Biomarkers and Translational Tools to Inform Development of New Therapeutics for Neurodegeneration

Chairs

Raj Macha, PhD

Merck & Co., Inc.

Daniela Conrado, PhD

Critical Path Institute

List of failed AD trials keeps growing…

Forbes / Pharma & Healthcare / #Medicine

JAN 24, 2018 @ 05:00 PM 6,003

Latest Alzheimer's Flop Raises Doubts About 'Amyloid Hypothesis'

#MARKET NEWS

DECEMBER 21, 2017 / 9:28 AM / A MONTH AGO

Biogen's Alzheimer's drug fails to meet main goal in mid-stage trial

SCIENCE NOW SCIENCE LA TIMES

One of the most promising drugs for Alzheimer's disease fails in clinical trials

Another Alzheimer's failure: Axovant's drug flops in late-stage trial

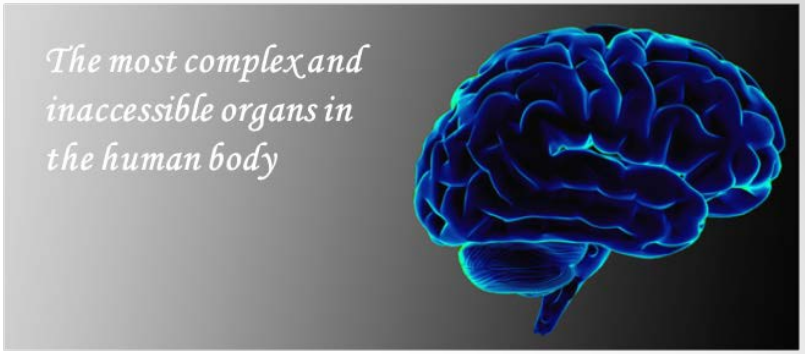
By Damian Garde @damiangarde

September 26, 2017

Biotech

Merck BACE1 drug fails in prodromal Alzheimer's phase 3

Challenges in neurology



- Limited understanding of the underlying biology
- Lack of validated biomarkers
- Lack of predictive disease models
- High cost and duration of clinical trials
- Operational challenges with multi-site trials
- Insufficient sharing of data
- Clarity on regulatory requirements

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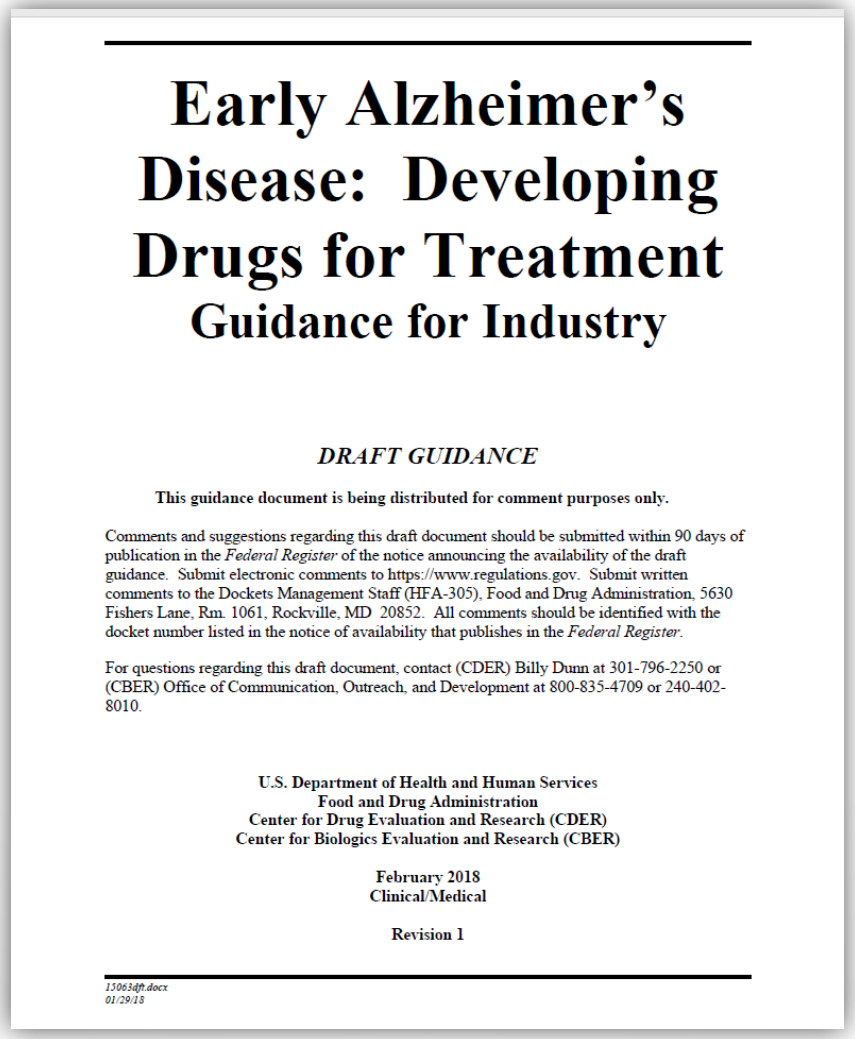
Recent Release of the Early AD FDA Guidance



Early Alzheimer's Disease: Developing Drugs for Treatment

Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Billy Dunn at 301-796-2250 or (CBER) Office of Communication, Outreach, and Development at 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

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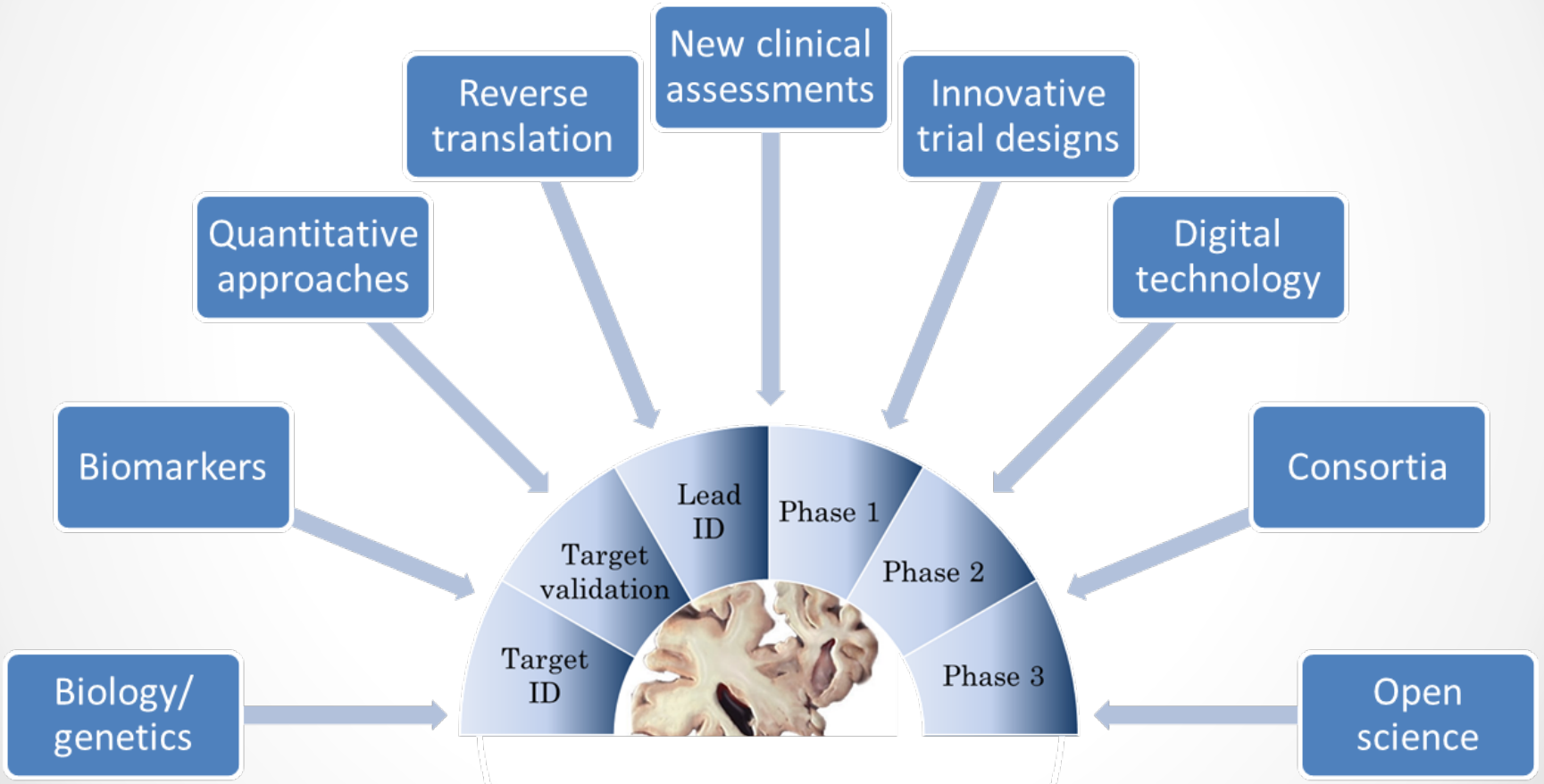
Integrated approach is the way forward



Session topics:

Can application of quantitative clinical pharmacology improve early clinical development success in neurodegenerative diseases?

Gianluca Nucci, PhD

Vice President, Clinical Pharmacology, Early Clinical Development, Pfizer. Inc.

Translation from bench to bedside: PET tracers for use in neuroscience drug development

Eric D Hostetler, PhD

Executive Director, Translational Biomarkers, Merck & Co., Inc.

Model-informed biomarker qualification: Alzheimer and Parkinson disease neuroimaging biomarkers

Daniela J Conrado, PhD

Associate Director, Quantitative Medicine, Critical Path Institute

Item response models for translation in CNS disorders

Mats Karlsson, PhD

Prof of Pharmacometrics, Dept. of Pharm Biosciences, Uppsala University

Session objectives

At the end of this session, the audience will:

- Understand the challenges unique to neurodegenerative disorders including disease heterogeneity, slow disease progression, and large inter-patient variability

- Learn about the integrated quantitative strategies being implemented in the neuroscience area utilizing biomarkers and translational approaches to inform early and late stages of drug development