


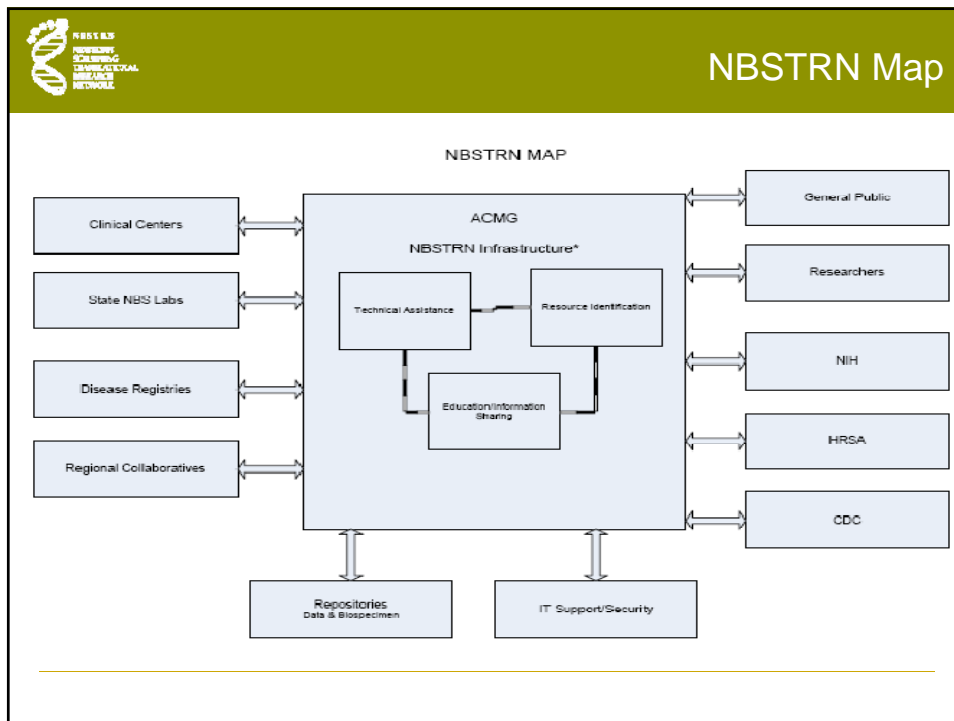
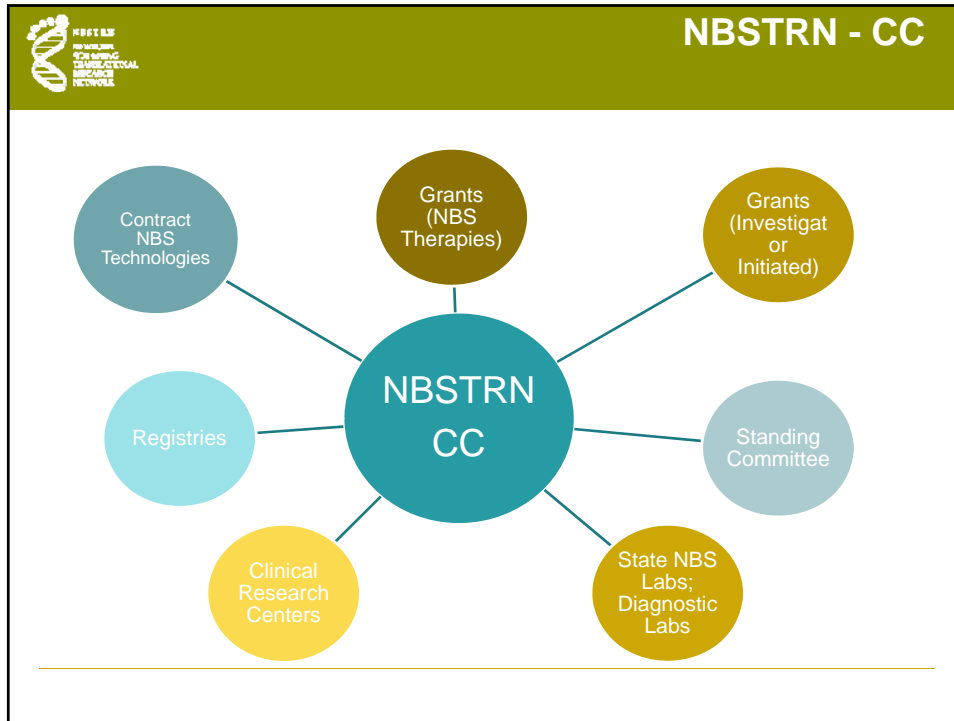
Newborn Screening Translational Research Network (NBSTRN) Overview and Update

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NBSTRN Key Features

- ◆ **NICHD initiative**
- ◆ **Development of national resources to support research and development related to newborn screening**
- ◆ **Long-term follow-up of NBS**
- ◆ **Developing evidence base for NBS candidate condition**
- ◆ **Newborn screening laboratory network**
- ◆ **Clinical centers network**
- ◆ **Infrastructure development (informatics based)**
- ◆ **ACMG serves as the coordinating center (NBSTRN-CC)**
- ◆ **NBSTRN-CC will facilitate research to develop new screening methods and support the conduct of clinical trials for new therapeutic interventions.**






NBSTRN-CC Objectives

- ◆ **Establish an organized network of state newborn screening programs and clinical centers.**
 - ◆ **Develop, implement and refine a national informatics system for investigators and policy makers**
 - ◆ **Establish and administer an efficient and reliable repository of residual dried blood spots**
 - ◆ **Provide expertise and support to researchers related to regulatory requirements associated with informed consent, IRBs, state and local research policy**
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NBSTRN-CC Objectives

- ◆ **Facilitate research on the development of new methods and technologies**
 - ◆ **Facilitate research on screened and treated patients to define effectiveness of treatments and long-term outcomes**
 - ◆ **Provide statistical leadership and clinical trial design expertise for the individualized needs of researchers**
 - ◆ **Facilitate the timely dissemination of research findings**
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Resource Identification

- ◆ **Clinical Centers Networks**
 - Diagnosis and long-term follow-up data
 - Biospecimen repositories
 - Funding opportunities
 - Disease registries
- ◆ **State Newborn Screening Laboratory/Program Networks**
- ◆ **Informatics and Information Technology**
 - IT infrastructure
 - Converting NCIs cancer biomedical informatics grid (caBIG) as appropriate or developing compatible tools
 - Developing tools to link specimen repositories with registries and long-term follow-up clinical information



Education and Information Sharing

- ◆ **Public relations**
- ◆ **Publications and meetings**
- ◆ **Future trends and guidance**



Technical Assistance

- ◆ **Disease specific data sets (protocols for diagnosis and follow-up) development and review**
 - Associated disease-specific standardized languages for laboratory and clinical parameters
 - ◆ **Laboratory standards**
 - ◆ **State newborn screening program policies, procedures and capacities**
 - ◆ **Bioethics review**
 - ◆ **Study design and statistical consulting**
 - ◆ **State and local IRB policies, procedures and alignment**
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Related Policy Work

- ◆ **SOP for NBS pilots**
 - ◆ **Access to Medical Foods and Formulas**
 - Directory of state rules and regulations related to coverage for medical foods and formulas has been updated.
 - Report forthcoming
 - Considering effort to have medical foods and formulas used in NBS reconsidered for regulatory oversight and coverage
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LTFU of NBS Patients

- ◆ **Focused on condition in newborn screening**
- ◆ **Working with states to determine type and level of detail of information needed by NBS Programs to provide evaluation tool**
- ◆ **Expanding to all regions of US**

