

Optimizing the Predictive Value of Preclinical Research

Lessons Learned

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What We Need (Stanley Lazic, F. Hoffman-LaRoche):

- Unbiased and reproducible estimates of effect size
- Appropriate and reasonable precision of these estimates

Consistent Theme: Treat preclinical study design the way you would clinical study design

Develop Standard Operating Procedures:

- Pre-specify measurements
- Pre-determine inclusion/exclusion criteria
- Use randomization methods, allocation concealment and blinding procedures
- Validate preclinical model, endpoints and statistical methodology
- Make sure scope of preclinical investigation is adequate
- Include PK/PD data in investigations

- **Alzheimer's Drug Discovery Foundation**

- Solution:** Used expert advisory panel to generate and disseminate standards and invest in resources

- **CAMARADES Collaboration**

- Solution:** Collaborative group to set standards and disseminate those standards

- **ALS Therapy Development Institute**

- Solution:** Building collaborative group to properly validate preclinical animal models



Is it enough? And how do you know if it's working?



Top Down Approaches **(Funders, Journals, Reviewers):**

- Consensus statements
- Funding Requirements
- Publication Requirements
- Review Requirements
- Educational/Training Efforts



Bottom Up Approaches **(Investigators):**

- Education/Training
- Consensus statements
- Transparency
- Cultural Shifts
- Incentive Shifts