

## Proposed Changes to List of Candidate Outcomes for Final Delphi Round\*

DECISION	OUTCOMES <i>(as included in Round 2 Delphi survey)</i>	RATIONALE	
<b>Remove from consideration for core set</b>  <i>(although recommendations regarding any of these outcomes might still be part of the reporting from this project)</i>	Acute kidney injury	Complication of treatment that would be considered as a safety measure	
	Pregnancy complications	Very important topic that needs further study, but not enough known at this point to include as a clinical trial outcome	
	Venous thromboembolism	Indirect outcome linked to heavy medication during acute pain crisis	
	Transcranial doppler velocities	Appropriate for pediatric but not adult trials	
	Level of hemoglobin	There is substantial agreement that the relevance of each of these blood biomarkers is dependent on the mechanism of action of the specific intervention, and therefore not appropriate for a core set of outcomes relevant across interventions	
	Level of fetal hemoglobin		
	Level of sickle hemoglobin		
	Hemolysis		
Change in hematocrit			
Oxygen % saturation			
<b>Move to different domain</b>	Pain interference/impact	These were originally included under physiological/clinical outcomes, but there is agreement that both should be considered aspects of functioning with the recommendation that they be measured as part of HRQOL	
	Fatigue		
<b>Combine</b>	Sickle cell nephropathy <u>and</u> kidney function	A diagnosis of nephropathy occurs along the spectrum of kidney function and is based on the same types of measures	
	Emergency department visit <u>and</u> acute care visit	Whether an individual with SCD goes to an emergency department or acute care center for a pain crisis is largely dependent on accessibility	
<b>Rename/redefine</b>	VOC	<i>(replaced by the following outcomes)</i>	Goal is to capture experience of acute severe pain directly related to sickle cell that may or may not result in an individual seeking medical care.
	Pain frequency	Frequency of acute sickle cell pain	
	Pain intensity	Intensity of acute sickle cell pain	
	Pain duration	Duration of acute sickle cell pain	

\*As discussed at in-person consensus meeting and subsequently endorsed by Delphi Panel