

Summary recommendations

Subdomain	Population	Instrument Name	Classification	Notes
Pain Intensity	Adults, children 8 years old and above	Numeric Rating Scale (NRS)	Core	With appropriate time anchors and serial measures for chronic and acute pain.
Pain Intensity	Adults, 8 years old and above	Visual Analog Scale (VAS)	Supplemental	The panel considered the Numeric Rating Scale to be more easily administered by a broader range of means, to require less equipment, and to be more generalizable to clinical practice. However, the VAS has certain favorable measurement properties and arguments could be marshalled for its use in some studies.
Pain Impact/ Interference	Adults	ASCQ-Me Pain Impact	Core	Preferred if the study population are adults.
Painful Crises	Adults, specific to SCD	ASCQ-Me Pain Episodes	Core	Preferred if the study population are adults
Pain Impact/ Interference	Children ages 5–18 years, specific to SCD.	PedsQL Pain Impact SCD module (children)	Core	Preferred if the study population are children. Parent proxy measures are available for ages 2–18 years.
Pain: Mixed	Children ages 5–18 years, specific to SCD	PedsQL Pain and Hurt, SCD modules	Core	Preferred if the study population are children. Parent proxy measures are available for ages 2–18 years.
Pain Impact/ Interference	Children and Adults, not SCD specific	PROMIS Pain Interference; Adult and Pediatric modules	Core	An appropriate alternative measure to ASCQ-Me and PedsQL, should comparison across conditions be highly desirable, or if the study population will involve a mix of adult and pediatric

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				participants. If the study population is limited to either adults or children; ASCQ-Me or PEDS-QL measures (respectively) are preferred.
Emotional Impact of SCD	Adults, specific to SCD	ASCQ-Me Emotional Impact	Supplemental, Highly Recommended	
Emotional Impact of SCD	Children, specific to SCD	PedsQL, SCD Module Emotions	Supplemental, Highly Recommended	
Emotional Impact of SCD	Children, specific to SCD	PedsQL, SCD Module Worrying	Supplemental	
Negative Affect: Mixed	Children	PROMIS Pediatric Physical Stress Experience	Supplemental, Highly Recommended	
Low Mood	Children and Adults, not SCD specific	PROMIS Emotional Distress: Depression (Pediatric and Adult)	Supplemental, Highly Recommended	While these measures are not designed to measure the emotional effects of SCD, they are available for broad age ranges and can be used to compare across conditions which might make them appropriate for some study designs.
Anxiety	Children and Adults, not SCD specific	PROMIS Emotional Distress: Anxiety (Pediatric and Adult)	Supplemental, Highly Recommended	While these measures are not designed to measure the emotional effects of SCD, they are available for broad age ranges and can be used to compare across conditions which might make them appropriate for some study designs.
Fatigue	Children and Adults, not SCD specific	PROMIS Fatigue/Pediatric Fatigue	Core	
Fatigue	Children with SCD	PedsQL Multidimensional Fatigue Scale	Core	

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Sleep Disturbance	Children and adults, not SCD specific	PROMIS Sleep Disturbance	Supplemental, Highly Recommended	Recommended for children with SCD; and in studies of mixed age
Sleep Disturbance	Adults	Pittsburgh Sleep Quality Index (PSQI)	Supplemental	
Sleep Disturbance	Adults with SCD	ASCQ-Me Sleep Impact	Supplemental, Highly Recommended	Recommended for studies exclusively of adults with SCD
Daytime Sleepiness	Adults, newer scale for children	Epworth Sleepiness Scale (and Epworth CHAD)	Supplemental	Child: http://pulmonary.pediatrics.med.ufl.edu/files/2012/09/epworth-sleepiness-scale-children.pdf
General Function	Adults	Canadian Occupational Performance Measure (COPM)	Supplemental	
Social Function	Adults with SCD	ASCQ-Me Social Functioning Impact	Supplemental	
Physical Function	Adults with SCD	ASCQ-Me Stiffness Impact	Supplemental, Highly Recommended	
Physical Function	Adults	PROMIS - Physical Function - 12a	Supplemental	
Physical Function	Children with SCD	Other PROMIS Physical Function measures, such as PROMIS Upper Extremity and Mobility, available for Pediatrics but not validated for Adults	Supplemental	
Global Health/ Quality of Life	Adults	PROMIS 10 Global Health	Core	
Global Health/ Quality of Life	Children	PROMIS 7+2 Global Health	Core	Parent/Caregiver Proxy measures are also available

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Global Cognition	0-3.5 years	Bayley-III	Supplemental	Should global cognition and development be a focus of the study for infants, this would be preferred.
Global Cognition	2.5-7 & 7/12	Wechsler Preschool and Primary Scale of Intelligence WPPSI-IV (+consider WPPSI Cancellation)	Supplemental	Should global cognition and development be a primary focus this will be a reasonable outcome measure; participant burden and resource need should be weighed against more specific measures
Global Cognition	6-16 & 11/12	Wechsler Intelligence Scale for Children (5 th Ed) WISC-V	Supplemental	Should global cognition and development be a primary focus this will be a reasonable outcome measure; participant burden and resource need should be weighed against more specific measures
Global Cognition	Adults	Wechsler Adult Intelligence Scale WAIS-III	Supplemental	Should global cognition and development be a primary focus this will be a reasonable outcome measure; participant burden and resource need should be weighed against more specific measures
Global Cognition	3-6, 1-17, and 18+ Depending on Battery	NIH Toolbox	Supplemental, Highly Recommended	Generally preferred over other cognitive measures due to minimal resource requirements, rapid administration requiring modest training, and inclusion of relevant subscales (such as processing speed).
Executive Functioning (and Attention)	Versions for 3-7, 8-11, and 12+	NIH Toolbox: Flanker Inhibitory Control and Attention Test	Supplemental, Highly Recommended	NIH Toolbox measures generally preferred over other similar measures due to low participant burden and minimal resource requirements, as well as broad age ranges.

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Executive Function	Versions for 3-7, 8-11, and 12+	NIH Toolbox: Dimensional Change Card Sort Test	Supplemental, Highly Recommended	NIH Toolbox measures generally preferred over other similar measures due to low participant burden and minimal resource requirements, as well as broad age ranges.
Executive Function	9 and up	Trail Making Test, parts A and B	Supplemental	
Executive Function	8-89 years	Delis-Kaplan Executive Function System D-KEFS	Supplemental	
Executive Function	6 years, 5 months to 89	Wisconsin Card Sort Test	Supplemental	
Processing Speed	Age 7+	NIH Toolbox: Pattern Comparison Processing Speed Test	Supplemental, Highly Recommended	NIH Toolbox measures generally preferred over other similar measures due to low participant burden and minimal resource requirements, as well as broad age ranges.
Processing Speed	Adults	Processing Speed Index (of WAIS-III)	Supplemental	
Working Memory	7+	NIH Toolbox: List Sorting Working Memory Test	Supplemental, Highly Recommended	NIH Toolbox measures generally preferred over other similar measures due to low participant burden and minimal resource requirements, as well as broad age ranges.

Exploratory Measures

Domain	Measure	Comments
Pain Mechanism	Quantitative Sensory Testing	If a study involves a hypothesis as to neural (and other) mechanisms of changes in pain, quantitative sensory testing may provide additional supporting evidence.
Sleep Impact/Sleep Disturbance	Polysomnography	If a study involves hypotheses as to mechanisms or physiologic correlates of sleep disturbance or sleep interference, polysomnography may provide additional evidence.
Life Satisfaction	PROMIS Pediatric Item Bank Life Satisfaction (Short Form 4a)	Life Satisfaction might be of interest beyond general health and quality of life in some studies.
Stigma	Measure of Sickle Cell Stigma	Stigma is often reported as a distressing psychological and social aspect of SCD, and the effect of curative therapy on this may be of interest in some studies.
Coping Skills / Pain Coping	Coping Strategies Questionnaire – Revised for SCD	Assessing changes in coping, as well interactions between coping styles and treatments or treatment selection, may be of interest in some studies.
Caregiver Burden	Caregiver Burden Scale	While the primary outcomes of curative therapies should center on the person with SCD; the effects on families and caregivers can be serious and improvements in this domain may be important to document.

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