

# 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**

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

**Pilot Objectives** Assess 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



# **Real-World Response Pilot**

# 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

# Measuring Real-World Response

The Definition	The Problem	The Solution
<p><b>Real-world response (rw-response) is a clinical outcome derived from real-world data (RWD) that provides valuable details about therapeutic efficacy.</b></p> <p>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.</p>	<p><b>Currently, there is no consensus definition or approach for measuring rw-response.</b></p> <p>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.</p>	<p><b>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.</b></p> <p><i>Friends</i> 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.</p>

# 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	<div> <b>Assess the Availability of Core Data Components for Measuring rw-Response</b> <p>Assess the availability and frequency of key data components for measuring rw-response, including:</p> <ul style="list-style-type: none"> <li>• Raw images</li> <li>• Image reports</li> <li>• Clinician assessment</li> </ul> </div>
	<div> <b>Evaluate the Consistency of a Composite Measure of rw-Response</b> <p>Evaluate the consistency of a measure of rw-response across data sources in the aligned patient population</p> </div>

# 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 1<sup>st</sup> and 2<sup>nd</sup> 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

# 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.