Advancement of Drug Development Tools

Potential Drug Development Tool or Biomarker

Actionable Drug Development Tool

Supporting Data & Evidence Scattered Across Multiple sources

Integrated Database using CDISC Data Standards

C-Path Data Collaboration Center

Regulatory-endorsed Tool or Biomarker: Context of Use Supporting Evidence Available Data Sources

Biomarker(s)

Drug disease trial model
Databases Supporting Regulatory Endorsement of New Tools

Individual data contributors determine how their data can be accessed

- Alzheimer’s disease placebo arm patient-level data
- Alzheimer’s disease Clinical Trial Simulation Tool
- Polycystic Kidney Disease database: patient-level data
- Multiple Sclerosis database: patient-level data (active and placebo arm)
- MDR Tuberculosis database: active and SOC patient-level data
- Relational Sequencing Tuberculosis Data Platform
- Kidney Safety Biomarkers (members)
- Nonclinical data across Numerous organ safety Biomarkers (members)
- Parkinson’s disease active and placebo patient-level data (members)
- Duchenne Muscular Dystrophy active and placebo patient-level data (members)
Critical Path for Parkinson’s Integrated Database

TOTAL = 5360
(available to CPP members)

PPMI (n=1223) [MJFF, K. Marek]
CamPaIGN (n=142) [Caroline Williams Gray]
Oxford Discovery Cohort (n=877) [Michele Hu]
ICICLE (n=314) [David Burn]
Tracking Parkinson’s (n=1996) [Donald Grosset]
PRECEPT (n=806) [Teva, I. Shoulson, Lundbeck]

From: Heinzel et al., JPD 2017;7(3):423-432
PPMI Data Contributes to the First Biomarker Qualified for Parkinson’s Disease

Qualification Opinion

Qualification Opinion on Dopamine Transporter Imaging as an Enrichment Biomarker for Parkinson’s Disease Clinical Trials in Patients with Early Parkinsonian Symptoms

CPP is developing computerized models to simulate different ‘what if’ scenarios aimed at optimizing trial design for clinical trials targeting early motor PD.

CPP is well underway with FDA and EMA on advancing modeling tools for PD.

UPDRS = Unified Parkinson's Disease Rating Scale; PD = Parkinson disease

D. Conrado
Data Sharing Principles for Parkinson’s in the Future

Parkinson’s Disease Therapeutic Area Supplement to the Study Data Tabulation Model
User Guide

Prepared by
CDISC, National Institute of Neurological Disorders and Strokes (NINDS) and the Coalition Against Major Diseases (CAMD)

Collaboration for Alzheimer’s Prevention: Principles to guide data and sample sharing in preclinical Alzheimer’s disease trials

Stacie Weninger, Maria C. Carrillo, Billy Dunn, Paul S. Aisen, Randall J. Bateman, Joanne D. Kotz, Jessica B. Langbaum, Susan L. Mills, Eric M. Reiman, Reisa Sperling, Anna M. Santacruz, Pierre N. Tariot, Kathleen A. Welsh-Bohmer

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Perspective
Concise informed consent to increase data and biospecimen access may accelerate innovative Alzheimer’s disease treatments

Ann M. Hake, Penny A. Dacks, Stephen P. Arneric, CAMD ICF working group

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