Real world data breakout session

Sept 24, 2019
What are RWD and where do they come from?

Real-world data are the data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources. RWD can come from a number of sources, for example:

- Electronic health records (EHRs)
- Claims and billing activities
- Product and disease registries
- Patient-generated data including in home-use settings
- Data gathered from other sources that can inform on health status, such as mobile devices

https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence
Who is currently using Real-world data and Real-world evidence?

Real-world data (RWD) and Real-world evidence (RWE) are playing an increasing role in health care decisions.

- FDA uses RWD and RWE to monitor postmarket safety and adverse events and to make regulatory decisions.

- The health care community is using these data to support coverage decisions and to develop guidelines and decision support tools for use in clinical practice.

- Medical product developers are using RWD and RWE to support clinical trial designs (e.g., large simple trials, pragmatic clinical trials) and observational studies to generate innovative, new treatment approaches.

The 21st Century Cures Act, places additional focus on the use of these types of data to support regulatory decision making, including approval of new indications for approved drugs.

https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence
Example of moving from conventional clinical study to incorporation of real-world data collection

Phase 3 clinical trials

● Phase 3 RCT (STEADY PD III) of Isradipine in recently diagnosed PD cases N=336 participants

● Phase 3 RCT (SURE PD III) of Inosine N=298 participants

● Both trials consented participants for the ability to be recontacted.

Ancillary study with real-world data collection

● Virtual longitudinal observational study - At Home PD

● Re-consent STEADY PD III and SURE PD III participants for broad data sharing using cloud infrastructure N=420

● Include telemedicine visit, couple with collection of data on mPower platform (Sage Bionetworks) and coordinated with collection of participant entered data through MJFF Fox DEN

Use of Cloud storage and computing

● Establish global unique identifier for each participant that can link individual level data across the clinical trial and virtual observational study

● Incorporate global unique identifier into data streams for mPower platform and Fox Den

● Submit harmonized clinical dataset, with features from mPower platform and data from MJFF Fox DEN into AMP PD cloud platform
**AT HOME PD**
Virtual longitudinal observational study
N = ~420

**SURE PD3**
Phase 3 RCT of inosine
N = 298 participants
PI: Michael Schwarzschild

**STEADY PD III**
Phase 3 RCT of isradipine
N = 336 participants
PI: Tanya Simuni, MD

**Virtual visits**
- Annual visits
- Clinical assessments
- Patient surveys

**Smartphone sessions**
- Quarterly sessions
- Activities of daily life and mood surveys
- Quarterly sessions
- Active daily motor tasks
- Passively collected activity data

Zhan et al, JAMA Neurology, 2018