Attaining Symbiosis in Therapy Development Efforts in NMDs

Symbiosis = close and often long-term interactions between different biological species
(Wikipedia)

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NIH/DHHS
NIH Funding for NMDs

- NIH funding for coded NMDs: $239M
- Dollars/disease determinants: # and quality of applications & NIH appropriation level
- Fundamental need: common ground across NMDs, disciplines, & organizations in push to translate!
New Paradigm for Therapeutic Success in NMDs

• Change **Mindsets**
  – Academic investigators & clinicians
  – Biotech & pharma

• Change **Partnering Paradigms**
  – Evolving the academic team model & academic-corporate partnering
  – International partnerships
  – Experimentation & innovation by partners

• Change **Funding Strategies**
  – No one group can do it all
  – Experimentation & innovation by funders
  – Collaborations of academics, advocacy, corporate, government, and patients/families *essential* for rare disease
Beyond ‘Normal Grants:’ NINDS Translational Research Pgm

Basic

Target ID → Assay → Screening → Proof of Concept → Lead Optimization → Pre-clinical Testing → Clinical Trials

Translational

Clinical

Opportunity to Efficiently and Effectively Exploit All Viable Therapeutic Strategies

Exploratory/Developmental Grants

Cooperative Agreements
Key Tenets of NINDS Translational Program

- Focus on the goal of an IND
- **Annual Milestones**: quantitative go/no-go goals
- Special review
- Extensive NIH staff involvement
- Encourages academic-advocacy-corporate-NIH collaborations
- Partnering: Projects started via advocacy &/or industry now NINDS/advocacy/industry co-funded
NINDS Translational Program
Awards in NMDs

• Active pre-IND awards (U) in NMDs
  – Gene therapy: Mendell & McPhee (both DMD)
  – Small molecule: Higgins (SMA), Karlsson (ALS), Rothstein (ALS), Sweeney (MD), & Guttridge (DMD)
  – Others: Fallon (DMD/CMD), Kelly (ALS), & Lu (DMD)
  – Resource: Kornegay (DMD)

• Neuropathy and MG applications
• See the PARs and talk with us…
Symbiosis on Science
Symbiosis on Funding

No Symbiosis
No One Gets a Drug
Talk with NINDS
Symbiosis: Project Catalyst—NIH

Project Catalyst

DMD Assays & HTS

NINDS Trans Program

Sweeney/PTC U54 Application

Sweeney/PTC U54 Award (NS & AR)

U54 Management

Project Catalyst & PTC Co-funds

PPMD/MDA/CF input
Other Notable NIH Awards in NMDs: K’s

Other Notable NIH Awards in NMDs: NINDS ARRA

• RC1s (“Challenge” Grants)
  – Eva Feldman, Biomarkers in diabetic neuropathy
  – Madhuri Hegde, Gene chip diagnostics for NMDs
  – Richard Liem, Small molecule therapy for CMT2E
  – Grace Pavlath, Stem cell theory of OPMD

• RC2s (“Grant Opportunity” Grants)
  – Kevin Campbell, High throughput genetic (diagnostic) & small molecule (drug) screens for CMD
  – Arthur Burgess/Don Cleveland, Delivery of gene therapy in motoneuron disease (synergism for ALS & SMA)
Other Notable NIH Awards in NMD: NINDS Misc.

- **RDCRN**
  - Berch Griggs, Nervous system channelopathies consortium
  - Mike Shy, Inherited neuropathies consortium

- **Clinical Trials**
  - Berch Griggs/Katie Bushby/Mike McDermott, FOR-DMD
Working Across NMDs

Gain (and Share) Insights into the Therapy Development Process for NMDs

Efficiently and Effectively Develop Specific Drugs and Biologics

Develop Shared Trial Infrastructure and Expertise for NMDs
TREAT-NMD/NIH International Conference
Bringing Down the Barriers: Translational Medicine in NMD

• November 17-19, 2009
• Brussels, BE
• http://www.treat-nmdconference2009.eu/