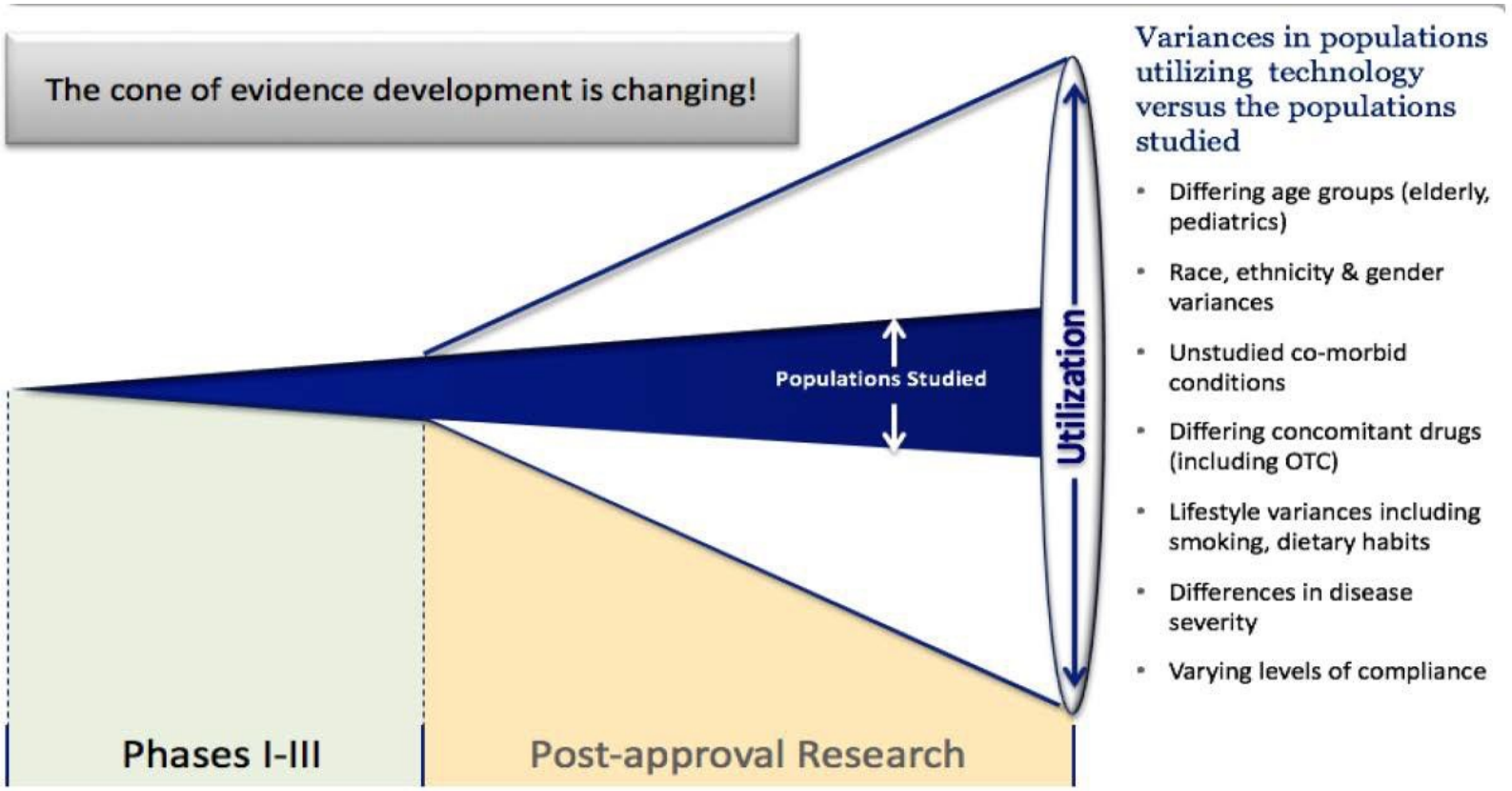
EMA possible issues on Real World Evidence



Variances in populations utilizing technology versus the populations studied

- Differing age groups (elderly, pediatrics)
- Race, ethnicity & gender variances
- Unstudied co-morbid conditions
- Differing concomitant drugs (including OTC)
- Lifestyle variances including smoking, dietary habits
- Differences in disease severity
- Varying levels of compliance

What impacts access to data?

- **GDPR regulations**
- **National and local guidelines**
- **Role of anonymity**
- **Storage and security**

How to reconcile RWE Data and Privacy

Could good faith legal safeguards make de facto Europe non-competitive?

- The new EU General Data Protection Regulation has been implemented on May 25th, 2018
  - Driven by the principle of data minimisation
  - Privacy by design and default
  - Right to opt out
  - Informed consent
  - Data ownership – personal / public / private
  - Need access to a sufficient amount of “good quality data”

How to reconcile RWE Data and Privacy

Could good faith legal safeguards make de facto Europe non-competitive?

- The new EU General Data Protection Regulation has been implemented on May 25th, 2018 but…
  - Driven by the principle of data minimisation (it will preclude machine learning)
  - Privacy by design and default (it will preclude predictive analytics)
  - Right to opt out (but how?)
  - Informed (really informed?) consent (impossible to predict to what I am giving consent to)
  - Data ownership – personal / public / private (issue is not on ownership but on access)
  - Need access to a sufficient amount of "good quality data" (indeed impossible with limits above)

- EU Regulators / Payers will have these additional problems:
  - Not enough competence (sometime none) with in-house and hands-on skills
  - Education of new types of assessors with very broad data science and life science knowledge.
  - Inability to certify and validate different data sources to be integrated among them.
  - Rule the emerging strong engagement by patients as data generators.

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Mod. from Pani L., IMI Stakeholder Forum 2019. Brussels, Belgium – June 12th , 2019

What could impact access to data for EMA?
Example: establishment of RWE and registries

- **Challenges of development of RWE data including provision of informed consent**
- **Clear communication with appropriate stakeholder involvement at all stages**
- Inclusions of outcomes relevant to patients
  - Collection and analysis of meaningful data
  - Impact on willingness to take part
- Data acquisition
  - Motivation/reward (patient and clinician)
  - Accuracy, quality and point of entry
  - Collected in a “real world” setting (e.g. community or primary care)
  - **Data protection and custodianship**

FDA's Current Thinking on Real-World Data seems different

Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products

Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document or the RealWorld Evidence Program, please email CDERMedicalPolicy-RealWorldEvidence@fda.hhs.gov

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Oncology Center of Excellence (OCE)

September 2021
Real World Data/Real World Evidence (RWD/RWE)

Sept. 2021

Data Standards for Drug and Biological Product Submissions Containing Real-World Data

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October 2021
Real-World Data/Real-World Evidence (RWD/RWE)

Oct. 2021

Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products

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For questions regarding this draft document, contact (CDER) Ansalan Stewart, 240-402-6631, or (CBER) Office of Communication, Outreach and Development, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Oncology Center of Excellence (OCE)

November 2021
Real World Data/Real World Evidence (RWD/RWE)

Nov. 2021

Considerations for the Use of Real-World Data and Real-World Evidence to Support Regulatory Decision-Making for Drug and Biological Products

Guidance for Industry

DRAFT GUIDANCE

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For questions regarding this draft document, contact (CDER) Tala Fakhouri, 301-837-7407, or (CBER) Office of Communication, Outreach and Development, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

December 2021
Real World Data/Real World Evidence (RWD/RWE)

45010354dft.docx
12/07/2021

Dec. 2021

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Real-World won't change how data are treated

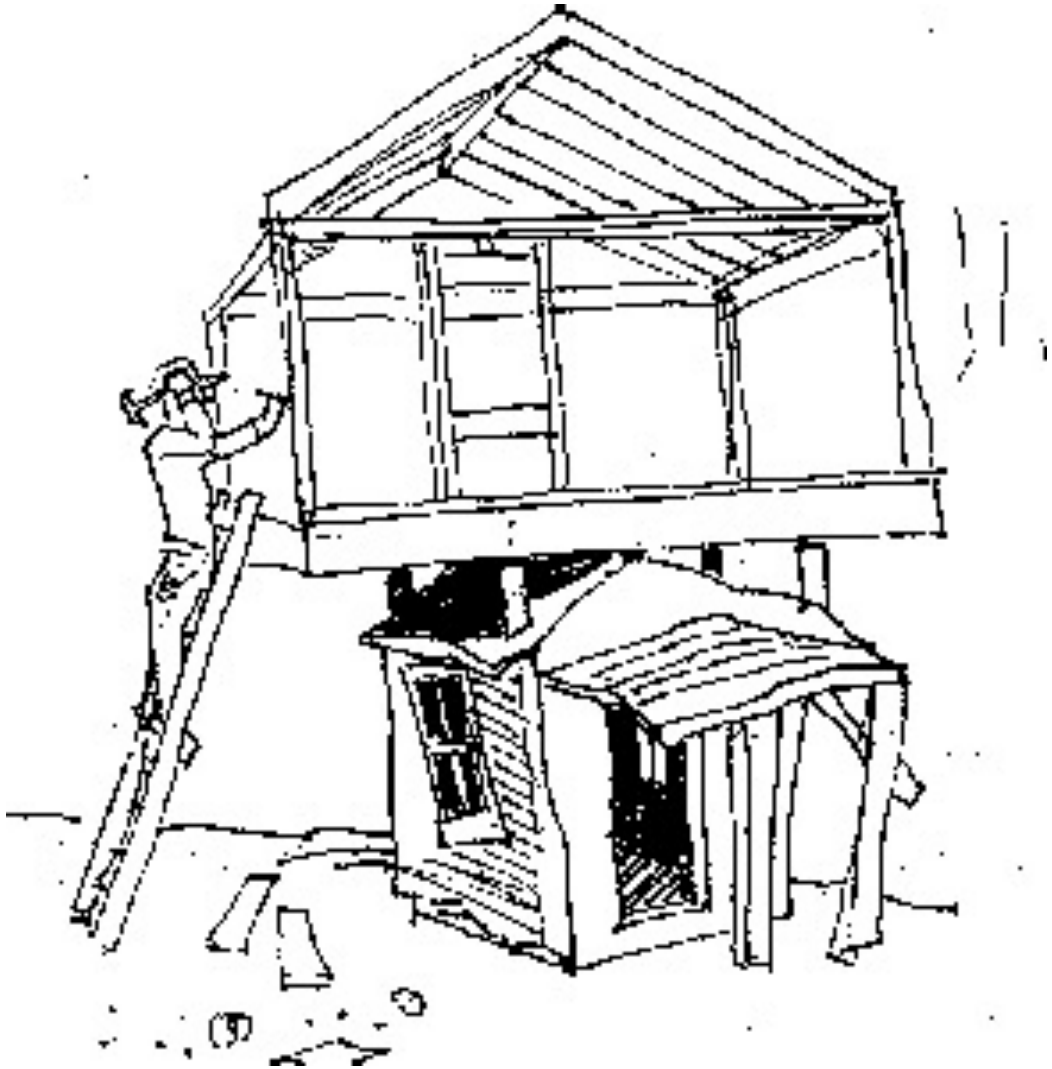
- FDA standard for "substantial evidence" stay unchanged
  - Goal is to distinguish the effect of the drug from other influences such as spontaneous change in disease course, placebo effect, or bias
  - Common practices:
    - Probabilistic control of confounding through randomization
    - Blinding
    - Controlled/standardized outcome assessment
    - Adjudication criteria
    - Audits

The FDA perspective on Data (big or small) is clear

- **Data** are raw measurements
- **Information** is obtained from data combined with critical context about what is being measured
- **Evidence** is derived from the analysis of information

http://blogs.fda.gov/fdavoice/index.php/2015/12/what-we-mean-when-we-talk-about-data/

Good evidence cannot be built on bad data



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RWE and Safety Signals

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Intussusception Risk after Rotavir Vaccination in U.S. Infants

W. Katherine Yih, Ph.D., M.P.H., Tracy A. Lieu, M.D., M.P.H., Martin Ku
David Martin, M.D., M.P.H., Cheryl N. McMahill-Walraven, M.S.
Richard Platt, M.D., Nandini Selvam, Ph.D., M.P.H., Mano Selva
Grace M. Lee, M.D., M.P.H., and Michael Nguyen, M.D.

ABSTRACT

BACKGROUND

International postlicensure studies have identified an increas
tion after vaccination with the second-generation rotavirus
a pentavalent vaccine) and Rotarix (RV1, a monovalent vac
association among infants in the United States.

Annals of Internal Medicine

ORIGINAL RESEARCH

Outcomes of Dabigatran and Warfarin for Atrial Fibrillation in Contemporary Practice

A Retrospective Cohort Study

Alan S. Go, MD; Daniel E. Singer, MD; Sengwee Toh, ScD; T. Craig Cheetham, PharmD, MS; Marsha E. Reichman, Ph
David J. Graham, MD, MPH; Mary Ross Southworth, PharmD; Rongmei Zhang, PhD; Rima Izem, PhD; Margie R. Gou
Monika Houstoun, PharmD; Katrina Mott, MS; Sue Hee Sung, MPH; and Joshua J. Gagne, PharmD, ScD

Background: Dabigatran (150 mg twice daily) has been associated with lower rates of stroke than warfarin in trials of atrial fibrillation, but large-scale evaluations in clinical practice are limited.

years; HR, 0.89 [CI, 0.72 to 1.09]) but were
intracranial bleeding (0.39 vs. 0.77 events per 10
HR, 0.51 [CI, 0.33 to 0.79]) and more likely to h
infarction (0.77 vs. 0.43 events per 100 person-
[CI, 1.22 to 2.90]). However, the strength and

Annals of Internal Medicine

ORIGINAL RESEARCH

Risk for Hospitalized Heart Failure Among New Users of Saxagliptin, Sitagliptin, and Other Antihyperglycemic Drugs

A Retrospective Cohort Study

Received: 1 May 2017 | Revised: 19 August 2017 | Accepted: 2 October 2017

WILEY

ORIGINAL REPORT

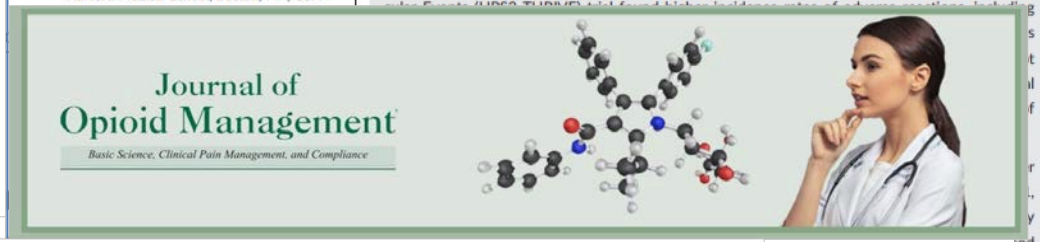
Safety assessment of niacin in the US Food and Drug Administration's mini-sentinel system

Joshua J. Gagne[1] | Monika Houstoun[2] | Marsha E. Reichman[2] | Christian Hampp[2] | James H. Marshall[3] | Sengwee Toh[3]

[1] Division of Pharmacoepidemiology and Pharmacoeconomics, Department of Medicine, Brigham and Women's Hospital and Harvard Medical School, Boston, MA, USA

Abstract

Purpose: The Heart Protection Study 2—Treatment of HDL to Reduce the Incidence of Vas-



Arch Womens Ment Health (2016) 19:969–977
DOI 10.1007/s00737-016-0637-1

ORIGINAL ARTICLE

Use of selective serotonin reuptake inhibitors (SSRIs) delivering liveborn infants and other women of child within the U.S. Food and Drug Administration's Mini-Sentinel program

Susan E. Andrade[1] · Marsha E. Reichman[2] · Katrina Mott[2] · Marilyn Pitts[2] · Caren Kieswetter[2] · Miriam Dinatale[2] · Marc B. Stone[2] · Jennifer Popovic[3] · Katherine Haffenreffer[3] · Sengwee Toh[3]

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Diabetes Care Volume 41, January 2018 39

Check for updates

Prospective Postmarketing Surveillance of Acute Myocardial Infarction in New Users of Saxagliptin: A Population-Based Study

*Sengwee Toh,[1] Marsha E. Reichman,[2] David J. Graham,[2] Christian Hampp,[2] Rongmei Zhang,[3] Melissa G. Butler,[4] Aarthi Iyer,[1] Malcolm Rucker,[1] Madelyn Pimentel,[1] Jack Hamilton,[5] Samuel Lendle,[5] and Bruce H. Fireman,[5] for the Mini-Sentinel Saxagliptin-AMI Surveillance Writing Group**

Diabetes Care 2018;41:39–48 | https://doi.org/10.2337/dc17-0476

…se or long-acting

…analgesics was approved by the … drug testing for individuals

…mmercially insured members. …09 and December 2013. …initiates meeting tolerance …ensing. We separately examined …a claim for a urine drug test in the …

…ntanyl opi-oid-tolerant-only …e and 40 percent of transdermal …. Use of urine drug testing for …s prior to initiation and 9 percent …

CONCLUSIONS: These results suggest potential areas for improving appropriate ER/LA opioid analgesic prescribing practices.

PMID: 29199397 DOI: 10.5055/jom.2017.0400

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RWE and Efficacy Signals

DRUG	INDICATION	APPROVED	DATA
Defitelio *(defibrotide sodium)*	Severe hepatic veno-occlusive disorder	2016	▪ Two prospective clinical trials enrolling 179 patients and **an expanded access study with 351 patients**
Lutathera *(lutetium 177 dotate)*	Gastroenteropancreatic neuroendocrine tumours (GEP-NETs)	2017	▪ Open-label clinical trial ▪ **Analysis of a subset of 360 patients who participated in an investigator sponsored, open-label, single-arm, single institution study of 1214 patients that started as an expanded access program**
Zostavax *(Zoster Vaccine Live)*	Prevention of herpes zoster (shingles) in persons 50 years of age and older	2018	▪ **Prospective, observational cohort study using electronic health records in Kaiser Permanente Northern California (KPNC)** to characterize the duration of protection in persons 50 years of age and older
Ibrance *(palbociclib)*	Men with certain types of advanced or metastatic breast cancer	2019	▪ Data from electronic health records and postmarketing reports of the **real-world use of Ibrance in male patients**

Bold = RWE

List not exhaustive

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Modified after Jacqueline Corrigan-Curay, JD, MD, FDA, May 2021

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A final take: Data Re-utilization

European Commission | CORDIS EU research results

HOME RESULTS PACKS RESEARCH*EU MAGAZINES NEWS & MEDIA PROJECTS & RESULTS ABOUT US LOG IN

HORIZON 2020

FrAmework for CIInicaL trIal participants daTA reutilization for a fully Transparent and Ethical ecosystem

Fact Sheet | Results

Objective

FACILITATE is a project built on a patient-centered, data-driven, technological platform where an innovative data sharing and re-use process allows the returning of clinical trial data to study participants within a GDPR compliant and approved ethical framework. FACILITATE starts-off by providing clear rules in a trusted ethical, legal and regulatory ecosystem before engaging patients as data generators. This avoids the current situation where clinical data are siloed in separate repositories without any possibility to be used beyond their original single-sided purpose. FACILITATE will provide the technological solutions to comply with GDPR in medical research by building on the empowered stakeholders' willingness to share and re-use their data. The FACILITATE Consortium was constituted by drawing from a broad range of capacities to tackle the ambitious challenges related to future clinical trials, such as preventive, long-term and real-world evidence trials. The Consortium took an innovative approach to the data return to study participants by asking them what they needed to be implemented to feel in a trusted ecosystem. This required all Consortium participants to leverage on their existing networks to bring together stakeholders at all levels in the decision-making chain, including patients, healthcare professionals, software designers, clinical trials repositories processors and controllers, ethicists, lawyers and other active regulators. Having obtained a consent on the data portability FACILITATE will re-use and cross-reference them with those contained in other repositories including RWE data captured across multiple settings and devices. FACILITATE will last 4 years and will participate in the extended Pilot on Open Research Data of Horizon 2020. Its strategy represents a unique and innovative opportunity for medicines drug development and regulation to better understand the clinics of diseases, and to evaluate the effectiveness of products in the healthcare system.

Project Information

FACILITATE

Grant agreement ID: 101034366

Start date 1 January 2022

End date 31 December 2025

Funded under
H2020-EU.3.1.
H2020-EU.3.1.7.

Overall budget
€ 6 886 711

EU contribution
€ 3 260 000

Coordinated by
UNIVERSITA DEGLI STUDI DI MODENA E REGGIO EMILIA
Italy

FACILITATE

efpia

imi innovative medicines initiative

UNIMORE

https://cordis.europa.eu/project/id/101034366

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Data Re-utilization: The FACILITATE Project

European Commission | CORDIS EU research results

Objective

FACILITATE is a project built on a patient-centered, data-driven, technological platform where an innovative data sharing and re-use process allows the returning of clinical trial data to study participants within a GDPR compliant and approved ethical framework. FACILITATE starts-off by providing clear rules in a trusted ethical, legal and regulatory ecosystem before engaging patients as data generators. This avoids the current situation where clinical data are siloed in separate repositories without any possibility to be used beyond their original single-sided purpose FACILITATE will provide the technological solutions to comply with GDPR in medical research by building on the empowered stakeholders' willingness to share and re-use their data. The FACILITATE Consortium was constituted by drawing from a broad range of capacities to tackle the ambitious challenges related to future clinical trials, such as preventive, long-term and real-world evidence trials. The Consortium took an innovative approach to the data return to study participants by asking them what they needed to be implemented to feel in a trusted ecosystem. This required all Consortium participants to leverage on their existing networks to bring together stakeholders at all levels in the decision-making chain, including patients, healthcare professionals, software designers, clinical trials repositories processors and controllers, ethicists, lawyers and other active regulators. Having obtained a consent on the data portability FACILITATE will re-use and cross-reference them with those contained in other repositories including RWE data captured across multiple settings and devices. FACILITATE will last 4 years and will participate in the extended Pilot on Open Research Data of Horizon 2020. Its strategy represents a unique and innovative opportunity for medicines drug development and regulation to better understand the clinics of diseases, and to evaluate the effectiveness of products in the healthcare system.

Project Information

FACILITATE

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FACILITATE

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