

Attaining Symbiosis in Therapy Development Efforts in NMDs

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

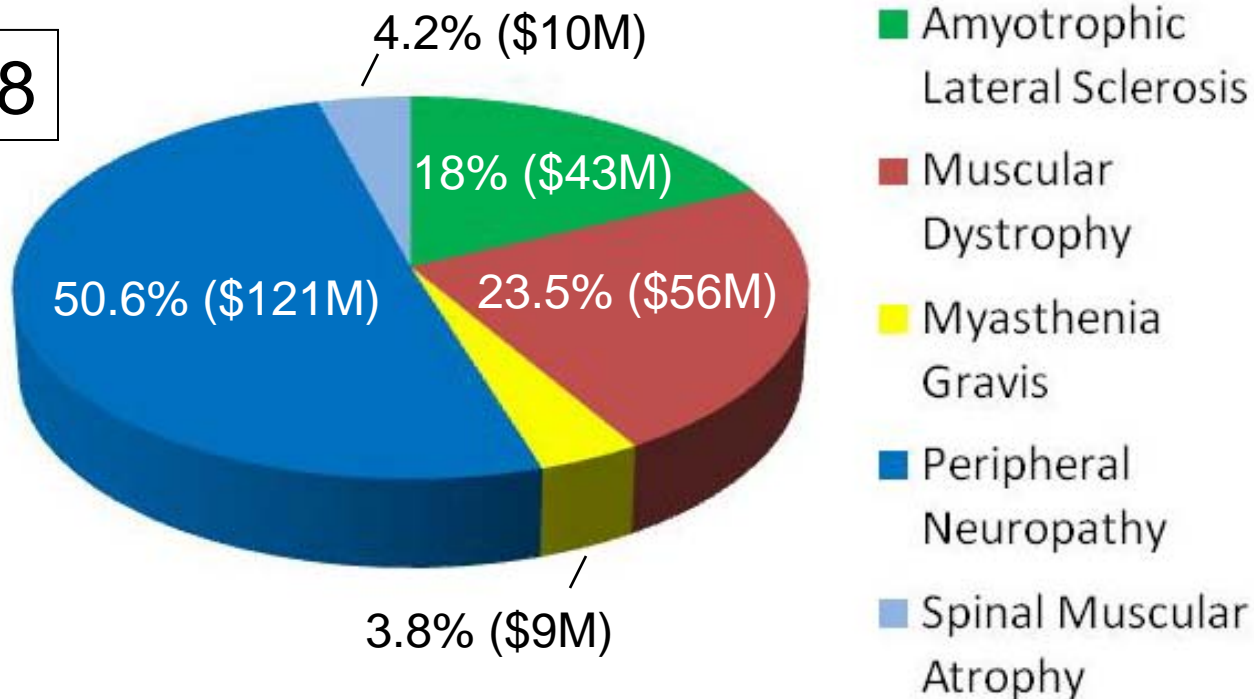
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NIH/DHHS



NIH Funding for NMDs

FY2008



- 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



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...

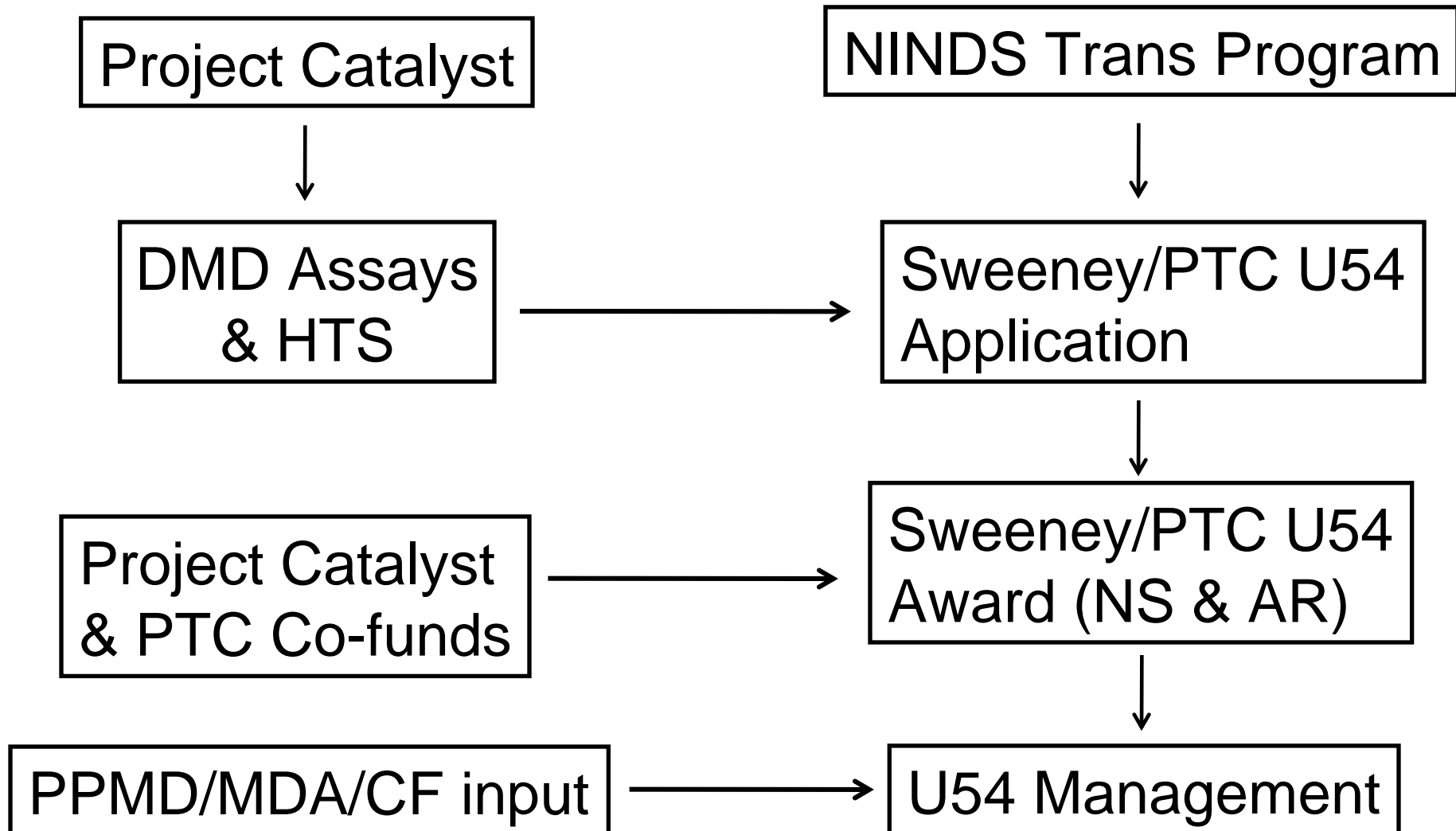


Symbiosis on Science Symbiosis on Funding

No Symbiosis
No One Gets a Drug
Talk with NINDS



Symbiosis: Project Catalyst—NIH



Other Notable NIH Awards in NMDs: K's

- **K awards (new and continuing):** C. Abrahms, R. Baloh, M. Cartwright, R. Cohn, N. Frank, K. Gruis, P. Kang, C. Klein, J. Li, L. Lu, M. Meriggioli, C. Pierson, D. Selcen, T. Wheeler , & J. Wang (all NINDS); P. Agrawal, J. Dowling, S. Leving, A. Mammen, M. McCue, R. Orellana, B. Tseng, C. Buettner, M. Kitazawa, N. Weisleder, & C. Heatwole (all NIAMS)

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/>

