

ADVANCING RWE

Leveraging Data from Routine Care as a Research Tool

GOAL

Every day many patients are treated for cancer, and each patient's experience generates data on a treatment's effectiveness and safety to provide valuable insights and knowledge to advance cancer research and improve patient care. These data are also known as real-world data (RWD), and Friends of Cancer Research (*Friends*) leads critical work to advance methodology for using RWD as a research tool more effectively. *Friends'* unique collaborations lead to important insights into the accuracy and reliability of RWD, the ability to transform RWD into real-world evidence (RWE) related to the usage and potential benefits or risks of a treatment, and new policies to advance the use of this important data source to enhance cancer research and care for patients.

BACKGROUND

On average, fewer than 5% of patients with cancer receive treatment through a clinical trial. By leveraging RWD, the information gap between data generated from clinical trials and from routine care can be bridged. RWD are captured from sources such as insurance claims, electronic health records, and patient registries. There is growing recognition that these data sources, when analyzed appropriately, can generate RWE in broader patient populations to inform treatment effectiveness, safety, and patient outcomes.

Recent U.S. Food and Drug Administration (FDA) guidance and legislation have highlighted the possibility of using RWD and RWE to support drug development, however, strategies and methodologies for analyzing RWD must be consistent. Data captured in the real world are not always collected at pre-established timepoints or reported uniformly which leads to variation within and across data sources. In addition, outcome measures typically collected as part of a clinical trial are not easily obtained from RWD sources. Identifying and aligning on new endpoints and metrics specific to RWD is necessary.

Friends' portfolio of RWE projects informs and establishes methodology for using RWD to evaluate how treatments work in patients with cancer. Aligning best practices and frameworks for aggregating and analyzing RWD will ensure RWE is high quality and reliable for supporting oncology drug development, regulatory decision-making, and real-world use of products.

APPROACH

Since 2017, *Friends* facilitated collaboration among numerous RWD partners, pharmaceutical companies, government officials, and academic researchers to advance the understanding and applications of RWD and RWE in oncology:

- **RWE Pilot 1.0:** *Friends* developed a framework for operationalizing and validating real-world endpoints in advanced non-small cell lung cancer (aNSCLC). The pilot used a harmonized protocol and set of aligned definitions to identify similar patient populations across data sources and extract real-world endpoints.
- **RWE Pilot 2.0:** RWD partners showed that RWD can generate similar results to clinical trials when measuring treatment effect in patients with aNSCLC across RWD sources. The group developed a list of considerations for the design, conduct, and interpretation of RWD studies from different data sources.
- **rw-Response Pilot:** Project partners established a framework for measuring rw-response to treatment and assessed the consistency of the measure across RWD sources in patients with aNSCLC. Results showed that rw-response was relatively consistent across data sources in an aligned patient population using clinician-stated response.

NEXT STEPS

These multistakeholder partnerships support alignment on best practices, provide a venue to develop and test methodologies for aligning on and analyzing RWD, and help identify opportunities to proactively strategize on future use of RWD/E in oncology.

RWE Portfolio Development and Milestones

CATEGORIES ■ FRIENDS' RWE PORTFOLIO ■ FDA ■ LEGISLATION

2023

- > **Draft Guidance:** Considerations for the Design and Conduct of Externally Controlled Trials for Drug and Biological Products
- > **ASCO Annual Meeting Poster Presentation:** Friends' Real-world Response Pilot
- > **Friends Launches Working Group:** Developing Considerations for Use of rw-Endpoints
- > **Public Meeting:** Supporting the Use of RWD in Oncology Drug Development
- > **Final Guidance:** Submitting Documents Using RWD and RWE to FDA for Drug and Biological Products
- > **FDA Program Announced:** Advancing RWE Program
- > **FDA User Fee Reauthorization Act:** Requires FDA to Establish and Communicate the Advancing RWE Program Pilot

2022

2021

- > **Draft Guidance:** RWD: Assessing EHRs and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products
- > **Draft Guidance:** Data Standards for Drug and Biological Product Submissions Containing RWD
- > **Draft Guidance:** RWD: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products
- > **Draft Guidance:** Considerations for the Use of RWD and RWE To Support Regulatory Decision-Making for Drug and Biological Products
- > **Publication in Clinical Pharmacology and Therapeutics:** The Friends of Cancer Research RWD Collaboration Pilot 2.0
- > **Publication in Clinical Pharmacology and Therapeutics:** rw-Overall Survival Using Oncology EHR Data
- > **Friends Launches Real-world Response Pilot**

2020

- > **Friends' Public Meeting:** An International Framework for RWE
- > **Friends' White Paper:** Considerations for Use of RWE in Oncology: Lessons Learned from Friends Collaborations

2019

- > **Friends Launches Pilot 2.0**
- > **Publication in JCO Clinical Cancer Informatics:** Exploratory Analysis of Real-World Endpoints

2018

- > **Final Guidance:** Use of EHR Data in Clinical Investigation Framework Published: Framework for FDA's RWE Program
- > **Friends' Public Meeting:** The Future Use of RWE
- > **Friends' White Paper:** Establishing a Framework to Evaluate rw-Endpoints

2017

- > **Final Guidance:** Use RWE to Support Regulatory Decision-Making for Medical Devices
- > **FDA Reauthorization Act Signed into Law:** Requires FDA to Draft Guidance on How RWE Can Contribute to the Assessment of Safety and Effectiveness in Regulatory Submissions
- > **Friends Launches Pilot 1.0**

2016

- > **21st Century Cures Act Signed into Law:** Requires FDA to Develop a Framework and Guidance Evaluating RWE for Drug Regulation

