U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES
National Institutes of Health
National Institute of Neurological Disorders and Stroke

NeuroNEXT

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Webinar for industry

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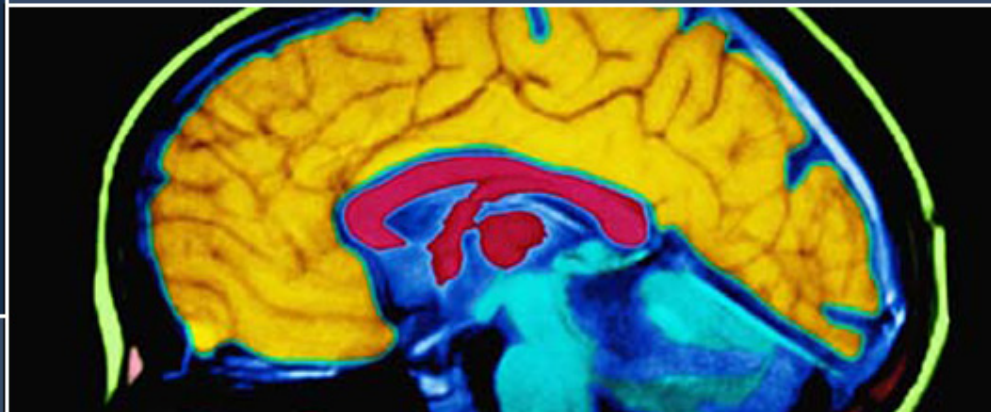


- NINDS has a diverse disease portfolio
- NINDS has extensive clinical trials experience
- NINDS has preexisting relationships with academic investigators and advocacy groups
- Utilizing these strengths NINDS has created an infrastructure, NeuroNEXT, to efficiently conduct Phase II trials targeted towards potential marketing applications

NeuroNEXT goals

- Test highly promising therapies in phase II clinical trials potentially increasing the impact of NINDS funded clinical research.
- Accelerate drug development through an established clinical trials infrastructure
- Decrease the time/cost between trial design and trial completion
- Coordinate public/private sector efforts by leveraging NINDS' existing relationships with academic investigators and patient advocacy groups

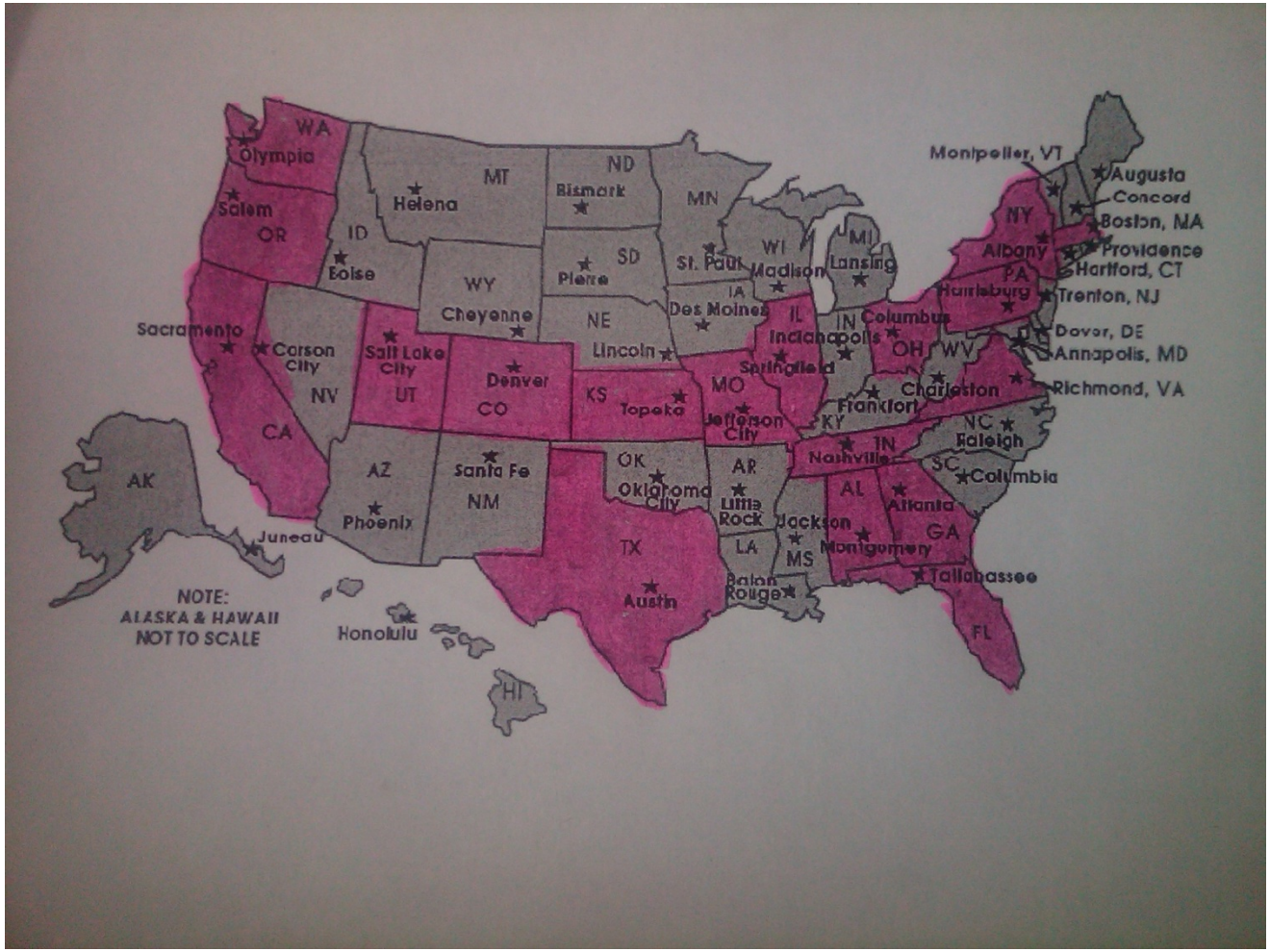
NeuroNEXT Sites

- Clinical Coordinating Center
  - Massachusetts General Hospital
- Data Coordinating Center
  - University of Iowa
- 2 Children's Hospitals
  - Children's Hospital of Boston
  - Children's National Medical Center (DC)

Clinical sites (Adult/Pediatrics)

- Albert Einstein College of Medicine-Yeshiva
- Columbia-Cornell
- Emory
- Massachusetts General Hospital
- Northwestern University
- Ohio State University
- Oregon Health and Science University
- Swedish Health Services (Seattle)
- SUNY (Buffalo, Downstate, Upstate, and Stony Brook)
- University of Alabama, Birmingham
- University of California, Davis
- UCLA
- University of Cincinnati
- University of Colorado, Denver
- University of Kansas
- University of Miami
- University of Pittsburgh
- University of Rochester
- University of Utah
- University of Virginia
- University of Texas, Dallas
- Vanderbilt
- Washington University in St. Louis

Distribution of NeuroNEXT clinical sites



Ways for industry to utilize NeuroNEXT

- Three possible mechanisms-all are accepting applications now
  - Traditional U01-standard timelines
  - Small business grant-standard timelines
  - Resource Access Award (X01)
    - Allows industry access to NeuroNEXT infrastructure
    - Pre-existing master trial agreements for all 25 sites
    - Central IRB review administered through NeuroNEXT CCC
    - Expedited review-submission dates every two months
    - NINDS provides infrastructure/industry partner provides funding

NeuroNEXT applications must include:

- Compelling scientific evidence for evaluating the proposed investigational agent in the disease under study
- Proposed methodology for testing whether the investigational agent has the expected biological effect in study patients
- Clinical Protocol including a summary of potential human risks and the measures being taken to monitor for those risks during the proposed trial.
- Documentation of an active IND/IDE for the investigational agent

NeuroNEXT applications do not need to include:

- Investigator Brochures
- CMC information
- Safety toxicology information
- Clinical pharmacology information other than justification of the proposed dosing regimen

NeuroNEXT-decisions:

- “no-go” decisions for use of the network will be relayed to the applicant as soon as they are made.
- “go” decisions, which entail layers of review, may be given 4 months after formal receipt of the application.

NeuroNext-Special Emphasis Panel

- Core members with expertise in the following:
  - Bioethics
  - Biostatistics
  - Clinical trial methodology
  - Drug development (though not current industry employees)
  - Neuroscience
  - Outcome measures
  - Pharmacology
  - Regulatory sciences
- Patient representative
- Ad hoc expertise depending on requirements of given application

NeuroNEXT -confidentiality:

- All review personnel and government employees with access to application information will be under strict confidentiality requirements.
- Confidential Disclosure Agreements will be used for outside experts brought in for ad hoc review due to specialized expertise.
- We will not choose current pharmaceutical industry employees as reviewers .
- The names of review panelists will be made public.

IP OPTIONS FOR RESOURCE ACCESS AWARD (X01)

IP options under a Cooperative Research and Development Agreement (CRADA) between company and NINDS

Options to company for inventions claiming use and/or composition of agent(s):

- (1) a sublicenseable, royalty-free, non-exclusive license (NERF) for development purposes; and
- (2) time-limited first option to negotiate in good faith a royalty-bearing, exclusive license for commercial purposes; and
- (3) a NERF for research purposes only

IP OPTIONS under a CRADA between company and NINDS

- **Options to company for inventions using non-publicly available clinical data or specimens treated with agent:**
- (1) NERF for research purposes; and
- (2) NERF for disclosing invention: (i) to regulatory authorities; and (ii) on product inserts or promotional material after marketing approval

IP OPTIONS for RESOURCE ACCESS AWARD (X01) under a CRADA between company and NINDS

- **Options to company for unauthorized inventions (discovered under research conducted outside the scope of the approved project):**
- NERF or co-exclusive license (note that clinical site retains NERF for research purposes)

IP OPTIONS for RESOURCE ACCESS AWARD (X01)

- NeuroNEXT Clinical Sites may *choose* to participate in a clinical study supported by a CRADA
- The IP terms (described on previous slides) are included in grant terms and conditions for each Clinical Site and may be viewed in full detail on the NeuroNEXT website
- Terms are non-negotiable for both company and clinical sites

NeuroNEXT-Contacts

- Elizabeth McNeil (general information on NeuroNEXT)
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One stop shopping:

- www.ninds.nih.gov/NeuroNEXT