Establishing a Framework for Evaluation of Real-World Response

Goal

Develop and establish methodology for using real-world data (RWD) to measure rw-response to guide regulatory decision-making

Pilot Cohort

Adult patients diagnosed with metastatic NSCLC, treated with a first-line platinum doublet chemotherapy regimen in the metastatic setting

7 Participating EHR-Data Vendors Contributing 200 Patients Each

Pilot Objectives

Assess Availability of Core Data Components for Measuring rw-Response	Evaluate the Consistency of a Composite Measure of rw-Response
Assess availability and frequency of key data components for measuring rw-response, including: • Raw images • Image reports • Clinician assessment	Evaluate the consistency of a measure of rw-response across data sources in the aligned patient population

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Real-World Response Pilot

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RWE Pilot 3.0: Establishing a Framework for Evaluation of Real-World Response

Broad Goal: Develop and establish methodology for using RWD to demonstrate benefit to patients to guide regulatory decision-making

Pilot 1.0
Established aligned definitions and protocols for capturing rw-endpoints in a feasibility study

Pilot 2.0
Assessed performance of rw-endpoints to identify the direction and magnitude of treatment effect
Evaluated the internal consistency of real-world datasets by applying RCT I/E criteria

rw-Response
Establishing a framework for evaluating rw-response and assessing the consistency of the measure to generate RWE

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Measuring Real-World Response

The Definition	The Problem	The Solution
Real-world response (rw-response) is a clinical outcome derived from real-world data (RWD) that provides valuable details about therapeutic efficacy.	**Currently, there is no consensus definition or approach for measuring rw-response.**	**A unique research partnership to develop an aligned framework for measuring rw-response across datasets and a pilot to assess the feasibility and consistency of the measure.**
Data vendors largely derive rw-response from the clinician's assessment of change in tumor burden. This endpoint has promise in the post-market setting to attribute a real-world outcome to a drug intervention in a single arm cohort.	In the real-world setting, data are not consistently captured in a structured or systematic way. Further, there is not a uniform criterion (e.g., RECIST 1.1) in the observational setting for determining tumor response. Therefore, an effort to evaluate, establish, and validate a uniform definition for rw-response is needed.	*Friends* has assembled data partners with EHR-based datasets to develop an aligned framework for measuring rw-response and conduct a pilot to evaluate the availability of data for the measure and the consistency of rw-response through an assessment across groups based upon an aligned patient population.

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rw-Response Approach

7 Participating Data Vendors Contributing 200 Patients Each

Pilot Cohort

Adult patients diagnosed with metastatic NSCLC, treated with a first-line platinum doublet chemotherapy regimen in the metastatic setting.

Pilot Objectives

Assess the Availability of Core Data Components for Measuring rw-Response

Assess the availability and frequency of key data components for measuring rw-response, including:

- Raw images
- Image reports
- Clinician assessment

Evaluate the Consistency of a Composite Measure of rw-Response

Evaluate the consistency of a measure of rw-response across data sources in the aligned patient population

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rw-Response Analysis Strategy Overview

Objective 1 — **Assess Availability and Frequency of Data Components**

Availability of Core Data Components

- Percent of patients with each data component
- Median (IQR) data components per patient
- Percent of clinician assessments with a specific source of assessment
- Percent of imaging with a specific indication and modality

Frequency and Timing of Data Components

- Time between index date and first occurrence of data component
- Time between the 1st and 2nd data components
- Time between each sequential pair of components
- Time between all sequential pairs

Objective 2 — **Measure rw-Response**

Estimation of rw-Response Rate and 6-Month rwR Duration Rate

Within and across datasets, with subgroup analyses planned

Calculation of rw-Duration of Response

Kaplan-Meier analysis, also accounting for interval censoring

Association between rw-Response and Time to Event rw-Endpoints

Association with rwOS, rwTTD, rwTTNT

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rw-Response Timeline

Create Framework	Plan Pilot	Evaluate Objective 1	Assess Objective 2
Q2-Q3 2021	**Q4 2021-Q1 2022**	**Q2-Q3 2022**	**Q3-Q4 2022**
Establish working group to develop discussion draft describing key components of a rw-Response framework.	Plan the pilot, including identifying the patient population and aligning on key data components.	Evaluate the availability of core data components for measuring rw-response across datasets.	Assess the consistency of the rw-response measure across data sources.

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