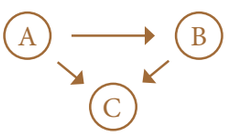
The role of real-world evidence in the COVID-19 era

Real-world evidence (RWE) can be used across development cycle to highlight unmet need, define priority patient segments, competitively position a product before launch, understand real-world treament patterns and outcomes, and to build an evidence base when clinical trial programs are delayed.

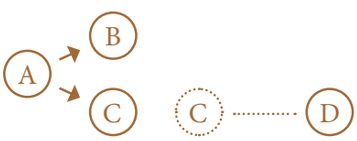
Discovery	Exploratory development			Full development			Life-cycle development	
Pre-Clin	Ph I	Ph IIa	Ph IIb	Ph III/IIIb	File	Launch	Ph IV	
How can RWE inform and strengthen the clinical evidence and help build a new product's value narrative?	• Establish unmet need that the new therapy will address • Describe current treatment landscape and strengths and weaknesses of available therapies • Design Phase 3 clinical studies to capture desired patient population and clinical endpoints			• Provide clinical experience for clinicians not involved in Phase 3 clinical study programs • Create advocacy within medical community and better understand clinical challenges • Extend evidence of effectiveness and safety to real-world setting			• Strengthen and extend value proposition of the new therapy with further evidence generation • Respond to value positioning of competitors and strengthen evidence base for access and pricing negotiations • Confirm safety of the new therapy (i.e. pharmacovigilance)	
What types of RWE can be generated?	• Burden of illness (epidemiology and healthcare resource utilization (HCRU) and costs) • Profile of patint population, including biomarkers • Current treatment patterns and treatment needs • Effectiveness, safety, HCRU and costs, and impact on patent-reported outcomes of current therapies			• Effectiveness/safety of new therapy in real-world setting • Broader understanding impact of new product on HCRU and costs of disease management to feed into economic models • Understanding of compliance, need for does reductions and/or treatment switching or discontinuation			• Descriptive and comparitive effectiveness in real-world setting • Descriptive and compartive safety, including long-term safety data on new therapy • HCRU and costs of new therapy to strengthen value narrative	

Real-world solutions can strengthen clinical evidence.



Indirect treatment comparisons

Compare outcomes of healthcare interventions from separate studies in absence of head-to-head Phase III studies



Single-arm studies

Remove control arm so all study participants receive the novel healthcare intervention and compare with historical controls

Case study: historical control and single-arm study.

Challenge

Slow recruitment into head-to-head Phase III study caused a delay in study readout, so there was the need for comparative data with standard of care in addition to existing Phase 3 clinical efficacy data for the new therapy

Approach

Conducted retrospective analysis of Flatiron electronic health record database to establish historical control to compare with existing Phase 3 clinical data on the new therapy

Impact

Single-arm study findings were shared as part of discussions with the FDA and supported regulatory approval under an agreement to deliver head-to-head Phase III data at a later stage

Using real-world evidence (RWE) intelligently to help adapt to the 'new normal'.