
Newborn Screening Translational Research Network Coordinating Center

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Statement of Work/Objectives

- 1) Establish an organized network of State newborn screening programs and clinical centers
 - Collect information from state labs and registries that will provide information as well as archival blood spots
 - 2) Develop, implement and refine a national research informatics system for investigators and policy makers that dovetails with the established national clinics network
 - Informatics system to link researchers with potential subjects for trials
 - Liaison between researchers and registries, NBS programs and clinical centers
 - Actively link grantees with technology validation sites
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- 3) Establish and administer an efficient and reliable repository of residual dried blood spots comprised of those stored by State NBS programs and other resources.
 - 4) Provide expertise and support to researchers related to regulatory requirements associated with informed consent, IRBs and state and local research policy associated with NBS.
 - 5) Facilitate research on the development of new methods and technologies by maintaining close contact with the scientific and biomedical research communities.
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Statement of Work/Objectives

- 6) Facilitate research on screened and treated patients to define effectiveness of treatments and long-term outcomes through
 - Monitoring new approaches to treatment for screened disorders
 - Encourage and support outcome studies related to newborn screening
 - 7) Provide statistical leadership and clinical trial design expertise for the individualized needs of researchers through the NBSTRN Coordinating Center
 - 8) Facilitate the timely dissemination of research findings
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Statement of Work/Objectives

- 9) Establish a steering committee comprised of knowledgeable healthcare professionals, public health professionals, ethicists and scientists to make recommendations to NIH program regarding research proposals to have access to NBSTRNCC
 - Review consistent with NIH standards
 - 10) NIH-supported researchers, in conjunction with their Institutes program officer, will nominate research projects for consideration by the network in order to gain access to the NBSTRNCC
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Budget

■ FY08	\$ 998,927	9/08-9/09
■ FY09	\$1,874,630	9/09-9/10
■ FY10	\$2,318,711	9/10-9/11
■ FY11	\$3,569,588	9/11-9/12
■ FY12	<u>\$4,737,915</u>	9/12-9/13
■ Total	\$ 13,499,771	

Newborn screening-update

- Contracts
 - Newborn Screening Translational Research Network (NBSTRN)
 - New Technologies in Newborn Screening
 - Lysosomal Storage Disorders; Assay validation

 - PAR: “Innovative Therapies and Clinical Studies for Screenable Disorders” (R01, R03, R21)
 - Technologies related to therapies for currently screened disorders
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Examples of Current Newborn Screening Projects

- R01 – Spinal Muscular Atrophy – Newborn screening, novel therapies
 - R42 – Turner syndrome and other sex chromosome abnormalities – Screening techniques
 - R44 – Newborn screening blood spot quality control
 - P01 – Newborn screening Fragile X – Family impact.
 - R03 – SCID – Newborn screening
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