Objective

To describe the development of a uniform system of common data elements (CDEs), including Patient Reported Outcomes (PROs) for data collection within the Sickle Cell Disease Implementation Consortium (SCDIC) needs assessment and registry.
Genesis of the SCDIC 2002-Present

2002

Adults with SCD: Meeting Unmet Needs

2004-Current

WGs on Healthy People Objectives

2006-2010

ASCQ-Me® and PROs

2008

NIH Consensus Development Conference on Use of Hydroxyurea in SCD

2010-2014

NHLBI Evidence-Based Management of SCD

2014-Present

PhenX Toolkit in SCD

2016-Present

SCDIC

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From Research to Practice

Bench
- Basic Science Research
  - Preclinical Studies
    - Animal Research
- Preclinical Studies
  - Phase 1 and 2 Clinical Trials
- Preclinical Studies
  - Phase 3 Clinical Trials

Bedside
- Human Clinical Research
  - Controlled Observational Studies
  - Phase 3 Clinical Trials
- Human Clinical Research
  - Case Series

Practice
- Clinical Practice
  - Delivery of Recommended Care to the Right Patient at the Right Time
  - Identification of New Clinical Questions and Gaps in Care
- Practice Based Research
  - Phase 3 and 4 Clinical Trials
  - Observational Studies
  - Survey Research

Translation To Humans
- T1 Guideline Development
  - Meta-analysis
  - Systematic Reviews
- T2 Dissemination Research
  - Implementation Research
  - Translation To Practice

From Research to Practice

Bench
- Basic Science Research
- Preclinical Studies
- Animal Research

Bedside
- Human Clinical Research
  - Controlled Observational Studies
  - Phase 3 Clinical Trials

Practice
- Clinical Practice
  - Delivery of Recommended Care to the Right Patient at the Right Time
  - Identification of New Clinical Questions and Gaps in Care

T1
- Case Series
- Phase 1 and 2 Clinical Trials

T2
- Translation To Humans
- Translation To Patients

T3
- Dissemination Research
- Implementation Research
- Translation To Practice

Takes average of 17 years for only 14% of new scientific discovery to enter day-to-day clinical practice.

Implementation Science
NHLBI’s Sickle Cell Disease Implementation Consortium

- NHLBI committed to funding research into the implementation of evidence-based therapies in heart, lung and blood disorders
  - Established NHLBI Center for Translational Research and Implementation Science in 2014
- Focus on disparities in healthcare and accelerations in advances needed in SCD
  - Patients often live in challenging social contexts, with poorly understood barriers to care
  - Youth and adults experience high mortality, severe pain, progressive decline in functional status, underutilization of evidence-based therapies, lack of access to knowledgeable care
Transition from Pediatric to Adult Care

**Healthcare Utilization**

- Hospital admissions and ED visits rise dramatically after age 18
- Over 40% of deaths occurred in patients 40 years and younger

**Age at Death for People with SCD, 2004-2008**

- 0-10 years: 4%
- 11-20 years: 6%
- 21-30 years: 15%
- 31-40 years: 17%
- 41-50 years: 24%
- 51-60 years: 20%
- >60 years: 14%

Paulukonis et al Pediatrics 2014;61:2271-6
<table>
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<td>Vichinsky/Treadwell</td>
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Coordinating Center – RTI
Steering Committee Chair – Alexis Thompson, MD
NHLBI Project Officer – Sharon Smith, PhD
SCDIC Phases

Phase I
- Needs Assessment
  - Qualitative and quantitative data collection agreed upon
  - Needs Assessment underway

Phase II
- Registry
  - Uniform system of common data elements including PROs determined
  - Sites beginning data collection

Phase III
- Implementation Studies
  - Planning studies:
    - Care Redesign
    - Emergency Department Care
    - Reducing # of unaffiliated patients
Conceptual Model for HRQOL in SCD

Interventions
Medical care
Coping techniques

Pain Impact
Very Severe
Urgent/unpredictable

Emotional Distress
Anxiety
Depression
Anxiety, depression about Health

Physical Distress
Fatigue
ADL/IADL Impact
Stiffness

Role Interference
Social
Family
Work (paid/unpaid)

Key:
- ASCQ-Me®
- PROMIS

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Treadwell et al Clin J Pain 2014;30;902-14
Keller et al Health Qual Life Outcomes 2014;12:125
Welcome to the PhenX Toolkit

Mental Health Research Collection - Early Psychosis is now available here!

The PhenX (consensus measures for Phenotypes and eXposures) Toolkit is a catalog of recommended, standard measures of phenotypes and environmental exposures for use in biomedical research. PhenX measures can be used to expand a study design beyond the primary research focus. Use of PhenX measures facilitates cross-study analysis, potentially increasing the scientific impact of individual studies. The PhenX Toolkit is a Web-based resource and is available for use at no cost. More >>
SCD Measures in PhenX
Needs Assessment Domains

- Patient Experience
- Patient Health Metrics
- Barriers to Care
- Demographics
- Medical History
- Health Literacy
- Utilization
- Treatments
Needs Assessment – Basic Survey

- phenX Toolkit
  - Demographics
  - SCD Genotype
  - Sickle Cell Disease
  - Self-Efficacy
- PROMIS
  - Pain Interference

- ASCQ-Me®
  - Pain Episode Frequency
  - Pain Episode Severity
  - Quality of Care
  - Provider Communication
  - Emergency Department Care
  - Access to routine and emergency care

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Needs Assessment – Optional Measures

- ASCQ-Me®
  - Emotional Impact
  - Social Functioning Impact
- PROMIS
  - Global Health – PROMIS 29
- HRSA SCDTDP
  - Barriers to Care
  - CAHPS
    - Prescription Medicine Costs
    - Health Literacy
    - Care from Specialists
    - Cultural Competency
    - Patient Centered Medical Home (Access)

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Registry -
Common Data Elements

- phenX Toolkit
  - Sickle Cell Disease Core Tier 1 and Core Tier 2 demographics
- PROMIS
  - Pain Quality
  - Emotional Distress – Depression
  - Fatigue
- Neuro-QOL
  - Cognitive Function

- ASCQ-Me®
  - Pain Episode Frequency
  - Pain Episode Severity
  - SCD Medical History Checklist
  - Emotional Impact
  - Pain Impact
  - Sleep Impact
  - Social Functioning Impact

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Research Questions

- Complications/Co-Morbidities
- Treatments
- Access to Care
- Quality of Life and PROs
  - Social determinants of health and HRQoL
  - Pain experiences, healthcare utilization as a function of age, gender
  - Geo-mapping and disability attributable to SCD
  - Longitudinal changes in HRQoL
Conclusion

- Comprehensive HealthMeasures systems, PhenX Toolkit
  - Provide foundation for understanding patient needs then developing and evaluating multi-modal, multi-sector interventions to address longstanding and pervasive disparities that youth and adults with SCD face in accessing quality healthcare