**The ALS Association**

**The Lawrence and Isabel Barnett Drug Development Program**

**Data Summary for Advanced Compound(s) of Interest**

Please include as much information as possible on small molecule agents or biologics that are to be tested in “proof-of-concept” ALS models. If information is not known or you are in the process of obtaining such data, please indicate that as well.

**Principal investigator:**

**Institution/Company:**

**Project Title:**

**Therapeutic name:**

**Therapeutic category (small molecule, biologic, ASO, peptide, etc.):**

**Patent/License holder:**

**Target class (kinase, GPCR, etc.):**

|  |  |
| --- | --- |
| **Structure:**  Molecular weight:  cLogP:  PSA or TPSA: | |
| **Target-Related *In Vitro* Assays** (including off-target selectivity data and whole cell activities) | |
| Assay Name | Data/Comments |
|  |  |
|  |  |
|  |  |
|  |  |
|  |  |
| ***In Vivo* Disease Models (doses, routes of administration; comment on activity, significance, dose/reponse relationship)** | |
| Model description | Data/Comments |
|  |  |
|  |  |
|  |  |

|  |  |  |  |
| --- | --- | --- | --- |
| ***In Vivo* Pharmacokinetics** | | | |
|  | Species 1 | Species 2 | Species 3 |
| *Dose (mg/kg); route of administration* |  |  |  |
| *IV Vss (L/kg)* |  |  |  |
| *IV Cl (ml/min/kg)* |  |  |  |
| *IV t1/2 (h)* |  |  |  |
| *PO AUC (uM.h) @ \_\_ mg/kg* |  |  |  |
| *%F* |  |  |  |
| *Brain/Plasma Ratio* |  |  |  |
| *Unbound Brain/Plasma Ratio* |  |  |  |

|  |  |  |
| --- | --- | --- |
| **Additional MAP, Toxicology, Pharmaceutics (*in vitro*/*in vivo*)** | | |
| *Plasma protein binding (% Free)* | |  |
| *MDR1 efflux ratio (A-B/B-A ratio)* | |  |
| *In vitro stability (human liver microsomes or hepatocytes; other relevant species)* | |  |
| *CYP IC50 (3A4, 2D6, 2C9, 2C19, 1A2)* | |  |
| *hERG* | |  |
| *Broad in vitro screening hits* | |  |
|  | |  |
| *AMES (Gene tox)* | |  |
| *Toxicology studies: Rat/Mouse* | | NOAEL @ \_\_\_\_\_ mg/kg |
| *Toxicology studies: Dog/Primate* | | NOAEL @ \_\_\_\_\_mg/kg |
| *Aqueous solubility (pH)* | |  |
| *Other* | |  |
|  | |  |
|  | |  |
| ***Clinical experience*** | | |
| Disease Target: | | |
| Phase 1 | Data/Comments | |
| Dosing |  | |
| Safety and tolerability |  | |
| Pharmacodynamic outcome |  | |
|  |  | |
| Phase 2 | Data/Comments | |
| Dosing |  | |
| Safety and tolerability |  | |
| Efficacy |  | |