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NBSTRN
NEWBORN SCREENING TRANSLATIONAL RESEARCH NETWORK

Translational Research in Newborn Screening: Development of a Virtual Repository of Residual Dried Blood Spots

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Newborn Screening Translational Research Network (NBSTRN)

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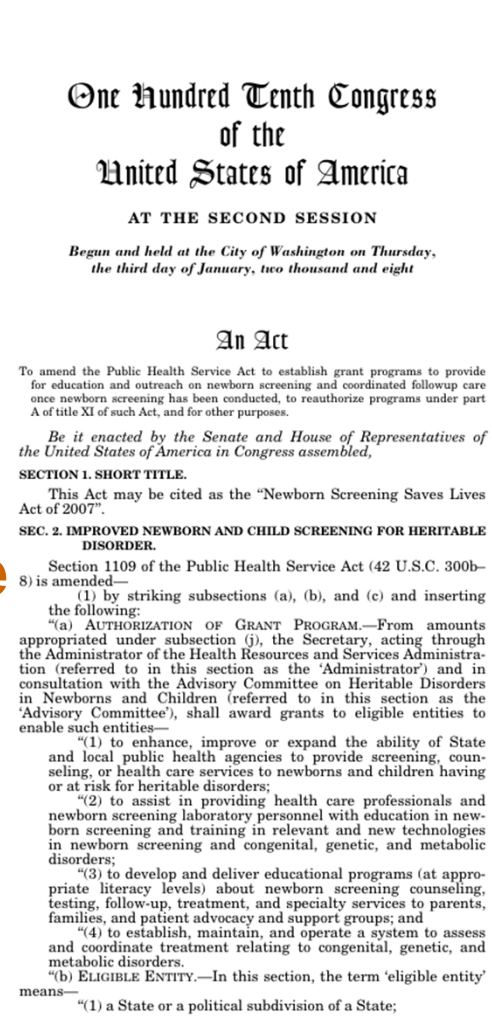
Outline

- The NBSTRN
- The VRDBS
- Partnering with Stakeholders
- Protecting Privacy
- Information Security
- Functional Assessment

** No financial disclosures*

The Newborn Screening Translational Research Network (NBSTRN)

- Newborn Screening Saves Lives Act of 2007
- Hunter Kelly Newborn Screening Research Program
- 5-year contract from NICHD to ACMG
- Develop a research infrastructure to support investigators with projects related to newborn screening



S. 1858

One Hundred Tenth Congress
of the
United States of America

AT THE SECOND SESSION

Begun and held at the City of Washington on Thursday, the third day of January, two thousand and eight

An Act

To amend the Public Health Service Act to establish grant programs to provide for education and outreach on newborn screening and coordinated followup care once newborn screening has been conducted, to reauthorize programs under part A of title XI of such Act, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Newborn Screening Saves Lives Act of 2007".

SEC. 2. IMPROVED NEWBORN AND CHILD SCREENING FOR HERITABLE DISORDER.

Section 1109 of the Public Health Service Act (42 U.S.C. 300b–8) is amended—

(1) by striking subsections (a), (b), and (c) and inserting the following:

"(a) AUTHORIZATION OF GRANT PROGRAM.—From amounts appropriated under subsection (j), the Secretary, acting through the Administrator of the Health Resources and Services Administration (referred to in this section as the 'Administrator') and in consultation with the Advisory Committee on Heritable Disorders in Newborns and Children (referred to in this section as the 'Advisory Committee'), shall award grants to eligible entities to enable such entities—

"(1) to enhance, improve or expand the ability of State and local public health agencies to provide screening, counseling, or health care services to newborns and children having or at risk for heritable disorders;

"(2) to assist in providing health care professionals and newborn screening laboratory personnel with education in newborn screening and training in relevant and new technologies in newborn screening and congenital, genetic, and metabolic disorders;

"(3) to develop and deliver educational programs (at appropriate literacy levels) about newborn screening counseling, testing, follow-up, treatment, and specialty services to parents, families, and patient advocacy and support groups; and

"(4) to establish, maintain, and operate a system to assess and coordinate treatment relating to congenital, genetic, and metabolic disorders.

"(b) ELIGIBLE ENTITY.—In this section, the term 'eligible entity' means—

"(1) a State or a political subdivision of a State;

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NBSTRN Scope of Work

Networks

- Clinical specialists
- State NBS laboratories & programs

Informatics

- Establish and administer an NBS biospecimen repository
- Facilitate research on development of new methods and technologies

Infrastructure

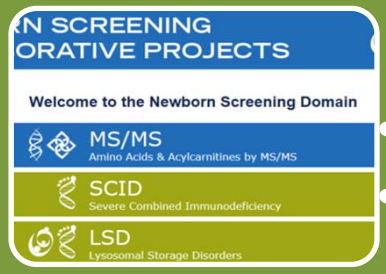
Facilitate Research

- Natural history studies including outcomes
- Novel screening technologies
- Novel therapies
- Genomics

Focus

- Ethical, legal and social issues
- Study planning
- Data aggregation and discovery
- Statistics

NBSTRN Research Tools

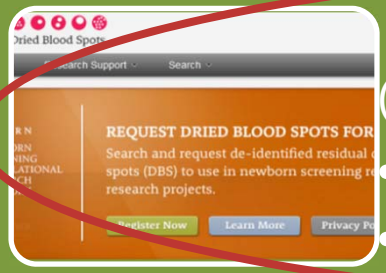


Region 4 Stork (R4S)

- Analytical and clinical validation
- Laboratory protocols, definitions

Longitudinal Pediatric Data Resource (LPDR)

- Secure, consensus-driven, standards-based clinical data collection & management
- Aggregate, share, and analyze data

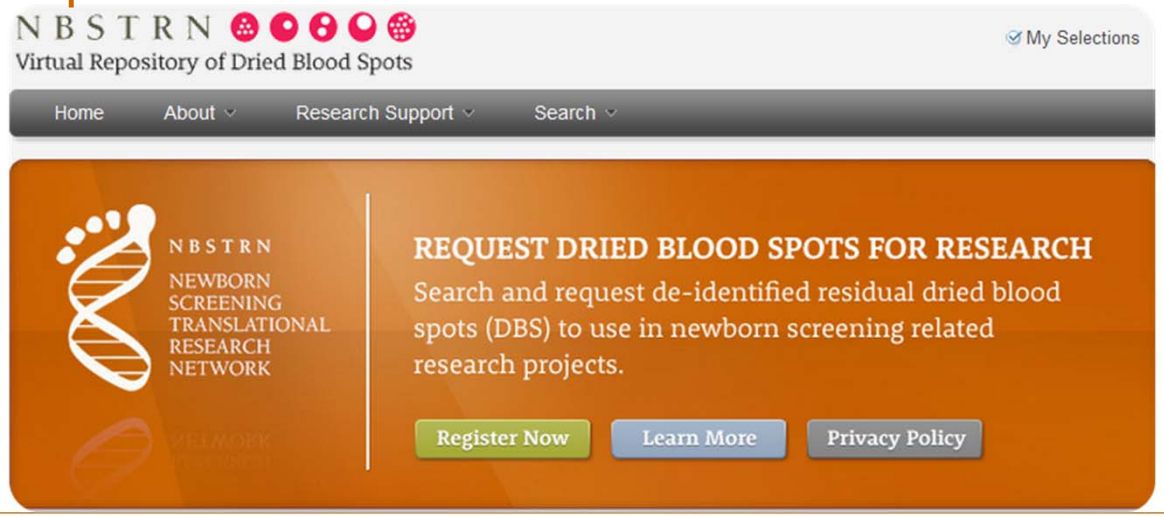


Virtual Repository of Dried Blood Spots (VRDBS)

- Search & request de-identified residual dried blood spots
- Secure, research support, & request management

Virtual Repository of Dried Blood Spots (VRDBS)

- **Secure, centralized & web-based**
- **Pilot phase 6/12 to 9/12 – production date: 9/26/12**
- **Inventory of DBS samples – over 2.6 million**
- **Investigators** can request letters of support, submit questions to participating states, browse & request specimens, track shipments & provide feedback
- **States** can respond to questions, review & manage requests, approve requests & control distribution



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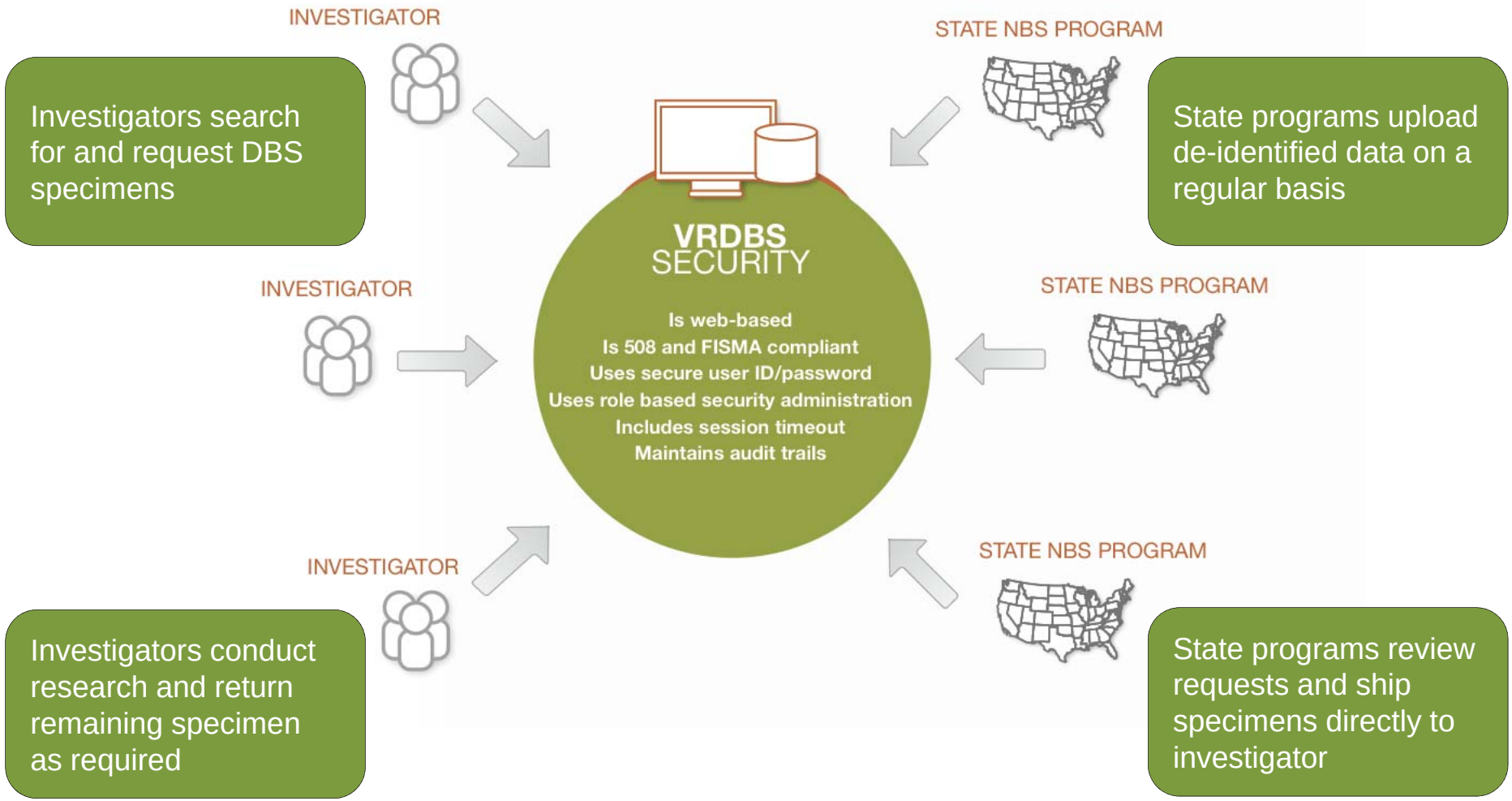
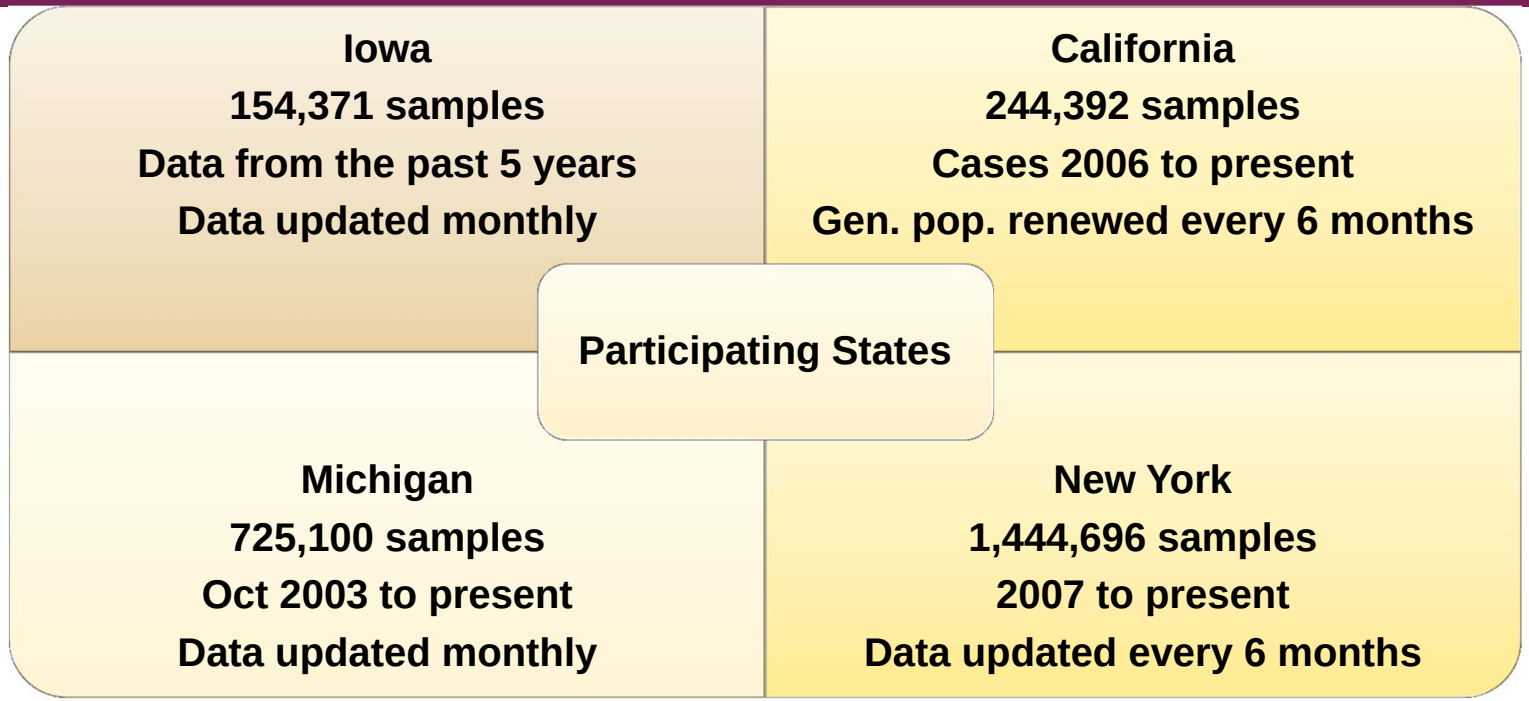
VRDBS Overview



VRDBS Status

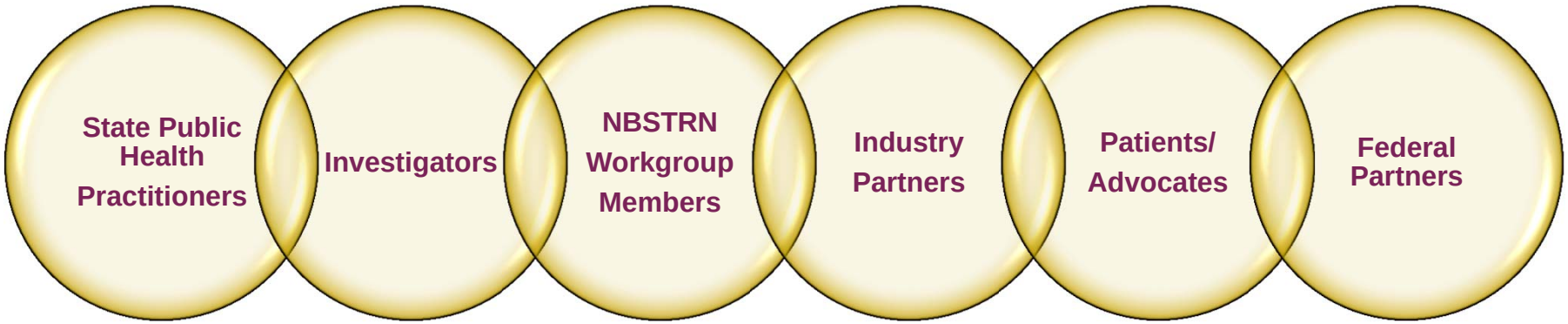


- 20 investigators have registered (1 more pending)
- 2 requests for specimens approved
- 2 questions submitted and responded to

* As of April 24, 2013

Working with Stakeholders

- **Engaged Stakeholders in 5 major ways:**
  - Rules under which the VRDBS operates
  - Privacy policies and site user agreements
  - Data Sharing Agreements with state programs
  - Material Transfer Agreements
  - Functional Assessments



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Protecting Privacy

- External ID
- State
- Birth year
- Storage Condition
- Birth weight
- Gender
- Age at Collection
- Race
- Ethnicity
- Condition
- Nutritional/ Feeding Status
- Status

"Rule of 5"

Search Results

You specified:

Diagnosed Condition: Primary Congenital Hypothyroidism - CH

Birth Year (yyyy): 2011 - 2013

Ethnicity: Hispanic, Latino/a, or Spanish origin

Sex: Female

The NBSTRN policy is to not list the number of specimens from a state if there are less than five. Your search resulted in less than 5 specimens that are from the following state(s):
Michigan
Please broaden your search (i.e., increase the year range and/or limit other search criteria). If this is still not resolved, please contact the state directly by clicking the state name above.

State	Stored Specimens	Qty Requested	Usage Restrictions
California	73		DBS Specimen Use Restrictions
New York	12		DBS Specimen Use Restrictions

1-2 of **2** Results **1** of 1 Page |< First << Prev 1 Next >> Last >| 20 Per Page

These numbers reflect only those cases in the virtual repository, not the incidence of the condition in the population.

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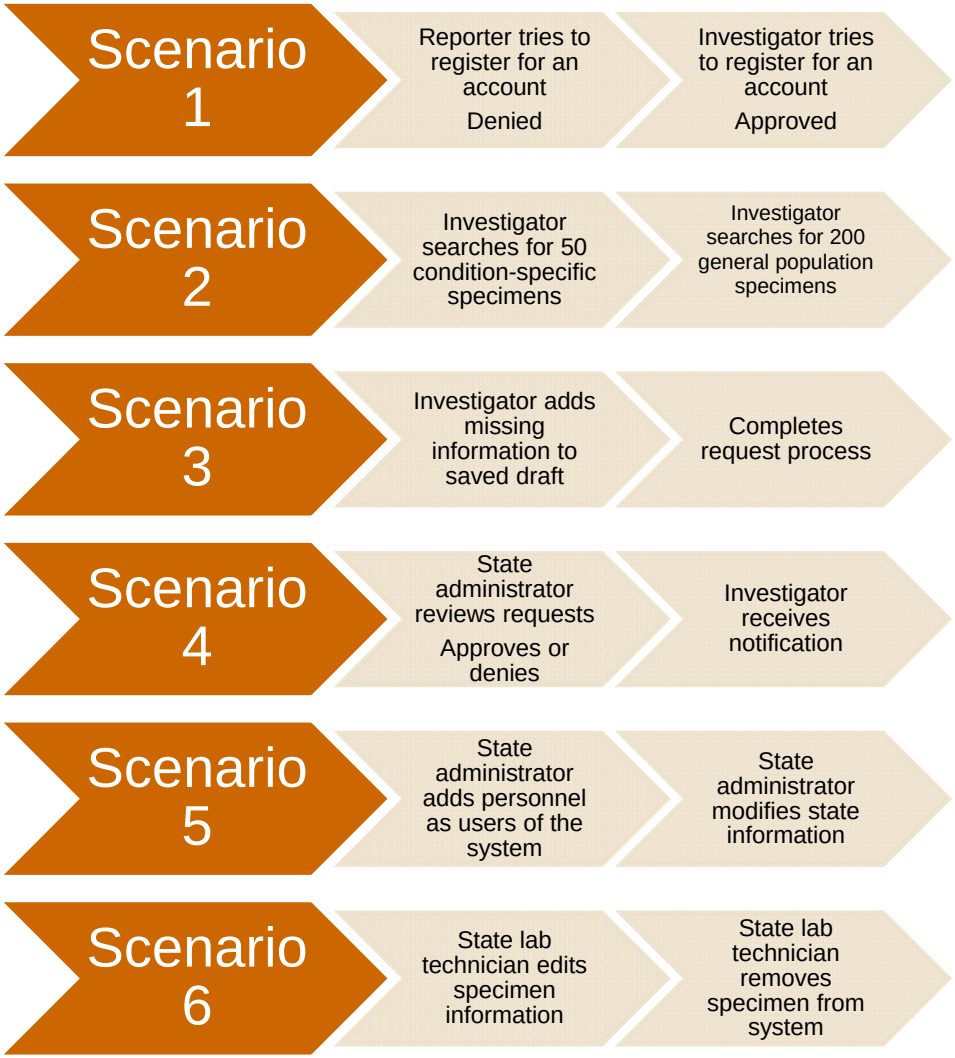
Information Security

- De-Identified data
- Registration approval
- Secure user ID and password
- Session timeout
- Role based security administration
- Secure data transfer for data imports
- Participating states retain control and distribution of their DBS

FISMA

- **Federal Information Security Management Act (FISMA)**
  - **Standards for categorizing information and information systems**
  - **Guidance for selecting appropriate security controls for information systems**
  - **Guidance for monitoring the security controls and the security authorization of information systems**
- **Categorization of Low, Moderate or High risk**
- **Annual review of system**

Functional Assessment



- **Homepage revamped**
- **Privacy policy link on homepage**
- **Account registration process streamlined**
- **Search page updates**
- **Developed research support section**
- **6 simple steps for "how to use this site"**

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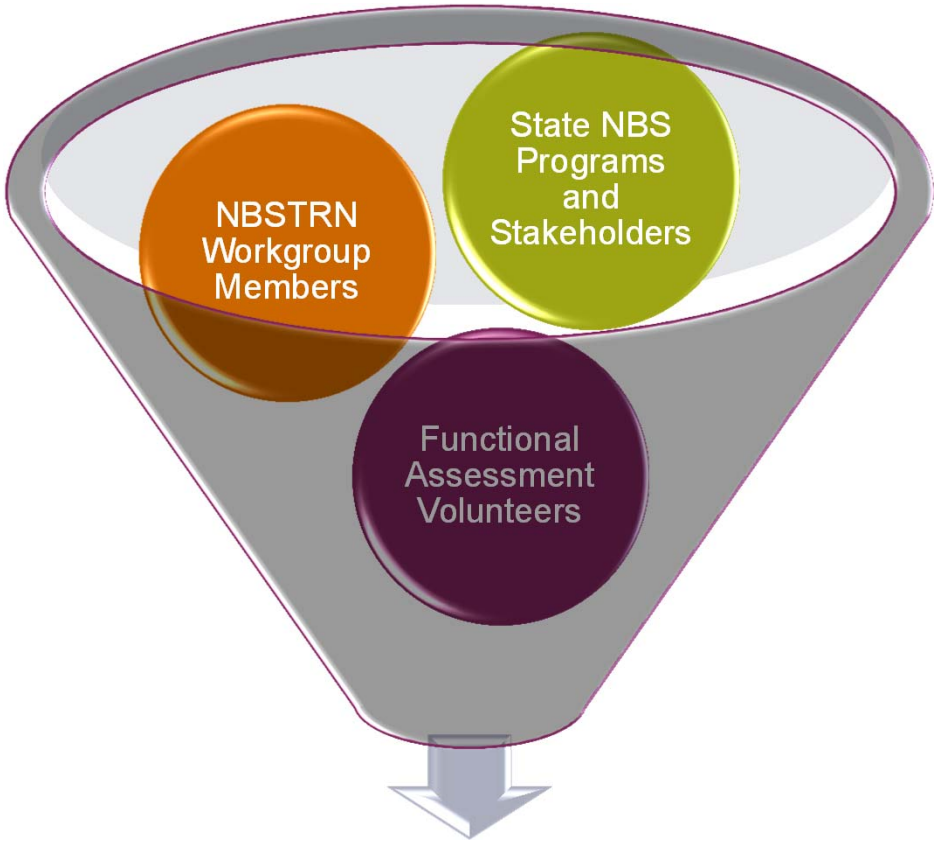
Coming to Consensus



VRDBS

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Acknowledgements

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NBSTRN Workgroup Members & NIH Investigators

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