CURE SICKLE CELL.

Questions and Answers
CureSCi Standard Form Development

What is the purpose of developing common data elements (CDEs) or standard forms?
The purpose of developing these CDEs and their related forms is to standardize the collection of investigational data to facilitate comparison of results across studies and more effectively aggregate information into significant metadata results.

The development of standard forms is part of a broader Cure Sickle Cell Initiative (CureSCi) effort within the National Heart, Lung, and Blood Institute (NHLBI). This is a collaboration with other harmonization efforts including but not limited to:

- Adult Sickle Cell Quality of Life Measurement Information System;
- Blood and Marrow Transplant Clinical Trials Network (BMT CTN);
- Center for International Blood and Marrow Transplant Research (CIBMTR);
- Center for Medical Technology Policy/Green Park Collaboratives coreSCD Outcomes;
- Data Consortium Metadata Catalog;
- Food and Drug Administration (FDA) and American Society of Hematology (ASH) End Points Publications;
- PhenX Measures for Sickle Cell Disease;
- Patient-Reported Outcomes Measurement Information System; and,
- Retrospective data elements – Historical/Current Study data elements.

What are the CDEs that standard forms collect?
The CureSCi library of recommendations or CDEs will facilitate the preparation of data collection tools and present the current state of science through a hierarchical presentation (Figure 1) of data elements to collect along with most guidelines for clinical research genetic studies in sickle cell disease (SCD).

Disease Core: Data element that should be collected in any sickle cell study. It is anticipated that investigators will need to collect the Disease Core CDEs.
Core: Data element that collects essential/required information based on the current clinical research best practices. If these elements are absent/lacking, it may impact the utility of the domain. These have been used previously in SCD studies and are required should the study design involve this domain as an important target outcome. Validated measures were used when available.

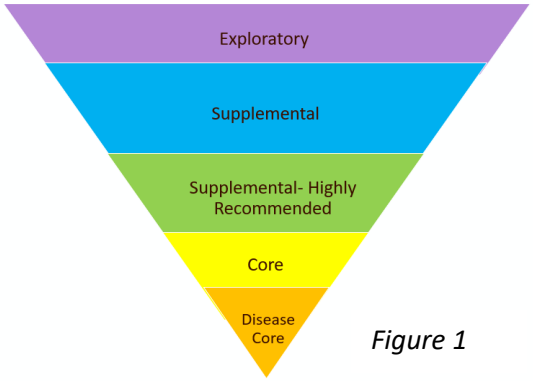

Figure 1

Supplemental-Highly Recommended: Data element that is important for study design or protocol but not required for collection.
Supplemental: Data element which is commonly collected in clinical research studies. Use depends upon the study design, protocol or type of research involved.
Exploratory: Data element that requires further development but addresses gaps in data reporting and/or could substitute for an existing element after validation. These type of data elements show promise but require validation before they are ready for standard use in clinical research studies.

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Why has NHLBI undertaken the effort to develop SCD Standard Forms?
To enable faster and more efficient study start-up, as well as better data sharing and data mining. This is part of a larger National Institutes of Health (NIH) roadmap for sharing data across studies.

Who was involved in the development of standard forms?
Five (5) working groups consisting of over 80 members were assembled to develop standard forms. The working groups were made up of clinicians, researchers, industry members and patients/patient advocates.

Who has been involved in the CureSCi SCD initiative? What types of participants? How many of each?
The working group roster is included with the public review packet and includes affiliation. Note that most groups have at least one patient, advocate or caregiver on them. There are about 12-18 members per working group.

What role has been expected of patients/patient advocates on these teams?
To participate alongside the investigators and comment on documents being created.

When will the first version be released? How will that be communicated to researchers?
Release is scheduled for Spring 2021. It will be sent to the emails and listservs and we will promote it through social media.

What are the expectations for researchers to use the standard forms in research?
NHLBI strongly encourages use of standard forms. CureSCi-funded studies are required to use the Core data elements unless an explanation is provided for not collecting them. The Supplemental-Highly Recommended elements within the forms may also be required based on study focus.

What is the overall timeline for standard forms development?
Development of standard forms entails work and coordination of several working groups, internal review, public comment period, edits, and the release of version 1.0. The timeline is approximately nine to 12 months from the orientation meeting.

When is the public comment period?
The public review period is from December 16, 2020 – January 31, 2021.

Who will NHLBI ask to give input, and what kind of input is NHLBI looking for during public comment?
Anyone can provide comment as the public review packet is posted to curesickle.org website. NHLBI will ask for input from investigators, patient advocate groups and others.

How can I submit my comments?
Comments can be accepted via email (CureSCinfo@emmes.com) with or without the comment spreadsheet provided.

Who is responsible for reviewing public comments and modifying CDEs?
Comments received during public review will be brought to the CureSCi standard form development working groups, who will review and determine if changes will be made. Responses are sent as needed directly to the those who made the comments.

After version 1.0, how will changes be made to the standard forms?
An Oversight Committee (OC) will be created. The OC will review updates to the forms and elements on an annual basis. Membership will be about 8-10 individuals from both the original working groups and new members not previously involved with the recommendations. Clinicians, researchers and patients/advocates/caregivers will be on the OC.

Who can request changes? How are they processed?
Anyone can submit feedback at any time through the website link. Proposed changes will be brought to the OC for review.

How will standard forms fit with the Data Consortium's efforts?
The development of genetic-specific data standard forms is part of this larger CureSCi project to develop standards for funded clinical research in SCD and facilitate data sharing. The expectation is that NHLBI-funded studies will incorporate the standard forms and elements as needed.