



National Institute of
Neurological Disorders
and Stroke

NINDS

**/Division of Translational
Research-Funded Drug Discovery
and Development Programs**

**Emerging Neurotherapeutics
Pipeline
ASENT 2021**

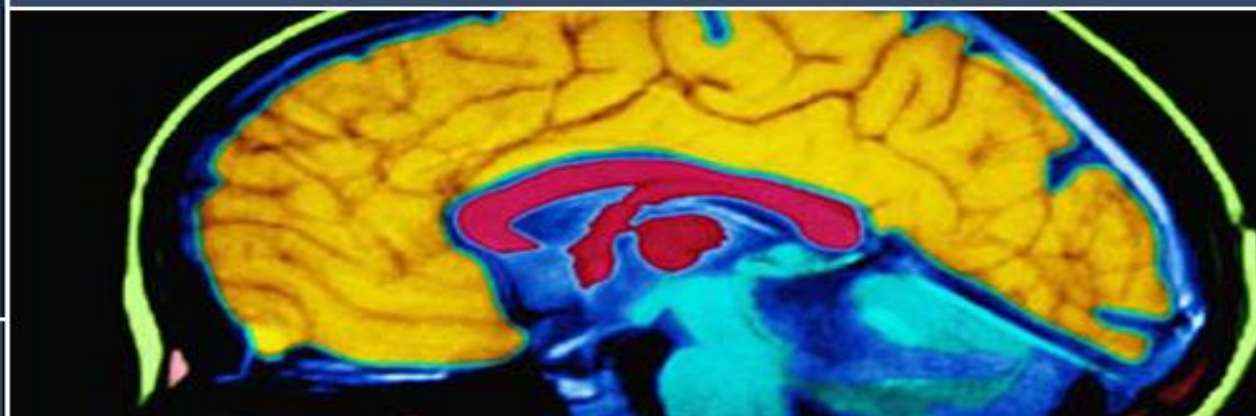
February 25th, 2021

11:45 PM – 1:30 PM, EST



**Mohamed Hachicha, Ph.D., Charles Cywin, Ph.D.
and Amir Tamiz, Ph.D.**

**Division of Translational Research
NINDS/NIH**



NINDS Division of Translational Research-Funded Drug Discovery and Development Programs

Initial Translational Efforts



- Assay Development and

Neurotherapeutic Agent Identification (**PAR-18-762**)

- Neurotherapeutic Agent Characterization and In vivo Efficacy Studies (**PAR-18-761**)
- Development and Validation of Model Systems and/or Pharmacodynamic Markers to Facilitate Neurotherapeutic Discovery (**PAR-18-763**)
- R61/R33, up to 3 years, up to \$750K for the entire project

Program Director: Rebecca Roof, Ph.D.
Email: rebecca.roof@nih.gov

Small Molecule Therapeutics



- Cooperative agreement and SBIR Fast-

Track award programs to support small molecule drug discovery and development

- Customized infrastructure, expertise, contract access, and grant funding (Combined up to \$10M/project)
- Grant and contract support: medicinal chemistry, compound database, PK, toxicology, drug manufacturing and formulation, phase I clinical trials
- U01 up to 5 years, **PAR-20-122**
- U44(SBIR) up to 5 years, **PAR-20-111**

Program Director: Charles Cywin, Ph.D.
Email: charles.cywin@nih.gov

Biotechnology Products and Biologics



- Cooperative agreement including SBIR

Fast-Track awards to optimize and advance therapeutic biologics into clinical development

Biologics Optimization: Characterize and optimize leads

- U01 up to 5 years, budget typically <\$2.5M in direct cost/5yr, **PAR-17-456**
- U44(SBIR) up to 5 years, budget typically <\$4.7M in total cost/5yr, **PAR-17-457**

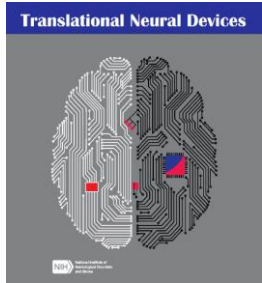
Biologics Development: IND enabling studies and optional FIH clinical trial

- U01 up to 5 years, budget typically <\$6.5M in direct cost/5yr, **PAR-18-542**
- U44(SBIR) up to 5 years, budget typically <\$6.5M in total cost/5yr, **PAR-18-543**

Program Director: Ann-Marie Broome, Ph.D.
Email: ann-marie.broome@nih.gov

NINDS Division of Translational Research-Funded Drug Discovery and Development Programs

Therapeutic and Diagnostic Devices



- Cooperative agreements including SBIR Fast-Track awards to support development, validation, verification, and early clinical studies of therapeutic and diagnostic devices
- Scope of this program is for projects that are either:
 - a. very close to the 'final system' to be marketed or studied in a larger clinical trial following the completion of this project, or
 - b. require early feasibility clinical data to inform the final device design or manufacturing processes.
- **RFA-NS-18-011** (UG3/UH3) up to 5 years
- **RFA-NS-18-012** (U44 SBIR Fast-Track) up to 5 years

Program Director: Nick Langhals, Ph.D.
Email: nick.langhals@nih.gov

Small Business Programs (SBIR/STTR)



SBIR · STTR
America's Seed Fund

- Specific set-aside for small business (3.65% of the extramural budget)
- Includes therapeutics (small molecules, biologics, and devices), diagnostics, and research tools
 - Awardees have access to assistance programs and investor opportunities
 - **PA-18-574 (SBIR), PA-18-575 (STTR)**, and other specific announcements
 - NOT-NS-19-014: HEAL Initiative: Notice of Interest in SBIR/STTR Applications Directed at Enhanced Pain Management

Program Director: Emily Caporello
Email: emily.caporello@nih.gov

Biomarker Program: Neurological and Neuromuscular Disorders



- Discovery of Biomarkers and Biomarker Signatures for Neurological and Neuromuscular Disorders

PAR-19-315

- Analytical Validation of a Candidate Biomarker for or Neurological or Neuromuscular Disorders
- **PAR-21-056, PAR-21-057**
- Clinical Validation of a Candidate Biomarker for or Neurological or Neuromuscular Disorders
- **PAR-21-058, PAR-21-059**

Program Director: Mary Ann Pelleymounter, PhD
Email: mary.pelleymounter@nih.gov
Program Director: Carol Taylor-Burds, PhD
Email: carol.taylor-burds@nih.gov

NINDS Division of Translational Research-Funded Drug Discovery and Development Programs

Epilepsy Therapy Screening Program (ETSP)



■ Screening program to facilitate the discovery of new therapeutic to address unmet medical needs in epilepsy

- Built on a strong foundation of rodent anti-seizure assays performed by world-class epilepsy preclinical researchers, at no cost to participant
- Established in 1975 and tested over 32,000 compounds; contributed to 9 marketed antiseizure drugs
- ETSP focuses on pharmaco-resistance and expansion into areas of disease prevention & modification
- Participant IP protected; confidentiality maintained

Program Director: Brian Klein, Ph.D.
Email: brian.klein@nih.gov

Preclinical Screening Platform for Pain (PSPP)



■ One-stop preclinical testing platform to accelerate discovery of non-addictive, effective therapies

- Incentivize both academia & industry
- Develop or refine animal models of pain conditions
- Generate high quality data to support partnerships, translational programs
- Provide access to research community

Program Director: Smriti Iyengar, Ph.D.
Email: smriti.iyengar@nih.gov

Therapeutics for Treating Chemical Injuries



■ Grant to understand mechanisms of toxicity caused by chemical threat agents and apply this knowledge to develop promising therapeutics

- Grants for mechanistic research, creation of animal models, study of pathophysiological mechanisms of toxicity, and in vivo Proof of Concept studies for therapeutics development
- Collaboration with BARDA and other federal agencies
- **PAR-18-657, PAR-19-039, PAR-19-040, PAR-18-721**

Program Director: David Jett, Ph.D.
Email: jett@ninds.nih.gov