**Animal Studies Questionnaire**

**For proposals that include *in vivo* efficacy or proof-of-concept studies in animals, provide responses to all of the questions.** Please see [Snyder, et al](http://www.alzheimersanddementia.com/article/S1552-5260%2816%2932676-0/fulltext) and [Shineman, et al](https://alzres.biomedcentral.com/articles/10.1186/alzrt90) for recommendations on your animal study design.

1. Provide justification for the animal model selected based on the mechanism of action for the therapeutic agent. Has the model been extensively characterized? (Genotype and phenotype should be confirmed throughout the duration of the study.)
2. Describe or reference pharmacokinetics (PK) data showing that your therapeutic agent reaches its intended target at a sufficient concentration to ensure that your hypothesis is being tested. PK should inform dose selection and should be performed with the same route of administration and formulation intended for use in your efficacy study.
3. What are your primary and secondary outcome measures? Disease-relevant outcomes should always be pre-specified and relevant to your compound’s mechanism of action.
4. What is your pharmacodynamic readout of target engagement?
5. How do you expect efficacy readouts in your animal study to translate into human clinical outcomes? Is a translatable biomarker for your target or mechanism of interest available?
6. What are your inclusion and exclusion criteria? (i.e. excluding sick animals, including only animals of a specific phenotype, etc.)
7. Provide a statistical analysis plan. Include a power analysis to justify your sample size. Has your power analysis accounted for previously observed variability in your outcome measures?
8. How will cohorts be randomized and what methods will be used to ensure experimenter blinding?
9. Do you plan to use both males and females? Will the results be analyzed separately?
10. How will dropouts or premature deaths be reported and dealt with in the statistical analysis?
11. If your study is negative, do you still intend to publish the findings?
12. Include a flow chart of your animals through the treatment plan of the study