



August 20th, 2019

Dear Ms. Edmond and Ms. Koola,

Sick Cells appreciates the opportunity to engage with the Institute for Clinical and Economic Review (ICER) during their assessment for treatment of sickle cell disease. We are thrilled that ICER recognizes the importance of receiving input from the patients, families, patient advocacy organizations, and clinicians that are affected by this condition. Below we have included a list of concerns and recommendations regarding the timeline and key dates associated with the assessment for sickle cell disease treatment:

1. **Concern:** The current “Open Input” period, spanning from August 9 through 27, does not allow enough time for significant input of the sickle cell disease community. Given the limited data that describes patient needs, preferences, and issues for the sickle cell disease community, additional time will be needed to collect data and ensure the patient perspective is incorporated into the ICER assessment.

**Recommendation:** ICER should expand the timeline for the current “Open Input” period to a 90-day period. This would allow time for Sick Cells and other key stakeholders to field a new survey to gather information on aspects of treatments that patients find most important. Data on responses from patients will be a critical complement to clinical data and help inform the clinical and economic value of new sickle cell disease therapies.

2. **Concern:** ICER has not yet distinguished methods of assessments of treatments for conditions impacting minority populations. Sickle cell disease affects predominantly Black and Brown communities. The current methods of value assessment are based on majority white populations.

**Recommendation:** ICER should host a stakeholder meeting to gather further input on necessary adaptations to the standard assessment methods to be used during the evaluation of treatments for sickle cell disease. There are important insights to be gained from discussions with patient groups and other stakeholders regarding unique tensions related to clinical and economic impact of sickle cell disease. Given the persistent racial and socioeconomic disparities that impact the community, modifications to the value assessment framework are needed to include a broader frame of evidence and perspective.

3. **Concern:** Clinicians advised patients with sickle cell disease to avoid extremes of temperature, especially cold weather, based on evidence that more red blood cells undergo sickling with temperature changes. There are concerns that the March 2020



public meeting of the New England Comparative Effectiveness Public Advisory Council (CEPAC) may be inappropriate given the risk to patients in order to travel to this event. Additionally, given the large concentration of sickle cell patients in the midwestern and southern regions of the United States, the location of this meeting also presents concerns.

**Recommendation:** ICER should modify the meeting date and location for the sickle cell disease public meeting. The Midwest Comparative Effectiveness Public Advisory Council should serve as the review board, given the centralized location to many patient advocacy groups. Additionally, the meeting shall be held in summer to avoid complications and risk to patients that will participate in the public meeting.

We sincerely hope that ICER takes the above recommendations into account in revising the key dates and timeline, and we welcome the opportunity to address any questions you may have about the information above.

Sincerely,

Ashley Valentine, MRes  
CEO, Sick Cells

Maggie Jalowsky  
Program Manager, Sick Cells

Adrienne Shapiro  
Board Member, Sick Cells