Probing Novel Regulatory Science Precedents in Alzheimer's, ALS, and Schizophrenia for Implications for all Neuroscience

Chair:

Frank Sasinowski, MS, MPH, JD

Disclosures

Frank Sasinowski

Director, Hyman, Phelps & McNamara, P.C.
FDA, Former Deputy Director of Health Policy, Commissioner's Office
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Vice Chair, Board, Everylife Foundation for Rare Diseases

Speakers

Paolo Bettica, MD, PhD
Wilson Bryan, MD
Teresa Buracchio, MD
Teresa Fecteau, PhD
Emily Freilich, MD
Charles Raver, JD
Barbara Tate, PhD
Ellis Unger, MD
Marc Walton, MD, PhD
Hao Zhu, PhD
Frank Sasinowski, MS, MPH, JD

Regulatory Science: Cutting Edge Approaches

1. Dr. Buracchio – FDA Perspective
2. FDAMA 115 – Single Trial plus Confirmatory Evidence
    a. Mr. Raver – Overview of FDAMA 115 Standard
    b. Dr. Bettica – Givinostat for DMD
    c. Dr. Tate – Omaveloxolone for FA
    d. Dr. Freilich – FDA's View of Utility of FDAMA 115 Standard
3. Rare Disease Novel Approaches
    a. Accelerated Approval
        * Dr. Fecteau – Toferson for SOD-1 ALS
        * Dr. Zhu – FDA's perspective
    b. Gene Therapy
        * Dr. Bryan – Zolgensma for Type 1 SMA
4. Treating an Acute, Intermittent Condition in Psychiatry
    a. Dr. Walton – Esketamine – Depressive Symptoms in those with Acute Suicidal Ideation or Behavior
5. Panel Discussion – Dr. Unger