NINDS Funding Opportunities

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NIH Institute Support for Neuromuscular Diseases

MG – myasthenia gravis
PN – acquired and inherited peripheral neuropathies
MD – muscular dystrophies; DBMD, DM, FSHD, CMD, LGMD, OPMD, EDMD etc
IM - inflammatory myopathies, DM, PM, IBM
MH - malignant hyperthermia
CNM - centronuclear myopathies
MChan - muscle channelopathies, nondystrophic myotonias, periodic paralyses

ALS, CMT, MG, PN
SMA
MD
Pompe
MChan, MH CNM, IM

NINDS
NICHID
NIAMS
NHLBI
NINDS Funding Opportunities

- Research Program Awards – R35
- Translational Research Programs
  - Innovation Grants to Nurture Initial Translational Efforts (IGNITE) – R21/R33
  - Cooperative Research to Enable and Advance Translational Enterprises (CREATE) – U01, UH2/UH3
  - NIH Blueprint Neurotherapeutics – UH2/UH3
- Clinical Trial Programs
  - Exploratory Clinical Trials – R01
  - Phase III Clinical Trials – U01
  - NeuroNEXT
- Clinical Trial Readiness for Rare Neurological and Neuromuscular Diseases
NINDS Research Program Awards (R35)

- Rolls all of an investigator’s NINDS research project grant support into one award
- Six page application, no specific aims
- Review based on track record of accomplishments and potential for future impact
- Up to $750,000 dc/year, up to 8 years of support (5 + 3)
- Requires 50% of research effort
- Eligible applicants must have an active NINDS R01 up for renewal in FY16 or FY17 and five consecutive years of NINDS R01 level support
- One receipt date, Oct 6, 2015
- Awards in July, 2016
NINDS Translational Research Funding Opportunities

**Assays & Screening**
- Small molecules or biologics
- R21/R33 linked awards, up to $750K over 3 yrs
- Assay development
- Screening for candidate therapeutics
- Initial testing in animal models

**In Vivo POC**
- Biologics or biotechnology derived products
- Convincing POC required
- U01,UH2/UH3 awards, 4 yrs
- Lead optimization to FIH trial

**Lead Optimization**
- Small molecules
- Validated hits required
- UH2/UH3 awards
- Combination of funding and access to services

**IND Enabling Studies**

**First in Human Trials**
Common Features of NINDS Translational Research Programs

- Emphasis on rigor and reproducibility – guidance to applicants and training of reviewers
- Budgets are based on project needs (for CREATE and BPN) and are not subject to administrative cuts
- Achievement of quantitative milestones is required for award of each budget increment
- Consulting, compound manufacturing, animal studies and other contract resources are available to awardees for certain programs
- Dedicated review panel with translational research expertise from academia and industry
- Funding decisions based on discussion of summary statement, programmatic considerations and availability of funds – no fixed payline
NINDS Support for Clinical Trials

Exploratory Clinical Trials (R01) PAR-13-281
- Examples of responsive studies:
  - Evaluate and optimize dose, formulation, safety, tolerability or PK
  - Short term studies testing target engagement or PD biomarkers
  - Selecting among 2 or more candidate interventions or dosing

Phase III Investigator-Initiated Efficacy Clinical Trials (U01) PAR-13-278
- Milestones for start-up, feasibility and completion phases
- Substantial NINDS Program staff involvement; monitoring enrollment and progress on milestones, oversee AE monitoring

All NINDS Clinical Trial Applications:
- Budget based on need for up to 5 years
- IND needed at time of application
- Dedicated peer review panel, no threshold payline
NeuroNEXT Clinical Trials (U01) PAR-13-343
NeuroNEXT Infrastructure Resource Access (X01) PAR-15-195
NeuroNEXT Small Business Innovation in Clinical Trials Direct to Phase II (U44) –PAR-15-194

- Robust, standardized and accessible infrastructure
- Support for study design and protocol development
- Access to clinical research expertise and patient populations
Clinical Trial Readiness for Rare Neurological and Neuromuscular Diseases

**Clinical Trial Readiness**
Validated biomarkers and clinical outcome measures, knowledge of disease course, effective recruitment strategies

**Clinical Research Infrastructure**
Supported through NeuroNEXT or other networks

**Candidate Therapeutics**
Supported through NINDS and other translational programs

Goals of this new initiative:
- To validate the accuracy, sensitivity and reliability of biomarkers and outcome measures that will enable well-powered trials of shorter duration
- To characterize clinical cohorts and test recruitment strategies that will increase the likelihood of successful trials
Description of the Initiative

Types of studies that would be supported through this initiative:

- Rare neurological or neuromuscular diseases (<200,000 pts in US)
- Needed to facilitate one or more upcoming trials
- Milestones for enrollment targets and biomarker/outcome measure performance
- Aims may include:
  - Biomarker or clinical outcome measure validation/qualification
  - Patient cohort characterization
  - Testing of recruitment strategies through above studies
  - Newly recruited participants or ancillary to an ongoing clinical trial

Types of studies that would be outside the scope of this initiative:

- Natural history studies without direct relevance to upcoming trials
- Biomarker discovery, pathophysiology or GWAS studies
- Support for establishing clinical research infrastructure or patient registries
• General funding opportunities for hypothesis-driven research (R01, R21, etc)
• New approach to provide stable support (R35) for successful labs
• Specialized programs for preclinical translational research
• Trial readiness studies to increase likelihood of successful trials
• Support for clinical trials and infrastructure

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