ONE-ON-ONE WITH THE NIH

BIO & HEALTH

CEO & INVESTOR FORUM

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North Carolina Biotechnology Center

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Sands Capital Ventures
Neuroscience Partnering at NIH

Sue Ano, Ph.D.

Technology Development Coordinator
National Institute of Neurological Disorders and Stroke (NINDS)
About Sue

Licensing and Patenting Manager
Infectious Diseases
NIH OTT

2002

Branch Chief
Infectious Diseases and Medical Engineering
NIH OTT

2008

Director
Technology Transfer
NINDS

2015
NINDS Technology Transfer Mission

Support Our Investigators

Build Partnerships

Facilitate Scientific Innovations

**NINDS mission:** to seek fundamental knowledge about the brain and nervous system and to use that knowledge to reduce the burden of neurological disease
NINDS Intramural Research Program (IRP)

- $169M Fiscal Year 2017 (~10% of NINDS overall budget)
- Basic and translational neuroscience, neurology and neurosurgery

Neuroimmunology

Neurogenetics

Functional and Molecular Imaging

Neuromuscular Diseases

Clinical Neuroscience
  e.g., Parkinson’s, epilepsy, multiple sclerosis, dystonia, neuropathies, and gene therapy

Cell/Membrane Biochemistry/Biophysics
Porter Neuroscience Research Center

- 85 research groups from 10+ Institutes/Centers at NIH
- World-class researchers
- Imaging and microscopy cores
- Transgenic animal cores
- Mark O. Hatfield Clinical Center (PBS special “First in Human”)
- Induced pluripotent stem cells (iPSC)
Example Successful Partnerships

• CRADA with GeNeuro SA
• Test and evaluate several monoclonal antibodies provided by Geneuro SA
  • Anti-HERV-K envelope protein (Env)
  • Evaluated for their properties to neutralize Amyotrophic Lateral Sclerosis (ALS)-related biological and pathological effects induced by HERV-K envelope expression in various model systems
Example Successful Partnerships

- Multi-IC CRADA with General Electric (GE) Healthcare
- >10 years and still going
- Improve quality and breadth of GE proprietary MRI technology
  - Hardware
  - Acquisition pulse sequences
  - Software for MR signal processing
Example Successful Partnerships

• Two clinical CRADAs with Audentes Therapeutics
  • one site in a multi-site pre-Phase 1 prospective, non-interventional clinical assessment study in X-linked Myotubular Myopathy (XLMTM) subjects aged 3 years and younger (INCEPTUS)
  • one site in a multi-site clinical trial, entitled “A Phase 1/2, Randomized, Open-Label, Ascending-Dose, Delayed-Treatment Concurrent Control Clinical Study to Evaluate the Safety and Preliminary Efficacy of AT132, an AAV8-Delivered Gene Therapy in X-Linked Myotubular Myopathy (XLMTM) Patients (ASPIRO)”

• Gene therapy following our role in GAN trial
  • www.cbsnews.com/news/a-mothers-quest-to-find-cure-for-rare-genetic-defect
Sample Partnering Opportunities

- **Licensing:** >100 technologies (NINDS)
  - Novel Dopamine D2 Receptor Antagonists
    - Small molecule
    - D2 specificity minimizes off-target side effects
    - Schizophrenia and psychotic syndromes, diabetes, cardiac arrhythmias
      Issued US patent (9,550,742); pending EP application
  - Wirelessly Powered MRI Signal Amplification System
    - Implantable or ingestible
    - Issued US Patents (9,864,026; 10,203,382); pending EP application
  - Research reagents
    - Cell lines
    - Antibodies
  - Visit www.ott.nih.gov/opportunities for NIH licensing opportunities

- **Contact us to discuss collaborations and other partnering opportunities**
  - Provide 1 page summary of opportunity
Neuroscience@NIH

Partnering Opportunities:
Collaborations
Clinical Trials
Research Materials
Patents
Licenses

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