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Welcome to the Sentinel Innovation and Methods Seminar Series

The webinar will begin momentarily

- Please visit www.sentinelinitiative.org for recordings of past sessions and details on upcoming webinars.
- Note: closed-captioning for today's webinar will be available on the recording posted at the link above.

U.S. FOOD & DRUG ADMINISTRATION

Overview of CDER OMP's Real-World Evidence Demonstration Projects

Sentinel Innovation Center and Sentinel Operations Center Innovation & Methods Webinar Series; August 5, 2024

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Disclosure and Disclaimers

- **No conflicts of interest to disclose**

- **Views expressed are those of the presenter and are not intended to convey official US Food and Drug Administration policy or guidance**

- **Mention of a commercial product should not be construed as actual or implied endorsement**

Outline

- Background to FDA RWE Program
- Overview of 2020 U01 projects
- Overview of 2023 U01 projects
- Summary

Background

‘FDA At a Glance’ (2024)

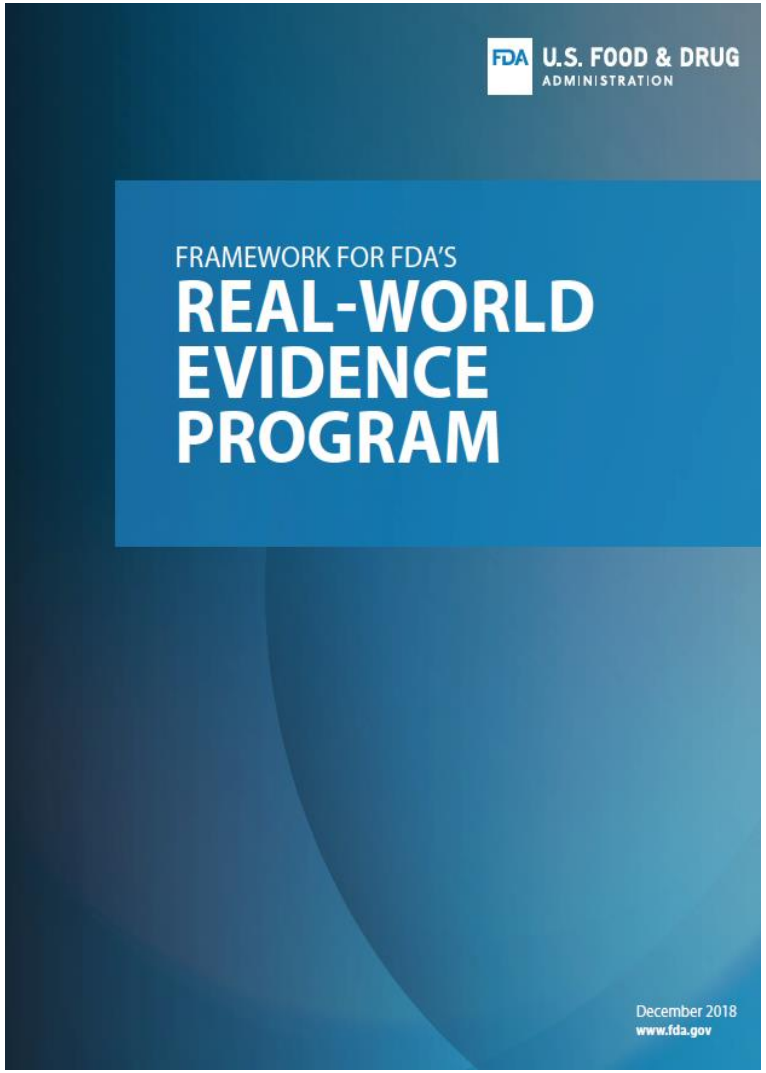
- >18,000 employees (domestic and global)
- Oversees safety of >$3.6 trillion worth of food, tobacco, and medical products
- Accounts for 21 cents of every dollar spent by U.S. consumers
- $6.7B budget (53% federal; 47% industry):
  - Human drugs 34% & biologics 7%
  - Food 18%
  - Other (e.g., devices, animal, tobacco) 41%

https://www.fda.gov/media/175664/download

21st Century Cures Act (December 2016)

- **FDA *shall establish* a program to evaluate the potential use of real-world evidence (RWE) to:**
  - **Support a new indication for a drug approved under section 505(c)**
  - **Satisfy post-approval study requirements**
- **FDA *shall establish* a draft framework [*by December 2018*]:**
  - **Describe sources of such evidence, gaps in data collection activities, etc.**
- **FDA *shall issue* draft guidance for industry [*by December 2021*]:**
  - **Describe "appropriate standards and methodologies"**

FDA RWE Framework (2018)



- **Applies to:**
  - **Center for Drug Evaluation and Research (CDER)**
  - **Center for Biologics Evaluation and Research (CBER)**
  - **Oncology Center of Excellence (OCE)**

- **Multifaceted program to implement RWE:**
  1) **Internal processes**
  2) **External stakeholder engagement**
  3) **Guidance development**
  4) **Demonstration (research) projects** ←

https://www.fda.gov/media/120060/download

Internal Processes & External Engagements

Real-World Evidence Subcommittee *internal* activities:

- **FDA staff from multiple centers and offices**
- **Providing oversight of policy development on RWE (e.g., guidances)**
- **Offering resources and leadership (e.g., consults to review divisions)**

Real-World Evidence Subcommittee *external* activities:

- **"listening sessions" on initiatives from sponsors, vendors, etc.**

***Additional* activities, beyond the Subcommittee, include:**

- **FDA or Center-level public meetings on RWE-related topics**
- **FDA webinars and speaking engagements**
- **Publications addressing issues related to RWE**

FDA RWD/RWE Guidance (2021-2024)

Topic	Category	Status
EHRs and medical claims data	Data considerations	final issued
Registry data	Data considerations	final issued
Data standards	Submission of data	final issued
Regulatory considerations	Applicability of regulations	final issued
Externally controlled trials	Design considerations	draft issued
Non-interventional studies	Design considerations	draft issued
RCTs in clinical practice settings	*Design considerations*	*in development*
Submitting RWE	Procedural	final issued

https://www.fda.gov/science-research/real-world-evidence/center-biologics-evaluation-and-research-center-drug-evaluation-and-research-real-world-evidence

Misconceptions Regarding RWD & RWE

Frequent instances of:

- ***Misconception #1 – RWD & RWE are new concepts*: "In reality, sources of data and types of study design haven't fundamentally changed, but electronic access to more detailed clinical data is evolving & the data are becoming more relevant and reliable"**

- ***Misconception #2 – A simple dichotomy of randomized trials vs. observational studies exists*: "In reality, clinical trials are defined by assignment of treatment according to an investigational protocol, and single-arm trials face challenges similar to those in observational studies in determining whether difference in clinical outcomes (compared to an external control group) represent actual treatment effects"**

*NEJM 386;18,May 5,2022:*1680-1682
Pharmacoepidemiol Drug Saf. 2024 Jan;33(1):e5715

Demonstration (Research) Projects: Overview

FDA supports projects evaluating the use of RWD to generate RWE in regulatory decision-making. Diverse portfolio uses various mechanisms:

- **U01 Cooperative Agreements**
- **Broad Agency Announcements (BAA)**
- **Centers for Excellence in Regulatory Science and Innovation (CERSI)**
- **Inter-Agency Agreements**
- **Sentinel & FDA-Catalyst**

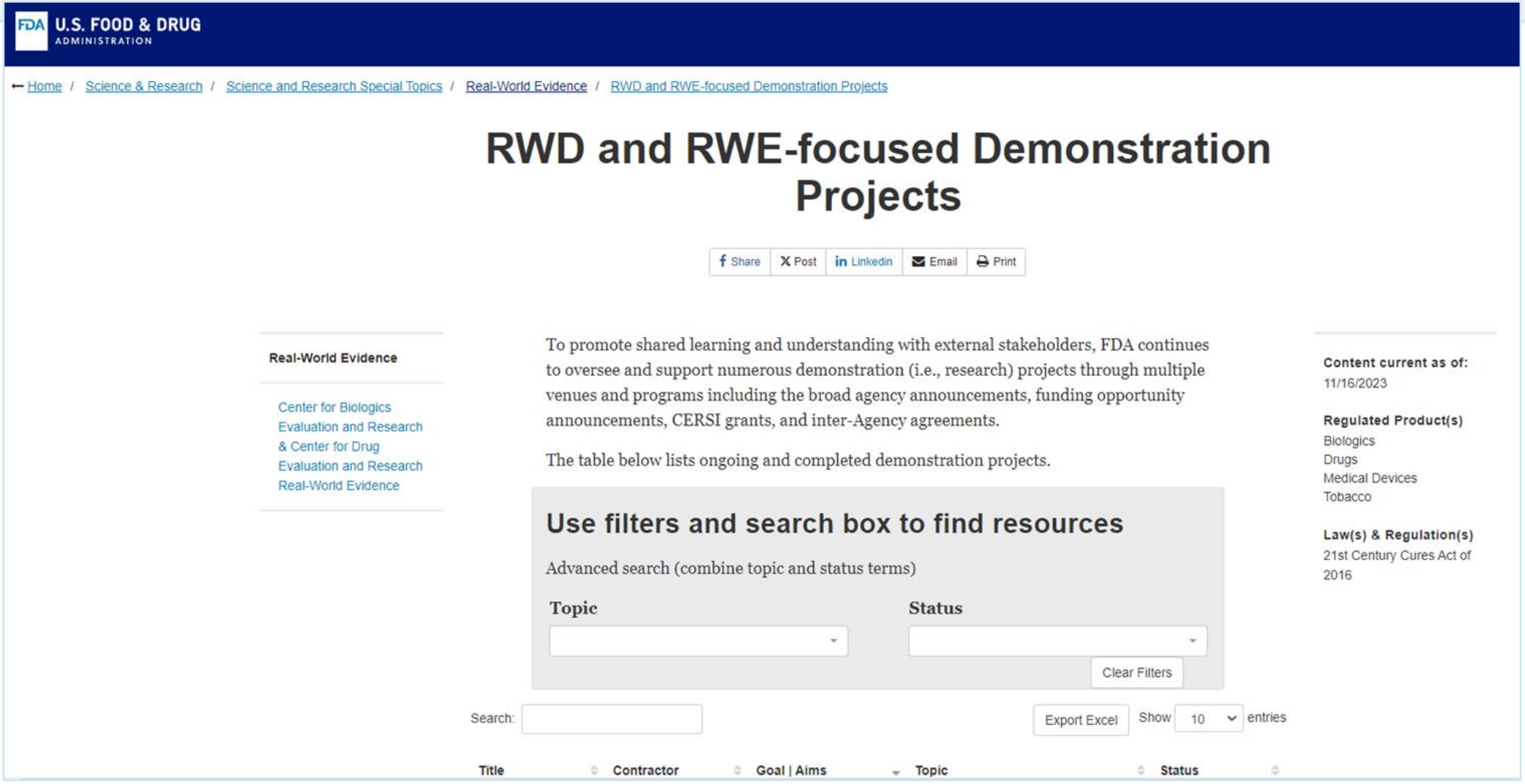
CERSI

Sentinel

Demonstration (Research) Projects: Overview

- Address gaps in methodologic approaches for studies using RWD
- OMP and other FDA offices provide technical expertise
- Awardees have specialized expertise from different backgrounds:
  - academia
  - industry
  - other

Demonstration Projects: Public-Facing Webpage



https://www.fda.gov/science-research/real-world-evidence/rwd-and-rwe-focused-demonstration-projects

U01 Awards

Four U01 awards were executed in 2020 (RFA-FD-20-030) and 2023 (RFA-FD-23-025) to examine the use of RWD to generate RWE in regulatory decision-making.

https://www.fda.gov/drugs/science-and-research-drugs/fda-grant-awards-projects-supporting-use-real-world-data-generate-real-world-evidence-regulatory

Specific goals of today's talk:

- **Summarize the status of the 2020 RWE U01 projects and provide an update on the 2023 U01s**

2020 U01 RWE Demonstration Projects

AWARD TO: University of North Carolina & Genentech

Applying novel statistical approaches to develop a decision framework for hybrid randomized controlled trial designs which combine internal control arms with patients' data from real-world data sources

PI Herb Pang and Jiawen Zhu

University of North Carolina and Genentech - Background

- Hybrid randomized controlled trial designs supplement internal control arms with patient level data from real-word data sources
- Dissimilarity between internal and external controls has the potential to negatively impact the trial (e.g., decrease power, inflate type I error rate)
- Bayesian methods which adaptively adjust the influence of external controls on the analysis of the trial data can help to mitigate these issues for complex trial designs
- Team were developing an adaptive borrowing approach with subject-specific discounting parameters specifically suited for time-to event analyses

University of North Carolina and Genentech - Overall Objective

Apply novel statistical approaches and data from completed clinical trials, RWD sources, and simulation studies to evaluate hybrid trial designs and develop a novel decision framework for reliable application of the methods when using hybrid clinical trials

University of North Carolina and Genentech - Specific Aims

(1) Evaluation of the hybrid designs and their operating characteristics, when combined with sequential monitoring and possibly use of adaptive randomization

(2) Assessment of possible extensions of the method beyond time-to-event settings when applied to diseases in different therapeutic areas, including rare diseases

(3) Development a recommended list of sensitivity analyses when using hybrid study designs

(4) Development R Packages and SAS macros to support study design simulations; offer training workshops on methods and use of the packages

University of North Carolina & Genentech - Select Outputs to Date

Publications

- **Fu, C., Pang, H., Zhou, S., & Zhu, J. (2023). Covariate handling approaches in combination with dynamic borrowing for hybrid control studies. Pharmaceutical statistics, 22(4), 619–632.**
- **Fu C, Pang H, Zhu J. (2022). Evaluating the impact of different randomization ratios in designing hybrid control trials, Biopharmaceutical Report, Vol 29 (2), Summer (p. 22-32)**
- **R Li, R Lin, J Huang, Lu Tian, J Zhu (2023). A frequentist approach to dynamic borrowing, Biometrical Journal.**
- **Kwiatkowski, E., Zhu, J., Li, X., Pang, H., Lieberman, G., & Psioda, M. A. (2024). Case weighted power priors for hybrid control analyses with time-to-event data. Biometrics, 80(2), ujae019.**
- **Zhou, X., Pang, H., Drake, C., Burger, H. U., & Zhu, J. (2024). Estimating treatment effect in randomized trial after control to treatment crossover using external controls. Journal of biopharmaceutical statistics, 1–29.**
- **Zhou, X., Zhu, J., Drake, C., & Pang, H. Causal Estimators for Incorporating External Controls in Randomized Trials with Longitudinal Outcomes. Journal of the Royal Statistical Society Series A, in press**

- **Presentations**
  - Multiple (FDA statistical association, CIRS, American Statistical Association CSP, DIA and BIO conference)

University of North Carolina & Genentech - Select Outputs to Date

- Short courses offered
  - Design and Analysis of Randomized Clinical Trials with Real-World Data, Society for Clinical Trials 2023, Baltimore MD
  - Regulatory Industry Statistics Workshop 2023 co-organized by ASA BIOP Section and the FDA
- Open-source tool
  - psborrow: Bayesian dynamic borrowing R tool for complex innovative trial designs, Y Lu, A Lin, H Pang, Zhu, J, Biopharmaceutical Report, Vol 28 (3), Summer 2021 (p. 11-19)
  - psborrow2 now on CRAN https://cran.r-project.org/web/packages/psborrow2/index.html and github https://genentech.github.io/psborrow2/index.html
- Expert workshops
  - 2021, 2022, 2024 provided an overview of progress to date and solicited feedback from experts

AWARD TO: Brigham and Women's Hospital and Harvard Medical School

Enhancing evidence generation by linking randomized clinical trials (RCTs) to real-world data (RWD)

PI Elisabetta Patorno

Brigham and Women's Hospital and Harvard Medical School - Background

- **RCTs may be limited by missing data, inability to capture important effectiveness and safety outcomes, short follow-up duration, and lack of generalizability**
- **Linking RCTs to real-world data (RWD), such as health insurance claims and electronic health records, may enhance traditional RCTs by addressing these shortcomings**
- **Need to demonstrate value of linked RCT-RWD design and to advance methods used for analysis of observational studies**

Brigham and Women's Hospital and Harvard Medical School - Overall Objective

To use linked RCT-RWD to develop and validate methods that can enrich both randomized and non-randomized studies

Brigham and Women's Hospital and Harvard Medical School - Specific Aims

Using two case studies: The INfluenza Vaccine to Effectively Stop Cardio Thoracic Events and Decompensated Heart Failure (INVESTED) trial and Dapagliflozin Evaluation to Improve the LIVEs of Patients With PReserved Ejection Fraction Heart Failure (DELIVER) trial

Aim 1. To enhance RCT findings using long-term, validated, RWD-based outcome measures

Aim 2. To develop methods for using RWD to replace or impute missing RCT data

Aim 3. To generalize RCT results to broader patient populations in clinical practice

Aim 4: To develop methods to identify sources of discrepant results between RCTs and RWD-based studies

Brigham and Women's Hospital and Harvard Medical School - Select Outputs To Date

- **Challenges include delays due to COVID-19 pandemic and issues obtaining consent for linkage**
  - **Feasibility challenges may require some modifications to the Aims**
  - **Additional RCT may be utilized**

- **Publications:**
  - ***High-dose vs. standard-dose influenza vaccine and cardiopulmonary hospitalization or mortality: emulating the INVESTED trial using insurance claims data*** ***Clin Pharmacol Ther. 2024 Jan;115(1):126-134; also presented as a poster at the 39th International Conference on Pharmacoepidemiology, Aug 25-27, 2023***

- **Presentations**
  - **Poster ISPE 2024 (upcoming)** ***"Enhancing Evidence Generation by Linking Randomized Clinical Trials to Real-World Data: A Validation Study from the INVESTED-Medicare Linkage"***

AWARD TO: Critical Path Institute

Advancing standards and methodologies to generate real-world evidence from real-world data through a neonatal pilot project

PI Klaus Romero

Critical Path Institute - Background

- **Urgent need to improve survival and outcomes in pre-term neonates where most drug use is off-label**

- **Minimal new drug development in neonates and many existing drugs have insufficient evidence to support safety, efficacy, and dosage**

- **Paucity of relevant data, limited data sharing, and lack of data standards have greatly limited drug development in neonates.**
  - Need to collect, curate and standardize RWD to generate RWE to attempt to address major challenges in neonatal drug development

Critical Path Institute - Overall Objective

Develop a real-world data and analytics platform and conduct a pilot project to facilitate the use of RWD to generate RWE in neonates, with learnings broadly applicable to other therapeutic areas

Critical Path Institute - Specific Aims

Aim 1: Develop a Real-World Data and Analytics Platform (RW-DAP) which can access RWD to generate RWE

- data collected by leveraging existing C-Path Data Collaboration Center (DCC) processes and infrastructure

Aim 2*:* Execute a pilot project that generates RWE to support regulatory decision making in neonatal drug development:

- **Data from Aim 1 will address two key challenges:**
  - 1) Lack of actionable reference values for routine laboratory tests (as a function of gestational/postnatal age)
  - 2) Lack of a disease progression model for bronchopulmonary dysplasia (BPD)

Aim 3: Identify gaps that hinder the optimal use of existing RWD:

- Highlight key lesson from Aims 1 and 2 specific to neonates relating to the access, quality, extraction, curation, standardization, and analysis of RWD

Critical Path Institute - Select Outputs To Date

- **Acquired anonymized EHR and registry data on 380,000 individual patient-level records from 30 hospitals in US and abroad**
  - **currently under curation and harmonization**
- **Presentations:**
  - **Extracting Lab Value Reference Ranges From Real-world NICU Data. Poster presentation at DIA, June 2024**
  - **Challenges in curating Real World Data for modeling: A Bronchopulmonary Dysplasia case study. *Accepted presentation at the 15th Annual Conference on Pharmacometrics (November 2024)**
- **Publications:**
  - ***Real-World Evidence for Neonatal Drug Development: Challenges and Opportunities.***
    - ***J Pediatr. 2024 Feb:265:113806. doi: 10.1016/j.jpeds.2023.113806. Epub 2023 Oct 31***
  - ***Publication Recommendations to Report Laboratory Data of Neonates - a Modified Delphi Approach.***
    - ***Pediatr Res. 2024 Jul;96(1):81-88. doi: 10.1038/s41390-024-03094-7. Epub 2024 Mar 6***

AWARD TO: Verantos

Transforming Real-world evidence with Unstructured and Structured data to advance Tailored therapy (TRUST)

PI Dan Riskin

Verantos - Background

- **Regulators, payers, and providers are increasingly incorporating RWE insights into their decision-making processes**

- **Current approaches may benefit from greater rigor**

- **Need for understanding on how data quality influences RWE assertions to promote application of informative and reliable RWE**

Verantos - Overall Objective

Examine how measurements of data quality can be distilled into research practice and how data quality can impact RWE. Enable high-validity evidence through advanced data and technologies.

Verantos - Specific Aims

1. Using severe asthma as a testbed, develop datasets from over 65 hospitals and 1500 clinics across the US, reflecting cohorts with varying accuracy, completeness, and traceability

2. Across cohorts, assess the impact of data reliability on cohort characteristics and outcomes by:

- Comparing distributions of demographic and clinical characteristics and comorbidities at baseline
- Comparing cumulative incidence of medication uptake during follow-up
- Comparing event rates, incidence rates, and cumulative incidence of asthma exacerbation during follow-up
- Comparing time to death during follow-up
- Comparing all-cause and asthma-related healthcare resource utilization rates during follow-up

Verantos - Select Outputs To Date

- **Presentations**
  - **Poster CHEST conference October 2023**

    "Generating real-world evidence in asthma: Advanced approaches to achieve high validity"
  - **Podium DIA June 2024**

    "Creating High-validity Real-world Data (RWD) through Tokenization and Data Curation"

- **Publications**
  - **Accepted Manuscript (Journal: *Epidemiology*, tentative publication date: Jan 2025)**

"Advanced Approaches to Generating High-Validity Real-World Evidence in Asthma"

2023 U01 RWE Demonstration Projects

AWARD TO: JOHNSON & JOHNSON

Developing Novel Methods to Enable Robust Comparison of Real-World Progression Free Survival (rwPFS) and Clinical Trial PFS in Multiple Myeloma (MM)

PIs: Khaled Sarsour & Ashita Batavia

Academic collaborators at other institutions

JOHNSON & JOHNSON – Background

- **RWE has played a limited role in the pre-approval MM setting**
  - alignment of RWD and RCT outcomes remains a challenge
  - opportunity to study effectiveness of MM therapies in under-represented populations and outside of a trial setting
- **Bias due to measurement error limits comparison of real-world progression free survival (PFS) and trial PFS**
  - **Misclassification bias:** In non-trial settings International Myeloma Working Group (IMWG) response criteria are applied differently. Discrepancies in progression assessment can result in misclassification of real-world progression events
  - **Surveillance bias**: Time-to event outcome (e.g., PFS) may differ based on progression assessment frequency and timing. Trial protocols specify a schedule of assessments that may not occur in routine care practice patterns

JOHNSON & JOHNSON – Overall Objective

Develop novel methods to address misclassification and surveillance biases in RWD, allowing for more robust comparison of rwPFS and trial PFS in MM

JOHNSON & JOHNSON – Specific Aims

AIM 1: Develop novel methods to correct for misclassification bias of IMWG progression criteria among newly diagnosed multiple myeloma (NDMM) patients in real world settings

AIM 2: Develop novel methods to correct for surveillance bias in disease assessment among NDMM patients in real world settings

AIM 3: Apply bias correction methods in trial eligible RWD and compare to the control arms of Janssen MM trials

JOHNSON & JOHNSON -Select outputs to date

- **Publications**
  - Measurement Error and Bias in Real-World Oncology Endpoints when Constructing External Control Arms. *Front. Drug Saf. Regul., 18 July 2024*
- **Presentations**
  - Developing novel methods for aligning real-world & trial endpoints in Multiple Myeloma. *American Statistical Association, Biopharmaceutical Section Webinar, 8 March 2024*
  - Poster-Comparing Progression-Free Survival Between Real-World and Clinical Trials in Multiple Myeloma: Surveillance Bias Due to Variations in Disease Assessments. *ASA BIOP Regulatory-Industry Statistics Workshop, 25-27 Sept 2024*
  - Poster-Quantifying misclassification bias from real-world versus trial-derived progression-free survival in multiple myeloma. *ASA BIOP Regulatory-Industry Statistics Workshop, 25-27 Sept 2024*

AWARD TO: ECOG-ACRIN
Cancer Research group

Prospective Non-Interventional Study Comparing Standard of Care Osimertinib +/- Chemotherapy for EGFR-Mutated Non-Small Cell Lung Cancer Patients

PIs: Peter O'Dwyer, Al Benson, Suzanne Cole

ECOG-ACRIN – Background

- **Interest in testing the feasibility of collecting data from patients in academic and community oncology practice sites and using it to determine effectiveness of approved therapies for metastatic EGFR-mutant lung cancers in routine clinical practice**

- **Patients with metastatic EGFR-mutant lung cancers receive EGFR tyrosine kinase inhibitors (TKIs) as first-line treatment**
  - Osimertinib TKI has shown superior PFS in the first-line setting compared to earlier generation standard-of-care EGFR TKI (erlotinib, gefitinib)
  - Osimertinib approved by FDA in 2015 for use in the first-line setting, positioning it as a first-line single agent EGFR TKI
  - Recent publication showed first-line treatment with osimertinib+chemotherapy led to significantly longer PFS than osimertinib monotherapy

ECOG-ACRIN – Overall Objective

Develop an infrastructure to conduct a study comparing outcomes in patients with EGFR-mutated NSCLC not being treated in a clinical trial but receiving osimertinib +/- chemotherapy in routine care

ECOG-ACRIN – Specific Objectives

Primary Objectives:

1. **To prospectively identify and collect key data elements, including exposures, covariates and outcomes, in routine clinical care for use in non-randomized treatment comparisons in patients with EGFR-mutated NSCLC**
2. **Conduct a prospective non-interventional study to assess rwPFS and rwOS in EGFR- mutated NSCLC patients treated with osimertinib +/- chemotherapy**

Secondary objectives:

1. **Identify predictors of outcomes in both treatment groups**
2. **Assess time to treatment discontinuation (rwTTD) and overall survival (OS)**
3. **Model differences in rwPFS and PFS in patients randomized to the osimertinib arm of the EA5182 trial including assessment of patient characteristics and potential confounders**

ECOG-ACRIN – Additional Study Details

- 250 participants to be recruited per treatment group
- Treatment assignment per routine care
- Study activation planned Q3 2024, enrollment completion target Q1 2026
- Protocol currently under review at 27 sites

AWARD TO: HARVARD UNIVERSITY

Scalable and Generalizable Real-world Evidence on Unstructured Efficacy and Adverse Effect Outcomes for Chronic Diseases

PIs: Tianxi Cai & Florence Bourgeois

Harvard University – Background

RWD are limited to assess outcomes of disease modifying treatments (DMTs) in rheumatoid arthritis (RA) and multiple sclerosis (MS)

- Lack of computable information on disease progression measures
  - Clinical data on disease progression (e.g., disability) is typically in clinical narrative EHR notes (unstructured text) rather that structured formats

- Free text documentations by physicians in the medical notes provide unique opportunities to impute these “imperfect outcomes“

Can disease progression outcomes be generated from EHR data by 1) linking information in EHRs to data on disease activity in registries and 2) building natural language processing algorithms for disease activity scores and remission status?

Harvard University – Overall Objective

Develop methods to generate reproducible and generalizable RWE on unstructured efficacy and adverse event (AE) outcomes used in the evaluation of therapies for rheumatoid arthritis and multiple sclerosis

Harvard University – Specific Aims

Aim 1: Link disease activity and progression data from registries to EHR data

Aim 2: Correct for noise in DMT prescription data; impute information for DMTs

Aim 3: Combine EHR data from multiple healthcare systems through federated learning to ensure generalizability of RWE

- Conduct observational studies to assess risks of adverse events associated with DMTs using multi-EHR data for RA and MS

Harvard University-Select outputs to date

Publications

- Wen J, Hou J, Bonzel CL, Zhao Y, Castro VM, Gainer VS, Weisenfeld D, Cai T, Ho YL, Panickan VA, Costa L, Hong C, Gaziano JM, Liao KP, Lu J, Cho K, Cai T. LATTE: **Label-efficient incident phenotyping from longitudinal electronic health records.** Patterns (N Y). 2023 Dec 27;5(1):100906. doi: 10.1016/j.patter.2023.100906.

- Nogues IE, Wen J, Zhao Y, Bonzel CL, Castro VM, Lin Y, Xu S, Hou J, Cai T. **Semi-supervised Double Deep Learning Temporal Risk Prediction (SeDDLeR) with Electronic Health Records.** J Biomed Inform. 2024 Jul 14;157:104685. doi: 10.1016/j.jbi.2024.104685. Epub ahead of print.

Presentation

- American Statistical Association, Biopharmaceutical Section webinar. **Deriving reliable real-world evidence with electronic health records data.** March 8th, 2024

AWARD TO: DUKE UNIVERSITY & NORTH CAROLINA STATE UNIVERSITY

Methods to improve efficiency and robustness of clinical trials using information from real-world data with hidden bias

PIs: Xiaofei Wang & Shu Yang

(co-investigators from Duke university, North Carolina State University, Brown University, and Eli Lily)

Duke & North Carolina State Universities – Background

- **Leveraging RWD for hybrid RCT external control arms (ECA) raises several concerns due to lack of randomization, potential confounding, and selection biases**
  - Hidden biases may arise from selection bias, a lack of concurrency, difference in variable definitions, or unmeasured confounding, which could result in biased treatment effect estimates
  - Numerous borrowing or adjustment procedures proposed to overcome these challenges, but many have limitations
- **Need for more flexible and robust statistical methodology that can simultaneously address multiple sources of hidden biases including unmeasured confounding**

Duke & North Carolina State Universities – Overall Objective

Develop and test methodology that enables more efficient and robust integration of RWD in clinical trials while addressing sources of hidden biases

Duke & North Carolina State Universities – Specific Aims

Aim 1: Develop and test a Bayesian sensitivity analysis framework for hidden biases when augmenting RCTs with controls from RWD

Aim 2: Develop and test efficient and robust integrative estimators of treatment effects for selectively borrowing external controls

Aim 3: Develop software packages, case studies, and hold workshops to facilitate uptake of the methods developed in Aims 1 and 2

Duke & North Carolina State Universities - Select Outputs To Date

Manuscript under review

- Gao, C., Yang, S., Shan, M., Ye, W., Lipkovich, I., & Faries, D. (2023). Integrating Randomized Placebo-Controlled Trial Data with External Controls: A Semiparametric Approach with Selective Borrowing. Biometrika

Presentations

- Strategies for utilizing external controls in randomized trials while mitigating bias in treatment effect estimation. *The 7th Stat4Onc Annual Symposium, UConn, Storrs, Connecticut, USA, May 2024*

- "Methods to Improve Efficiency and Robustness of Clinical Trials Using Information from Real-World Data with Hidden Bias" ASA BIOP DL webinar August 30, 2024

Summary

- **CDER-OMP has a comprehensive portfolio of demonstration projects funded under specific mechanisms**
  - total of eight U01s are in progress

- **These projects address key gaps in methodologic approaches for studies using real-world data**

- **Even when project aims are not fully achieved, the findings provide lessons learned for FDA and the wider community**

- **To date, work products include publications and trainings for FDA staff and others based on demonstration project findings**

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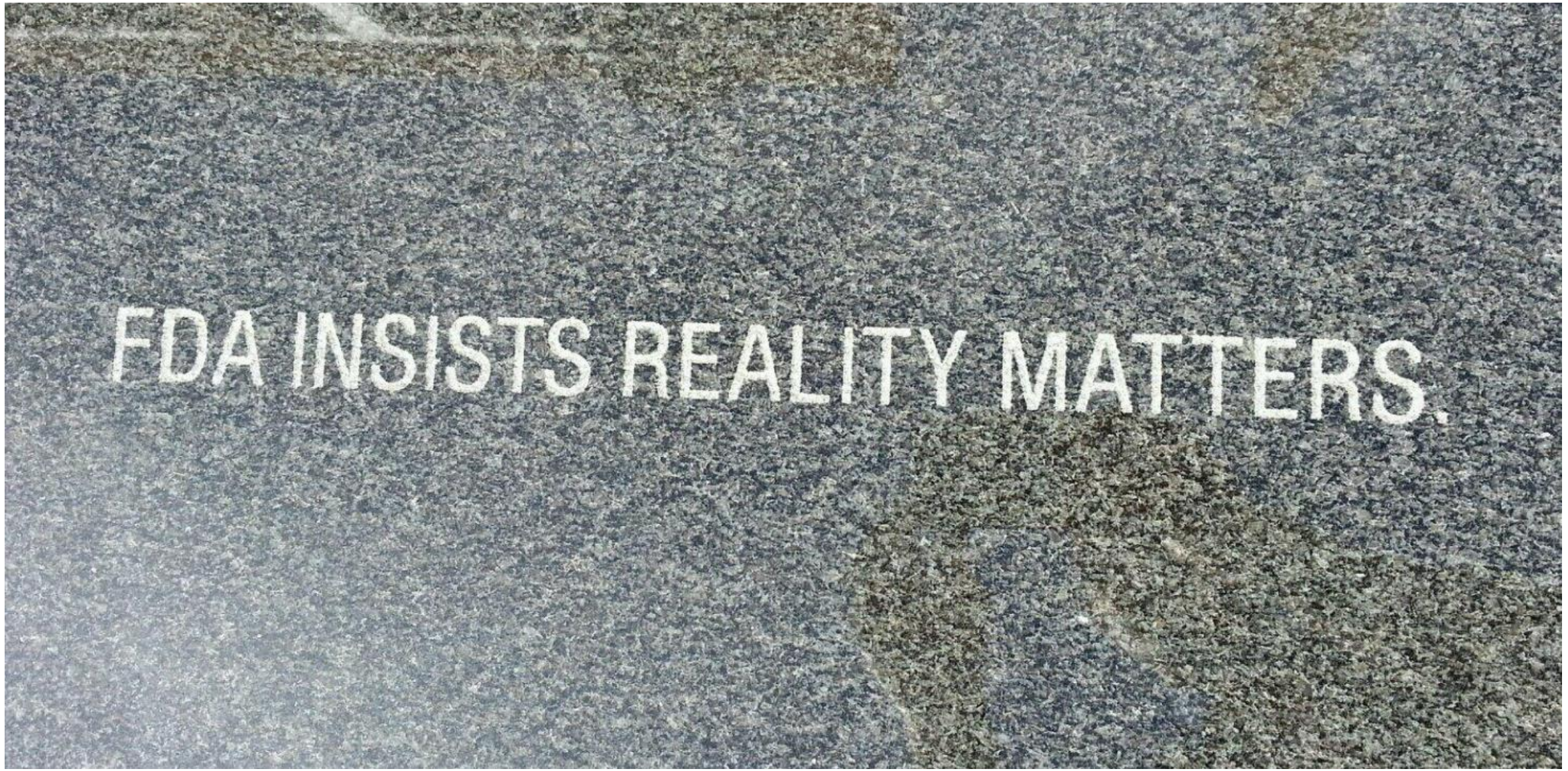
OPT: An Masaro, Melissa Lestini


FDA INSISTS REALITY MATTERS.

Thank you

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