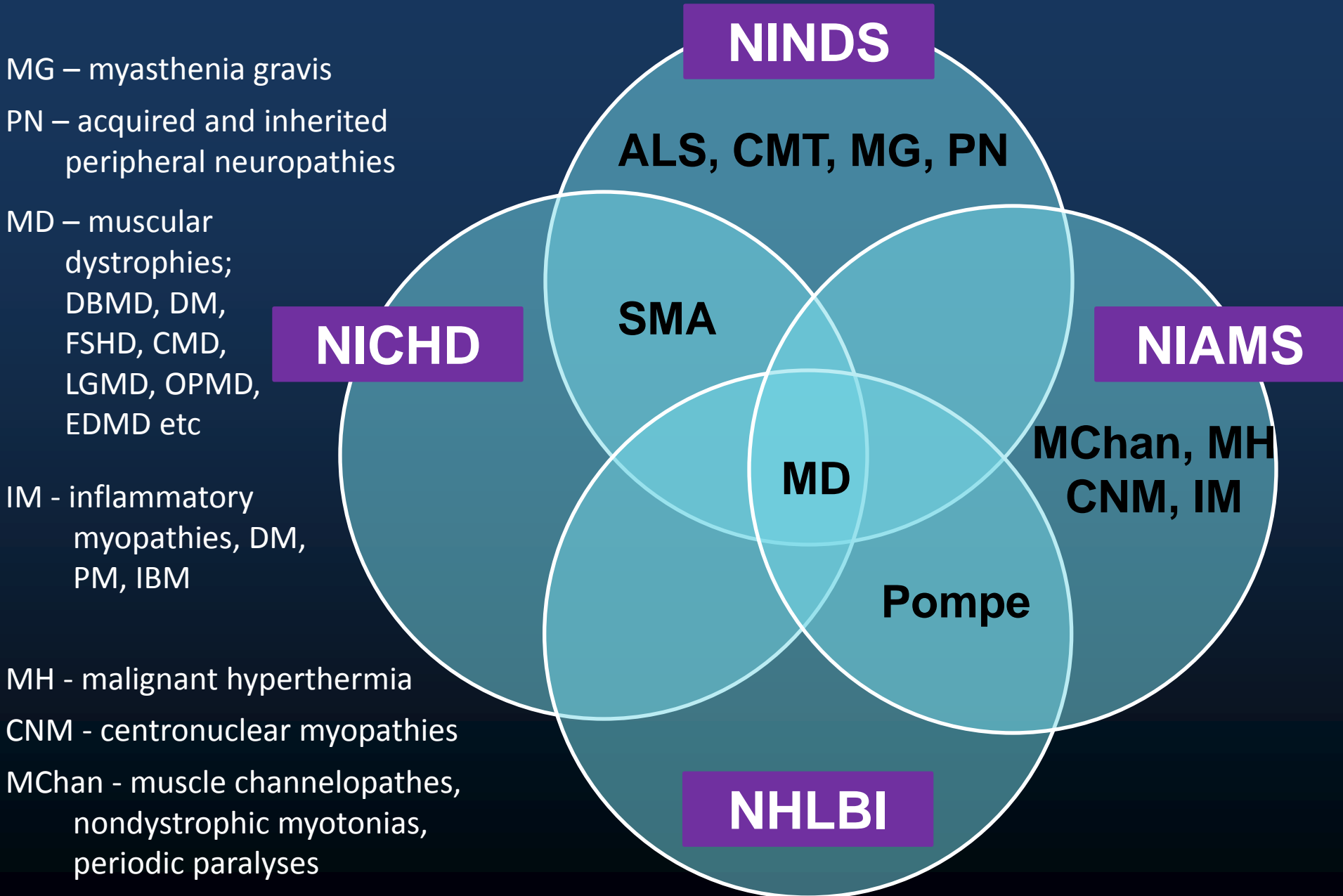




NINDS Funding Opportunities

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NIH Institute Support for Neuromuscular Diseases



NINDS Funding Opportunities

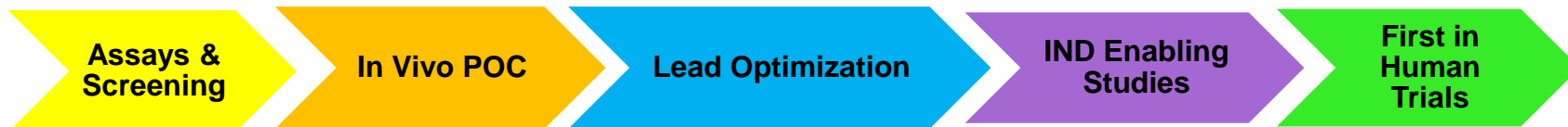
- **Research Program Awards – R35**
- **Translational Research Programs**
 - **Innovation Grants to Nurture Initial Translational Efforts (IGNITE) – R21/R33**
 - **Cooperative Research to Enable and Advance Translational Enterprises (CREATE) – U01, UH2/UH3**
 - **NIH Blueprint Neurotherapeutics – UH2/UH3**
- **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



- 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

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



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



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:

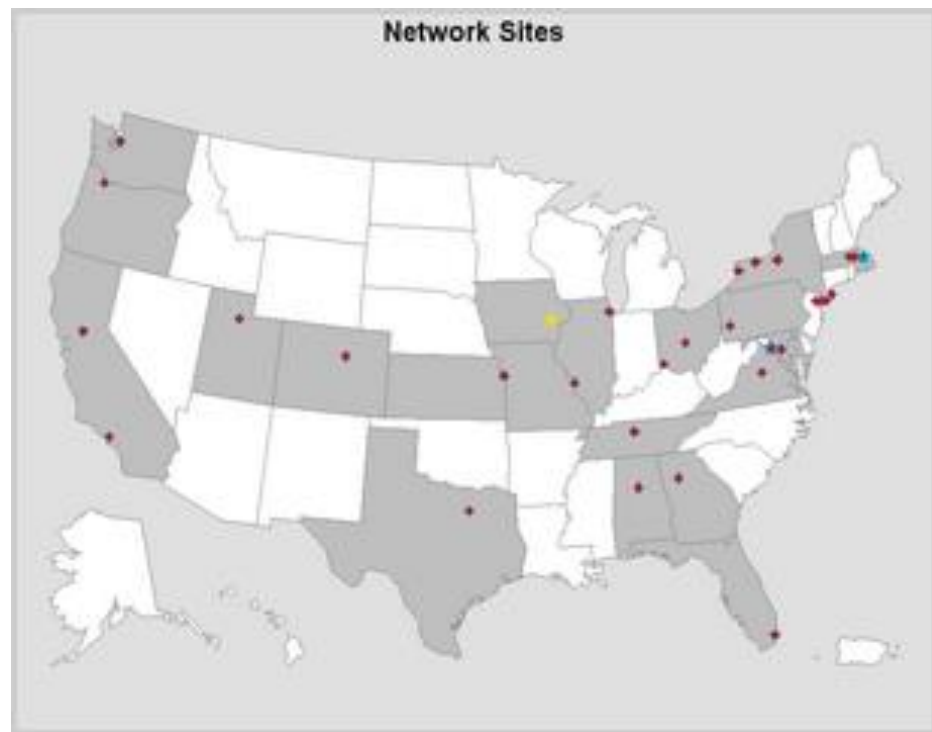
- **Budget based on need for up to 5 years**
- **IND needed at time of application**
- **Dedicated peer review panel, no threshold payline**

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

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

Clinical Research

Infrastructure

Supported through NeuroNEXT or other networks

Candidate

Therapeutics

Supported through NINDS and other translational programs

Clinical Trials

Description of the Initiative

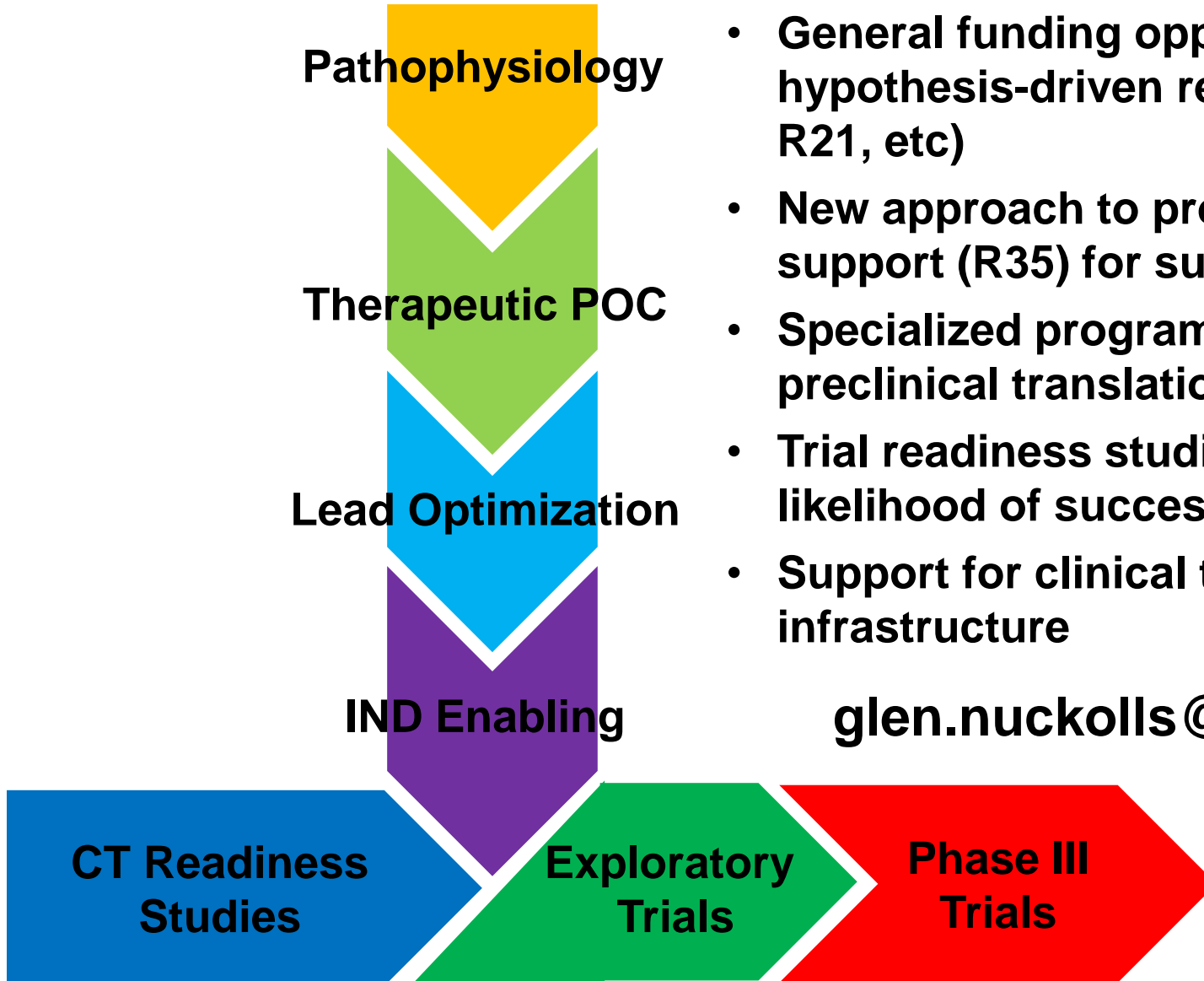
Types of studies that would be supported through this initiative:

- **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**

Summary



- **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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