Sickle Cell Disease Biospecimens in the NHLBI Biorepository

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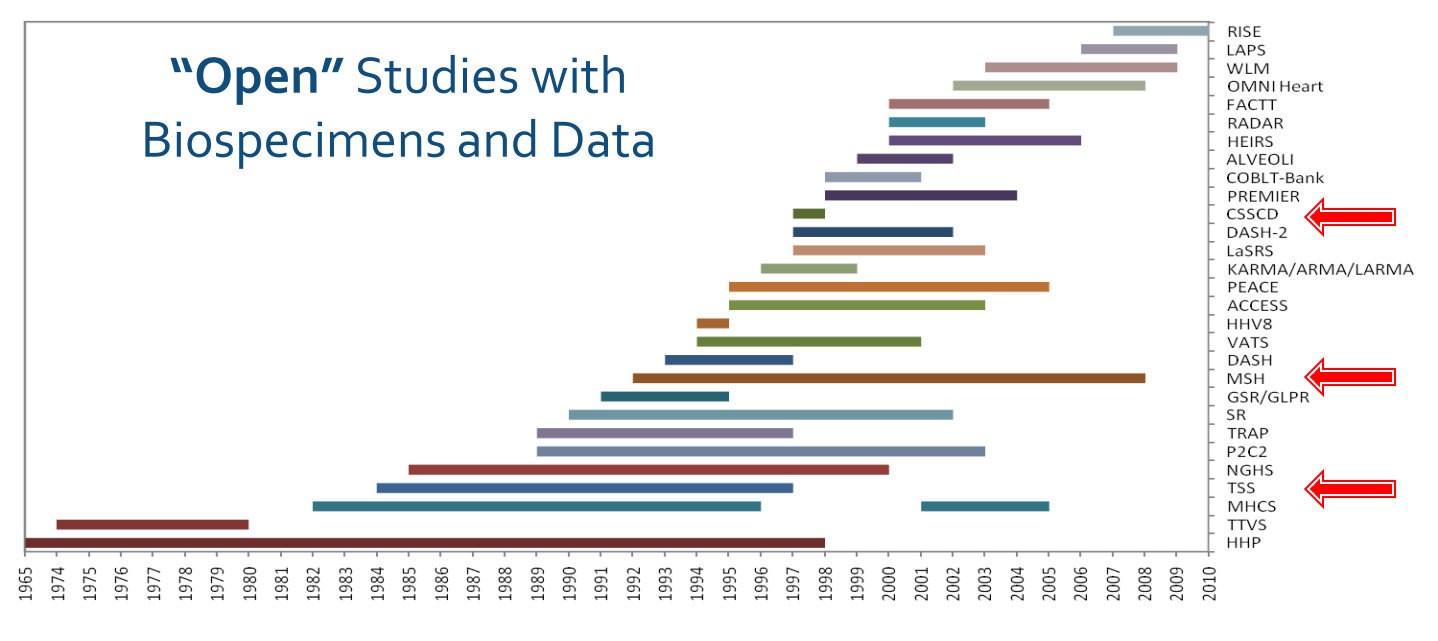
Biological Specimen and Data Repository Coordinating Center (BioLINCC)

- BioLINCC provides scientific researchers with online access to two unique NHLBI scientific resources:
  - NHLBI Biologic Specimen Repository (Biorepository)
  - NHLBI Data Repository
- The Biorepository was established in 1975 to provide long term storage and distribution of biospecimens collected on NHLBI clinical studies
- The Data Repository was established in 2000 to centralize NHLBI clinical study data and many of the these studies have biospecimen collections in the Biorepository

BioLINCC Holdings

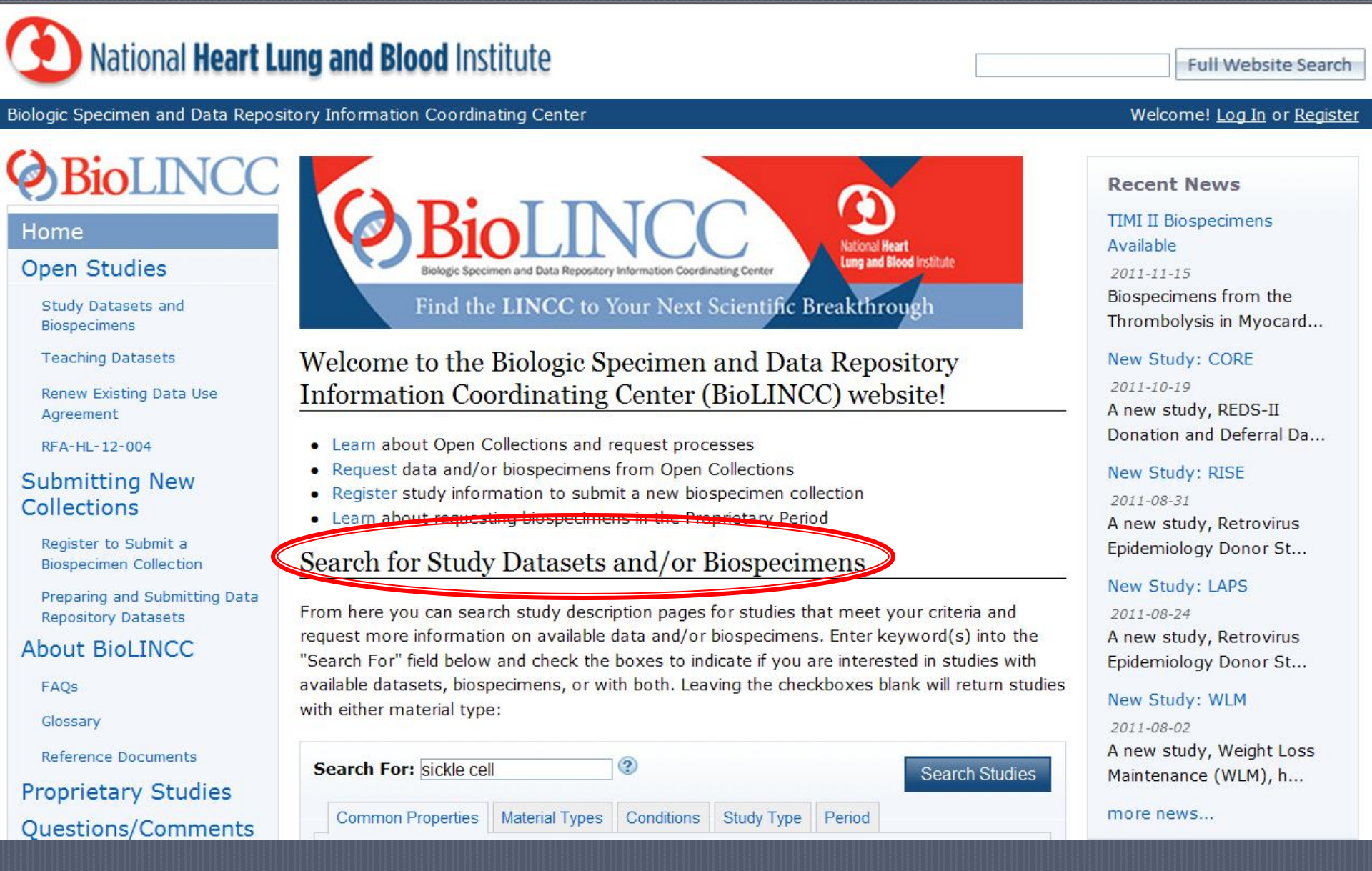
- The BioLINCC website currently has:
  - Datasets from more than 80 clinical studies
  - Over 4.5 million biospecimens from 30 clinical studies linked to their phenotypic data



BioLINCC Website

- Provides customized data-based searches for biospecimens
- Online submission of requests for datasets and/or biospecimens
- Allows submission of requests for additional information

www.biolincc.nhlbi.nih.gov



Multicenter Study of Hydroxyurea (MSH)

Clinical Trials URL: http://clinicaltrials.gov/ct2/show/...

Study Type: Clinical Trial

Prepared on March 16, 2010

Study Period: 1992-2008

Consent: Unrestricted Consent

Commercial Use Restrictions: No

Objectives

The primary objective of MSH was to determine whether or not treatment with hydroxyurea titrated to maximum tolerated doses would reduce the frequency of vaso-occlusive (painful) crises by at least 50%. The principal end point was the occurrence of a vaso-occlusive (painful) crisis, defined as pain not due to another cause, lasting at least four hours and requiring parenteral (or equivalent doses of oral narcotics)) narcotics or non-steroidal anti-inflammatory drugs for relief. Occurrences of chest syndrome were counted as crises. Pain due to chronic conditions such as ankle ulcers, osteomyelitis or aseptic necrosis of bone was not counted as crises. The secondary objectives investigated the correlations of fetal hemoglobin (HbF) levels and other patient or treatment characteristics with the occurrence of vaso-occlusive (painful) crises, and the effect of treatment on the quality of life.

Background

Sickle cell anemia affects nearly one in every five hundred African-American newborns in the United States. As of the early 1990's there was no available effective treatment for patients with sickle cell anemia for the prevention or reduction of recurrent, vaso-occlusive (painful) crises. There are an estimated 80,000 to 100,000 people in the United States with sickle cell anemia. At least 10% (8,000 to 10,000) of adults with sickle cell disease have more than three crises per year, based on projections from the Study of the Cooperative Study of Sickle

Resources Available

Specimens and Study Datasets

Materials Available

BUFFY COAT	150
DNA	261
SERUM	1,349

Study Documents

Data Dictionary (PDF - 1.5 MB)

Clinical Protocol (PDF - 218.1 KB)

EXT-1 Protocol (PDF - 1.7 MB)

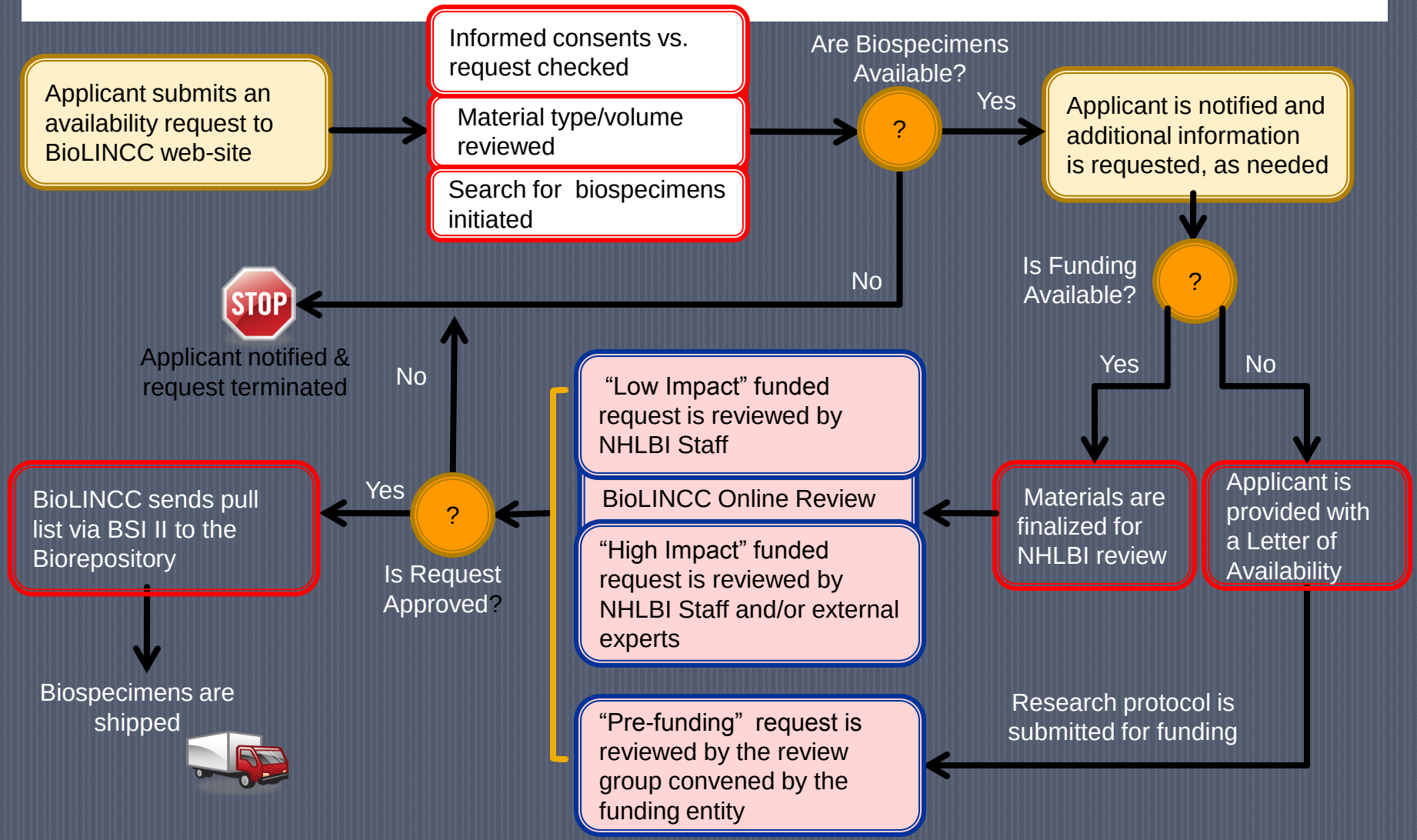
Forms (HTML)

PFU Protocol (PDF - 289.7 KB)

Publications (PDF - 39.7 KB)

Persons using assistive

Review of Biospecimen Requests



Multi-center Study of Hydroxyruea

- 1992-1994
- Phase III randomized double-blind study to assess the effect of hydroxyurea in reducing the frequency of painful SCD crises
- 299 adult subjects, who experienced 3 or more crises/year

Comprehensive Study of Sickle Cell Disease (CSSCD)

- 1978-1988
- Prospective study of clinical course of SCD
- 3,764 subjects enrolled from birth to age 66 at 23 centers in US

Comprehensive Study of Sickle Cell Disease (CSSCD)

- "Infant" cohort: subjects < 6 mo old at time of enrollment
- "Adult" cohort: subjects > 35 y/o at time of enrollment

Comprehensive Study of Sickle Cell Disease (CSSCD)

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- "Adult" cohort: subjects > 35 y/o at time of enrollment

Comprehensive Study of Sickle Cell Disease (CSSCD)--Limitations

- Informed consents for adult cohort did address DNA testing—collection can be shared
- Informed consents for infant cohort was silent on DNA genotyping—collection cannot be shared

Study Collection	Serum	Plasma	DNA
Cooperative Study of Sickle Cell Disease (CSSCD)	X		X (adult cohort)
Multicenter Study of Hydroxyurea (MSH)	X		X
Transfusion Safety Study (TSS)	X	X	

NHLBI FUNDING OPPORTUNITY

RFA-HL-12-004
Maximizing the Scientific Value of the NHLBI Biologic Specimen Repository: Scientific Opportunities (R21)

http://grants.nih.gov/grants/guide/rfa-files/RFA-HL-12-004.html

Non-AIDS Application Due Date: May 4, 2012
AIDS Application Due Date: August 17, 2012