ABSTRACT

There remains a high unmet need for novel treatments for CNS diseases. Many academic and industry scientists are eager to undertake novel drug discovery approaches for the treatment of neurological disorders but lack some of the drug discovery infrastructure. To boost and de-risk drug discovery and development in the neuroscience field, NIH Blueprint for Neuroscience Research introduced a series of translational programs to promote neuroscience drug discovery and development efforts to mitigate the current pipeline gaps.

In this presentation, we demonstrate how NIH Blueprint Neurotherapeutics Network (BPN), by providing funding, resources (contract access to medicinal chemistry, DMPK, toxicology, drug manufacturing and formulation, as well as contract access to perform the Phase I clinical study) and expertise, have contributed to the successful translation of academic and industry discoveries in basic disease biology into novel drug candidates in clinical testing.

MILESTONE PROGRESSION BY STAGE

- Exploratory (6-12 months)
- Lead Optimization (6-12 months)
- Development (6 months)
- IND enabling (6 months)
- Phase I (SAD study 6 months)
- Phase I

EXAMPLE: BPN Project - Treatment of Epilepsy

- Improved surgical approaches to resect epilepsy
- Designed PK studies in rodent and dogs
- Developed chemical for self-lamination
- Developed drug product formulation and manufacturing for clinical trial

ENTRY CRITERIA

- Novel target for the disease
- Strong biological validation
- Feasible path to the clinic
- Robust in-vitro assay for optimization
- Strong confirmatory assays
- Project must require medicinal chemistry
- Amenable to chemistry
- IP free of obvious roadblocks

BPN PROJECT ORGANIZATION

- Lead/Product Development Team
- Principal Investigator
- Industry-sponsored consultants
- NIH Staff

- Medical Chemistry
- DMPK
- Toxicology
- Drug Manufacturing and Formulation
- Clinical Trials
- Data Management
- Collaborative Drug Discovery (CDD)

- NINDS
- NIMH
- National Institutes of Health

- Preparatory Phase
  - U43 or UH3 (1-2 years)
  - Contract access to conduct SAR or IND-enabling studies

- Development
  - Fully Optimized Compound
  - Strong data linking target to disease
  - Biological or ADME activity appropriate for intended clinical use
  - Efficacy/PD when delivered by clinically intended route
  - Fully profiled, defensible ADME results
  - Feasible path to the clinic
  - IP free of obvious roadblocks

- BPN PROJECTS SUCCESSFUL POST-PROGRAM PROGRESSION

- 10 projects have announced additional industry funding since utilizing the BPN

CONTACT INFORMATION

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