



AMERICAN SOCIETY OF HEMATOLOGY

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September 20, 2019

Steven D. Pearson, MD, MSc
President
Institute for Clinical and Economic Review (ICER)
Two Liberty Square
Boston, MA 02109

Re: ICER's Assessment of Treatments for Sickle Cell Disease

Dr. Pearson,

The American Society of Hematology (ASH) is pleased to offer comments in response to the Institute for Clinical and Economic Review's (ICER) Draft Scoping Document on the assessment of the comparative clinical effectiveness and value of crizanlizumab (Novartis) and voxelotor (Global Blood Therapeutics) for the treatment of sickle cell disease (SCD).

ASH feels it is important to reiterate the concerns outlined in our [August 27 letter](#) regarding the history of SCD. As mentioned in our first comment letter, SCD was the first ever molecular disorder discovered, with the first case reported in 1846 and the first formal description coming in 1910. The SCD community has long been disenfranchised, and unfortunately, this has led to a lack of therapies and treatments. Additionally, there is a high level of mistrust of the health care community amongst this patient population. Currently, however, there is enormous opportunity for new, potentially life-changing treatments.

ICER does not conduct reviews for every approved or yet-to-be-approved therapy and this first review for treatments for SCD is coming at such a significant time in the research and development of new therapies for this disease. ASH is concerned that these factors could make this assessment appear as though SCD drugs are being held to a different standard; thereby, fueling the mistrust of this population and unintentionally, hurting access and future development of new therapies. ASH, the National Institutes of Health, and the Food and Drug Administration (FDA) have invested a great deal of resources to help change this dynamic. Given the number of new treatments in the pipeline, ASH is concerned that ICER's process could diminish the progress of the last several years. Therefore, the Society hopes that ICER's process will recognize the overall lack of progress on this disease until recently and the mistrust of the healthcare system felt by the patient population.

If ICER moves forward with this assessment, ASH would like to provide comments specific to the following areas of the Scoping Document:

1. Background
2. Analytic Framework
3. Interventions and Comparators
4. Key Outcomes and Harms

2019

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Background

ASH noted the following discrepancies in the *Background* section of the Scoping Document:

- There was a marked decrease in mortality in children under five in the US from 1979-2006, not in infants (Page 1).
- L-glutamine (Endari) is also FDA approved as a disease modifying therapy for SCD although the data is limited to sickle cell anemia (Page 2).

Analytic Framework

ASH offers the following comments on the Analytic Framework:

- ICER states that the population of focus for this review is children two years of age and older and adults diagnosed with SCD; however, there is no published data on the use of these new therapies in children age two. Crizanlizumab lower age was 16 and voxelotor was 12.^{1,2}
- As the Institute recognizes, there are a number of different genotypes of SCD and from what we know to date, not all genotypes will be eligible to receive both of the therapies under review. ASH requests that ICER's model be specific as to which segments of the SCD patient community it will include.

Interventions and Comparators

In the Scoping Document, ICER outlines its plan to compare crizanlizumab and voxelotor to “usual care alone,” defined in the document as hydroxyurea (HU) and transfusions. ASH wants to ensure that ICER's model will take into account that many patients do not receive what the Institute is defining as “usual care.” We strongly encourage the Institute to ensure that its model recognizes that “usual care” is suboptimal and accommodate for the current issues affecting access to “usual care,” as well as anticipate access challenges for the new treatments.

As was expressed on ASH's Scoping Call with ICER staff, “usual care” is very hard to define for this population. Care is not the same for any two patients with SCD – it may look different for different subtypes or for individuals who have suffered different complications of SCD. Many individuals living with SCD struggle to access care and those who successfully receive care may be hesitant to follow recommended care for a number of reasons. For example, ASH members find that many SCD patients are hesitant about taking HU because of the side effect profile that they find online while doing their own research. There is concern that it is “chemotherapy” and patients fear side effects, such as hair loss. While physicians do their best to dispel these myths, sometimes

¹ Ataga, K.I. et.al. Crizanlizumab for the Prevention of Pain Crises in Sickle Cell Disease. *New England Journal of Medicine*. 2017; 376(5):429-439.

² Elliott Vichinsky, et.al., A Phase 3 Randomized Trial of Voxelotor in Sickle Cell Disease. *The New England Journal of Medicine*. 2019;381(6):509-591.

families remain resistant to giving HU to their children. Similar concerns exist in the adult population. Additionally, some individuals cannot tolerate HU and therefore, do not take it.

Key Outcomes and Harms

ASH offers the following comments on the Key Outcomes and Harms:

- The Society would like to expand upon the significance of pain in the lives of individuals with SCD and wants to ensure it is adequately represented in ICER's model. ASH agrees that pain is a significant part of daily life for individuals with SCD and appreciates that both chronic and acute pain are listed in Table 1 and that a description of SCD pain is included in the *Background* section. Recurrent severe acute painful crises and chronic daily pain are the most common complications of SCD. Severe acute painful crises often require treatment in the hospital emergency department. In addition, many patients manage both their acute pain and chronic pain at home. Adequate management of acute and chronic pain associated with SCD is an ongoing challenge both for patients and the clinicians responsible for their care. ASH is currently in the process of finalizing five clinical practice guidelines on the management of acute and chronic complications of SCD, and pain is one of the topics addressed. Publication of the new ASH SCD guidelines is anticipated in late 2019 through early 2020.
- Hospitalization is listed under "Key Measures of Clinical Benefit." The Society recommends including emergency department visits and day hospital visits to this list.

Thank you for the opportunity to submit comments. Should you have any questions or if you would like to discuss these comments further, please reach out to Leslie Brady, ASH Policy and Practice Manager, at lbrady@hematology.org or 202-292-0264.

Sincerely,



Roy L. Silverstein, MD
President

Submitted electronically via publiccomments@icer-review.org.

September 18, 2019

Steven D. Pearson, MD, MSc
Institute for Clinical and Economic Review
2 Liberty Square, 9th Floor
Boston, MA 02109

Re: Institute for Clinical and Economic Review (ICER) Report on Treatments for Sickle Cell Disease (SCD)

Dear Dr. Pearson:

On behalf of our hospitals, primary care physician group and wellness centers in Nevada, Dignity Health-St. Rose Dominican appreciates the opportunity to submit comments on ICER's draft scoping document on two possible new treatments for SCD - crizanlizumab and voxelotor. Dignity Health is a part of CommonSpirit Health, a nonprofit, Catholic health system dedicated to advancing health for all people. With operations in 21 states and more than 140 hospitals, we are committed to creating healthier communities, delivering exceptional patient care and ensuring every person has access to quality health care. Again, we appreciate the opportunity to submit comments on this important measure.

As mentioned in your draft scoping document, there is clearly a large, unmet need for treatment options for SCD, and in general, the current health outcomes and treatment disparities for patients going through this disease are unconscionable. According to the American Society of Hematology's 2016 State of Sickle Cell Disease Report¹, SCD is also associated with high treatment costs. For an average person with SCD reaching age 45, total lifetime health care costs were estimated to be nearly \$1 million, with annual costs ranging from over \$10,000 for children to over \$30,000 for adults. The cost associated with these new drugs may end up being higher than the two current treatment options, but hope that they can bring about long-term cost savings, along with a formidable reduction in pain and suffering.

During the 2019 Nevada Legislative Session, a coalition of patients, family members and health care providers were able to pass a bill that will mandate the state to take a deeper look at this disease and how it affects its residents. AB 254 requires the State of Nevada's chief medical officer to establish and maintain a system for reporting information on SCD and make appropriate use of the data collected to report and assess trends regarding the disease. There are

¹ <http://www.scdcoalition.org/pdfs/ASH%20State%20of%20Sickle%20Cell%20Disease%202016%20Report.pdf>

Reference #4: Teresa L. Kauf, Thomas D. Coates, Liu Huazhi, Nikita Mody-Patel and Abraham G. Hartzema, "The cost of health care for children and adults with sickle cell disease," American Journal of Hematology 84, no. 6 (March 2009): 323-327.

anecdotal reports of challenges to care for SCD patients in Nevada, and this report will be used to determine any specific gaps in access to care for these patients, advance research of and improve treatment for those with the disease. This new law also requires screening for newborns susceptible to SCD and/or trait, along with voluntary screening for the newborn's parents, and mandates that Medicaid and other health insurers pay the non-federal share of expenditures for case management, programs and services for those with the disease. We hope that more states will pass similar laws to ensure that proper attention is paid to SCD. And now that better awareness will be paid to this disease in our state, we need to advocate for better treatments for those affected. We understand that both drugs being assessed are still with the US Food and Drug Administration (FDA) for clinical review and it hasn't been fully determined as to whether or not these pharmaceuticals will come to market for treatment of this disease. However, we also understand that the information found in this report will be used by others in the future, especially insurance companies, to determine whether or not these drugs will be allowed to be included in their formularies or authorized for patient use as a covered benefit. Please keep this in mind during the assessment process for this disparate patient population.

According to the Centers for Disease Control and Prevention (CDC)², sickle cell disease affects millions of people throughout the world and is particularly common among those whose ancestors came from sub-Saharan Africa, Spanish-speaking regions in the Western Hemisphere (South America, the Caribbean, and Central America), Saudi Arabia, India, and Mediterranean countries such as Turkey, Greece, and Italy. The exact number of people living with SCD in the United States is unknown, but it is estimated that it affects approximately 100,000 Americans, 1 in 365 Black or African-Americans and 1 in 16,300 Hispanic-Americans have the disease, and 1 in 13 Black or African-American babies is born with the trait. Our hope is that if the FDA approves these drugs for use in treatment of SCD, that your report will not delay access to these possibly lifesaving drugs for a patient population who so desperately needs them.

Compelled by our values – dignity, justice, collaboration, stewardship and excellence – St. Rose, along with our sister CommonSpirit Health hospitals, respectfully request your careful review of this matter. If you have any questions, please contact Katie Ryan, System Director of Grassroots Advocacy and Nevada Government Relations for CommonSpirit Health at 702-616-4847 or katie.ryan@dignityhealth.org.

Sincerely,

A handwritten signature in black ink, appearing to read "Chike M. Nzerue".

Chike M. Nzerue, MD, MBA, FACP
Chief Medical Officer
Dignity Health-St. Rose Dominican

² Centers for Disease Control and Prevention: <https://www.cdc.gov/ncbddd/sicklecell/data.html>



Kids Foundation

Submitted electronically via publiccomments@icer-review.org

Steven D. Pearson, MD, MSc

Institute for Clinical and Economic Review

2 Liberty Square, 9th Floor

Boston, MA 02109

Re: Institute for Clinical and Economic Review (ICER) Report on Treatments for Sickle Cell Disease (SCD)

Dear Dr. Pearson:

On behalf of Dreamsickle Kids Foundation, the first Sickle Cell Disease organization in Nevada and as a mother of a small child with Sickle Cell Disease (SCD), I would like to go on record to say that SCD has historically been ignored and there has been little to no medical interest taken in exploring this disease in effort to better manage or even cure the complications that afflict children and adults suffering from this illness. While I cannot speak for the entire SCD community, I can speak to the personal experience that I have had with being the mother, caregiver, and advocate for people affected by SCD.

In 2015, after being the mother of 1 for 13 years, I gave birth to a child who 1 week later was determined to have SCD. I initially had forgotten that I carried the Sickle Cell Trait (SCT), and though I was aware that her father carried the trait, I had been told by several medical professionals that I could not have more kids. I was already the great aunt of a little girl with SCD type SC and unfortunately with this information it still did not compel me to look into my own DNA especially since I thought I couldn't have any more kids that would run the risk of being born with the disease. Boy was I wrong.

After being notified that my child had SCD genotype SS and doing as much research on the condition. I was scared yet hopeful. I had been with my employer 10 years, so health insurance was not an issue and obtaining FMLA was also an easy task. For over a year, my child had no complications from SCD. We decided to prepare for future cost we would relocate from Orange County California (Disneyland) to Las Vegas, NV. In doing so I had to obtain a new job with a new employer. A few months into work I had revealed to employer my child's condition. I was told by management that not only had they not had an employee affected by SCD; they had never heard of the condition.

To give some insight on the burden of cost incurred by people affected by SCD I will share my personal experience. After leaving my employer in 2016, my new job did not start for 3 weeks. Being new to the disease and its affects, I figured 3 weeks without insurance would be no big deal as myself and my oldest child rarely had a need to visit a doctor or ER. This was a grave mistake, my child with SCD, Gia, came down with a serious case of pneumonia and was hospitalized in ICU for several days. This visit in 2016 has left me with a twenty-on thousand-dollar bill on my credit. After moving to Nevada Gia has had the misfortune of being hospitalized 6 times at least and receiving 8 transfusions. I was able to hang on to my job long enough to get FMLA often going to work from her hospital and leaving work to head straight back. By this time coupled with the fact that my employer had no knowledge of the disease other than what I educated them on. There was no compassion or understanding and I was let go after watching this same employer make accommodations for someone who had not worked long enough to have FMLA and had a child close to the age of my child, the only difference is the child had a heart condition. Those with experience with the effects of SCD know that crisis and complications are just as urgent and serious as a heart attack.

A week after being let go my child had another admission where it was discovered she had no insurance, having private insurance for 17 plus years, and remembering the 21k bill I had just received, I rushed to a state office to get medical as she was in hospital 5 days so the bill would be at least forty-thousand which would be another date I could not get from under. With her complications becoming more frequent, I knew that it was better to be home with her. Currently, we live off the \$771 from SSI, food stamps and free medical. This is from a person who left a job in California making almost 6 figures, to moving to NV and taking a pay cut of over half the salary to now only brining in \$771 monthly.

Due to the lack of awareness for SCD in NV, I founded Dreamsickle Kids Foundation to address those issues. With the two medications currently available for SCD, I have elected to hold off on the hydroxyurea and Gia is currently taking Endari along with several supplements. She has not had as many complications as many other people with SCD. The fact that only two drugs in 30 years are on the market to address SCD is disheartening. The fact that two more drugs are in the pipelines leaves me hopeful. It also gives us something to compare the current treatments to as opposed to those being the only option.

Currently, many researchers and pharmaceutical companies are taking interest to develop curative therapies for SCD. Patients with SCD have died and suffered for at least 100 years since the US discovery of this illness that is thousands of years old. People with SCD don't just want frivolous drugs or curative therapies thrown at them, but knowing that there has not been this much interest in the SCD community by the federal government or pharmaceutical companies the SCD community wants whomever is willing to invest into a better quality of life for the community to have a change.

As a mother and an advocate, I ask that you do your due diligence in reviewing the two new potential treatments for SCD from Novartis and GBT and make sure that a sound decision is determined on the necessity of these new treatments. Ask yourself, what is a life worth?

Sincerely,

Georgene' Glass, Executive Director

Dreamsickle Kids Foundation

www.dreamsicklekids.org

Global Blood Therapeutics (GBT) appreciates the opportunity to comment on ICER’s draft scoping document for crizanlizumab and voxelotor for Sickle Cell Disease (SCD). GBT’s demonstrated commitment to patients is our first priority, and we are uniquely and solely focused on SCD. Our commitment and focus remain on making novel therapies available for the improvement of the lives of SCD patients in the US and globally. As such, we intend to continue investing in innovative therapies, and ensure that patients have access to disease-modifying medicines.

Patients with SCD suffer from significantly reduced life expectancy, a profoundly compromised quality of life and multi-organ damage. Six percent of children with SCD do not live to adulthood.^{1,2} Those who survive childhood can at best hope to live only 3-4 more decades while facing a life with a devastating disease that will cause them an unimaginable series of symptoms, including: pain, fatigue, and a multitude of complications that will keep them in and out of the hospital throughout their lives.^{3,4} SCD robs patients, their caregivers and families of the precious things most people take for granted: the ability to work, to look after their families, and the freedom to pursue their dreams.

Voxelotor is the only late-stage drug to address the fundamental pathophysiologic mechanism of SCD, which results in multi-organ damage, the primary cause of SCD death.^{5,6,7} As oxygenated sickle hemoglobin does not polymerize, voxelotor may potentially modify the course of SCD by inhibiting polymerization and the resultant sickling of red blood cells, thus increasing hemoglobin levels and improving hemolytic anemia and oxygen delivery. Among persons with SCD in the HOPE trial who were treated with a 1500-mg dose of voxelotor, hemoglobin levels increased and incidence of worsening anemia decreased.⁸ Patients also experienced reductions in bilirubin levels and percentage of reticulocytes, consistent with a reduction in hemolysis.⁹ Higher hemoglobin is strongly associated with lower risk of end organ damage, thus voxelotor has the potential to reduce end-organ damage over time.¹⁰ Inhibition of HbS polymerization is critically important in improving SCD patient outcomes, which has been articulated by many SCD experts.^{11,12,13,14}

A premature ICER review could impact patient access to medicines in the near term and have a chilling effect on investment and R&D in SCD over the long-term. It is a national shame and tragedy that individuals living with SCD have been historically overlooked, underserved, and underrepresented with respect to overall quality of care and the availability of disease modifying therapies. Any review by ICER should not just capture, but also incorporate, a broad range of stakeholders’ feedback to have a truly inclusive process. Fears of treatment delays or the inability to access new treatments at all, features heavily in ICER’s draft scoping document: “*We heard...fears that even when new treatments become available there will be delays in their availability to patients because of a lack of provider knowledge*”,¹⁵ and “*delays in getting adequate pain medication, inappropriate delays in life-saving interventions in acute care facilities*”.¹⁶ As these delays and barriers to access are already so prevalent amongst the patient population, it is incumbent on ICER to assess voxelotor comprehensively and at the right time.

GBT’s main concerns can be summarized in three points: 1) GBT requests ICER delay the review of the novel and emerging SCD treatment, voxelotor; 2) ICER’s current value framework is inappropriate for assessing new SCD treatments under FDA’s accelerated approval pathway; and 3) multiple factors need to be considered for an accurate assessment, given the uniqueness of this disease. Based on the information provided, our detailed comments are below:

1. GBT requests and recommends ICER delay the review of the novel and emerging SCD treatment, voxelotor.

Insufficient investment has been a critical barrier to the development of new SCD therapies and the dearth of SCD patient data will make any assessment too theoretical and inaccurate. Despite the implementation of a nationwide screening program and the FDA's encouragement of the development of new treatments, there remains a lack of investment in, and sufficient knowledge of, SCD.^{17,18,19} One example of where this disparity is apparent is the level of government funding for SCD versus cystic fibrosis, a condition which affects approximately 30,000 individuals in the US. In 2012, the NIH spent \$65 million (\$650 per patient) on SCD versus \$86 million (\$2,867 per patient) on cystic fibrosis.²⁰ The result of increased funding from the NIH, as well as other sources such as private foundations, has led to an increased number of publications and therapies for largely Caucasian-based orphan drug conditions such as cystic fibrosis and hemophilia (see Figure 1, Appendix). For SCD, however, there are currently only two FDA approved drug therapies: hydroxyurea and L-glutamine, which speaks to the fragility of investment and research in this therapeutic category, and the importance of supporting, not unintentionally inhibiting, such focus.

To assess voxelotor (currently being reviewed under an FDA accelerated pathway), ICER will require long term natural disease history, which is inadequate due to historically limited SCD investment. ICER should defer review until outcomes from real-world data are available to inform ICER's analysis.²¹ The last large SCD registry was the Cooperative Study of Sickle Cell Disease (CSSCD), more than 25 years ago.²² The paucity of currently available longitudinal data will make it difficult, if not impossible, to understand the quality of patient care, health outcomes and utilization patterns for SCD. Patients with SCD face severe organ damage over their lifetime, and while it is a key outcome for patients and a point of value measurement to insurers, it would be extremely challenging for ICER to accurately measure the potential value of voxelotor to patients and the healthcare system with current data. Given the significant unmet need, voiced patient concerns on access delays, the monumental burden this disease places on patients and their families, and lack of information on the natural history of disease and confirmatory trial data, any value assessment of voxelotor conducted at this time would be fundamentally flawed and, therefore, lack the necessary validity and relevance to any decision-making.

2. ICER's current value framework is inappropriate for assessing new SCD treatments under FDA's accelerated approval pathway.

ICER has never assessed a disease like SCD. SCD's impact on vulnerable patient populations is profound. Matched with a marked lack of data within a multi-faceted disease, it is simply not possible to distill the impact of SCD or the value of any one therapy into a quality-adjusted life-year (QALY), nor the associated costs limited to that of just the payer. This renders traditional health economic assessments wholly incapable of accurately capturing value on which ICER's calculated recommended price would presumably be based. ICER's current value framework will predictably and inevitably unfairly penalize new SCD treatments that have undergone accelerated FDA approval, thereby negating the FDA's intention of ensuring that the agency's accelerated approval mechanism provides patients with earlier access to safe, effective, and promising treatments. Furthermore, ICER's value-based framework does not consider the social impact of SCD, including patients' loss of productivity, cognitive impairment, inability to work, school performance, and caregivers' burden, and tends to overestimate the long-term costs of a product by disregarding the inevitable precipitous price reduction after loss of exclusivity.

3. Multiple factors need to be considered for an accurate assessment given the uniqueness of SCD .

Societal and contextual considerations are base case for individuals living with SCD. While ICER claims it is planning to include broad and profound contextual considerations in SCD, and ensure that the voices of patients are heard and given the opportunity to more firmly influence decision-making in this assessment, these considerations are surprisingly not included in the base case assessment or value-based price (See Table 1, Appendix). ICER's current base case assessment tends to receive the most attention and communication without acknowledgement of additional considerations. Panel votes should have direct guidance on contextual considerations such that value is not solely determined by the incremental cost per QALY, but is founded on more inclusive, direct consideration of value to SCD patients, their families, and society.

Capture perspectives including cost and burden to patients and their caregivers in the base case. ICER acknowledged caregiver and family burden of SCD in the draft scoping document; however, this is not reflected in the base case of this assessment. When adjusted for diminished quality of life, the quality-adjusted life expectancy of patients falls to less than half of those without SCD. Conservatively, reduced life expectancy represents an estimated \$700,000 in lost lifetime earnings for those born with SCD compared to those without, equivalent to \$1.4 billion in lifetime income lost for an individual born with SCD in 1950 in the US, due to premature mortality (See Figure 2, Appendix).²³ In addition, ICER's base case should reflect the significant loss experienced by caregivers including lost earnings, care costs, and reduced caregiver QALYs.^{24,25}

Characterizing the pathophysiology by which anemia and hemoglobin impact end organ damage is exceedingly complex due to the effect on multiple organ systems, which is compounded by a poorly understood relationship between SCD genotypes and the associated phenotypes. SCD patients can experience cardiac, pulmonary, musculoskeletal, neurological, hepatobiliary, and splenic complications, which must be represented in ICER's assessment to comprehend the vast complexity and reach of this disease.²⁶ ICER's assessment should also include renal dysfunction, which accounts for 16-18% of SCD mortality.^{27,28}

ICER should not use the QALY or QALY thresholds to determine outcome or the value-based price for SCD patients. The quality-adjusted life-year, which is the key determinant of ICER's value calculation, is inadequate for SCD, and we are concerned that its application will be devastating for SCD patients by potentially discouraging coverage and access to treatments. It is impossible to condense into a single value (the QALY) all the multifaceted SCD quality of life and activities of daily living instruments of significantly heterogeneous areas used to measure SCD impact. These include not only physical manifestations, but pain, fatigue and cognitive effects notoriously more difficult to accurately measure and validate.²⁹ Also, there are unusual QALY patterns reflecting the intense burden of those ravaged by SCD: negative QALY values as high as -0.97 (values worse than death) from the additive effects of nausea, pain and other quality of life impacts are not uncommon in this patient population.^{30,31} Moreover, ICER thresholds are not appropriate for SCD, and it is patently inequitable to determine value with the same criteria as for common diseases.³²

Incorporate dynamic drug pricing. ICER should replicate guidelines from other International HTA bodies by including the entrance of lower-priced generics that follows the loss of exclusivity of branded products.^{33,34} Without the consideration of the inevitable availability of generics, innovative therapy cost will be significantly overestimated and societal long-term value underestimated.^{35,36}

The appropriate outcome for voxelotor is end-organ damage and mortality rates, not VOC.³⁷ The primary endpoint of voxelotor's phase 3 HOPE trial evaluates improvements in hemoglobin and was not enriched or powered to evaluate a statistically significant effect on vaso-occlusive crisis (VOC):³⁸ ICER will need to design a completely different model structure for voxelotor.

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- ⁸ *Op. Cit.*, Vichinsky et al. 2019. [Link](#)
- ⁹ *Op. Cit.*, Vichinsky et al. 2019. [Link](#)
- ¹⁰ Ataga, K. I., Gordeuk, V. R., Allen, I. E., Colby, J., Gittings, K., & Agodoa, I. (2018). Low Hemoglobin Increases Risk for Stroke, Kidney Disease, Elevated Estimated Pulmonary Artery Systolic Pressure, and Premature Death in Sickle Cell Disease: A Systematic Literature Review and Meta-Analysis. [Link](#)
- ¹¹ Dr. Elliot Vichinsky of the University of California, San Francisco, states, “Developing an HbS polymerization inhibitor has been a therapeutic strategy for the disease for decades ... Therefore, future drug development studies should focus on morbidity – not just pain management – as their primary outcomes.” ASH Clinical News. *Voxelotor Increases Hemoglobin, Decreases Hemolysis in Patients with Sickle Cell Disease*. 2019 Aug. [Link](#)
- ¹² “The improvement in anemia observed with voxelotor is extremely meaningful...hemoglobin (Hb) correlates directly to mortality and he absolutely expects accelerated approval based on the HOPE data generated to date. He believes it provides other clinical benefits as well (like improved QoL, transfusions, etc.).” PiperJaffray. *Sickle Cell Survey Findings; Bullish on GLYC & GBT Ahead of Upcoming Catalysts*. May 28, 2019, p. 16.
- ¹³ *Hb is not just a biomarker, levels are directly correlated with mortality.*” PiperJaffray. Insight by Key Opinion Leader. SCD KOL Call Highlights: Positive Outlook for both GLYC & GBT; Neutral on BLUE. May 13, 2019. P. 1.
- ¹⁴ Further, SCD patients with more hemolysis and lower Hb have more organ issues. Cowen. KOL bullish on novel therapies for SCD. *Equity Research*. June 30, 2019
- ¹⁵ ICER. *Crizanlizumab and Voxelotor for Sickle Cell Disease: Effectiveness and Value – Draft Background and Scope*. 2019 Aug, p. 3. [Link](#)
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- ²⁹ *Op Cit.* FDA. October 2014.
- ³⁰ Centre for Clinical Practice at NICE (UK). (2012). Full health economic report. In *Sickle Cell Acute Painful Episode: Management of an Acute Painful Sickle Cell Episode in Hospital*. National Institute for Health and Clinical Excellence (UK). [Link](#)

³¹ Floor effects of general measures such as EQ-5D are unable to capture SCD QALY accurately. *Op. Cit.*, Centre for Clinical Practice at NICE, 2012, [Link](#)

³² Another important consideration is that there is no research to demonstrate that QALY thresholds improve health, and initial research would suggest that it decreases health outcomes, which SCD patients can ill afford. IMS. Impact of cost-per-QALY reimbursement criteria on access to cancer drugs. *IMS Institute for Healthcare Informatics*. Dec. 2014.

³³ Hoyle M. Future drug prices and cost-effectiveness analyses. *Pharmacoeconomics*. 2008; 26(7), 589-602. [Link](#)

³⁴ Hua LH, Hersh CM, Morten P, Kusel J, Lin F, Cave, J, *et al.* The Impact of Price Reductions After Loss of Exclusivity in a Cost-Effectiveness Analysis: Fingolimod Versus Interferon Beta-1a for the Treatment of Relapsing-Remitting Multiple Sclerosis. *Journal of managed care & specialty pharmacy*. 2019; 25(4), 490-498. [Link](#)

³⁵ Hoyle M. Accounting for the drug life cycle and future drug prices in cost-effectiveness analysis. *Pharmacoeconomics*. 2011;29(1):1-15.

³⁶ *Op. Cit.*, Hua *et al.*, 2019 LH, [Link](#)

³⁷ ICER's draft scoping document says "the model will consist of health states including SCD with and SCD without VOC" ICER. *Crizanlizumab and Voxelotor for Sickle Cell Disease: Effectiveness and Value – Draft Background and Scope*. 2019 Aug, p. 7. [Link](#)

³⁸ *Op. Cit.*, Vichinsky *et al.* 2019. [Link](#)

APPENDIX

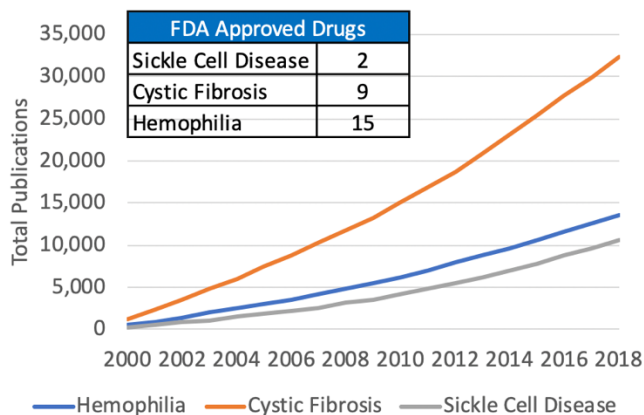
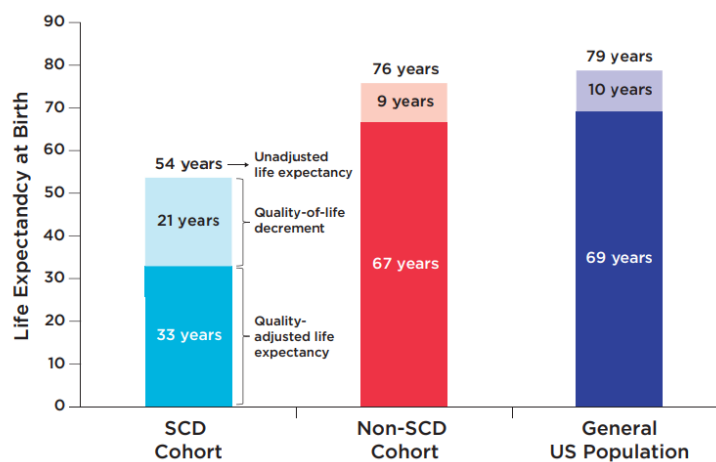
Figure 1: SCD Research Output^{39,40}**Figure 2: Projected Life Expectancy and Quality-Adjusted Life-expectancy at Birth ⁴¹**

Table 1: Potential Other Benefits and Contextual Considerations⁴²

Potential Other Benefits	
This intervention offers reduced complexity that will significantly improve patient outcomes.	Due to its narrow therapeutic window, required frequent monitoring, concerns over potential infertility, patient uptake and adherence of hydroxyurea (HU) is suboptimal. Given the manageable safety profile of voxelotor, we expect that the adherence to voxelotor to be much improved hence, a significant improvement in outcomes.
This intervention will reduce important health disparities across racial, ethnic, gender, socio-economic, or regional categories.	SCD is prominent in minority populations and those from lower socioeconomic status, primarily affecting African Americans and those of Hispanic descent. ⁴³ Voxelotor will provide benefit to patients who typically have a lower quality of life and severely shortened lifespan, thus reducing health disparities that tend to affect the poor and the underserved.
This intervention will significantly reduce caregiver or broader family burden.	In one study, a moderate negative linear correlation was found between caregivers of sickle cell disease patients and the financial and psychological impact to these caregivers and families of SCD patients is quite significant. ⁴⁴ Studies show that health expenditures for SCD make up 34% of the family income and was a source of financial stress, especially during SCD crises. ⁴⁵ By targeting the pathophysiology of the disease, Voxelotor will reduce the stress and related finances of hospitalization due to anemia and organ damage.
This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients for whom other available treatments have failed	Voxelotor is the only late-stage drug to address the fundamental pathophysiologic mechanism of SCD, which directly results in multi-organ damage, the primary cause of SCD death. ^{46,47,48}
This intervention will have a significant impact on improving return to work and/or overall productivity.	SCD patients can expect to lose at minimum approximately \$700,000 of their lifetime income compared to a person without SCD based on shortened life expectancy alone. ⁴⁹ Voxelotor has the opportunity to alleviate this burden and allow patients and their caregivers to return to work.
Other important benefits or disadvantages that should have an important role in judgments of the value of this intervention.	Insufficient investment in disease research, understanding and disease registries have been critical barriers to development of new SCD therapies, due in part to economic and social inequality concerns, despite the implementation of a nationwide screening program and the FDA's encouragement of the development of new treatments. ^{50,51,52}

Table 1: Potential Other Benefits and Contextual Considerations (*continued*)⁵³

Potential Other Contextual Considerations	
This intervention is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life	Those living with SCD have a compromised quality of life and many suffer from multi-organ damage, which is the primary cause of death. Starting in their first year of life, 6% of children with SCD do not live to adulthood. ⁵⁴ With a diminished average life expectancy, ⁵⁵ those who survive childhood face a life with an unimaginable series of symptoms: these include pain and fatigue, and a multitude of complications that will keep them in and out of the hospital throughout their lives. ^{56,57} The availability of Voxelotor will aid in preventing anemia and reducing the primary cause of death due to oxygen-deprived organ damage.
This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness	SCD affects every single facet of a patient's and caregiver's life across their entire lifetime. Voxelotor has a strong potential to revolutionize care, alleviating patient, caregiver and broader family burden and allowing them to return to work and school.
This intervention is the first to offer any improvement for patients with this condition	Due to its narrow therapeutic window, required frequent monitoring, and concerns over potential infertility, patient uptake and adherence of hydroxyurea (HU) is suboptimal. Similarly, L-glutamine is indicated to reduce the acute complications of SCD, <i>i.e.</i> , VOC; however, it does not affect hemoglobin levels and has been used by very limited numbers of patients. Voxelotor's Phase III HOPE trial inhibits HbS polymerization, the very mechanism by which the disease occurs, add that it is fundamental to changing the course of disease.
There are additional contextual considerations that should have an important role in judgments of the value of this intervention.	The absence of longitudinal disease registries creates a significant barrier in understanding the full value of treatments. The last large SCD registry was the Cooperative Study of Sickle Cell Disease (CSSCD), and the data collection for this registry was from 1978 to 1988 and the last follow-ups were conducted in 1992, more than 25 years ago. ⁵⁸ The paucity of currently available longitudinal data will make it difficult, if not impossible, to understand the quality of patient care, health outcomes and utilization patterns for SCD. ^{59,60}

³⁹ PubMed. Search of articles related to Hemophilia, Cystic Fibrosis, Sickle Cell Disease. 2000-2018. [Accessed August 2019]

⁴⁰ CenterWatch. FDA Approved Drugs by Medical Condition. Link [Accessed August 2019]

⁴¹ *Op. Cit.*, Global Blood Therapeutics Societal Cost of Sickle Cell Disease, 2018, [Link](#)

⁴² *Op. Cit.*, ICER Sickle Cell Disease Draft Background and Scope p. 7, 2019. [Link](#)

⁴³ CDC. Sickle Cell Disease: Data and Statistics. [Link](#)

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⁵⁰ *Op. Cit.*, Bahr *et al.*, 2015. [Link](#)

⁵¹ *Op. Cit.*, HRSA, 2019. [Link](#)

⁵² *Op. Cit.*, FDA, 2019. [Link](#)

⁵³ *Op. Cit.*, ICER Sickle Cell Disease Draft Background and Scope p. 3, 2019. [Link](#)

⁵⁴ *Op. Cit.*, Quinn *et al.* 2010

⁵⁵ *Op. Cit.*, Paulukonis *et al.* 2016. [Link](#)

⁵⁶ *Op. Cit.*, Carden *et al.* 2019

⁵⁷ *Op. Cit.*, Brown *et al.* [Link](#)

⁵⁸ NIH. Cooperative Study of Sickle Cell Disease (CSSCD). 2014. [Link](#)

⁵⁹ American Society of Hematology website. ASH Sickle Cell Disease Initiative. *SCD Policy Priorities. Talking Points for Expanding SCD Efforts at CDC*. [Link](#) [Accessed August 2019]

⁶⁰ American Society of Hematology. *Report on Sickle Cell Disease Suggests that Current State of Treatment and Care is Inadequate and Improvements are Needed*. September 6, 2016. [Link](#)

Crizanlizumab and Voxelotor for Sickle Cell Disease:
Effectiveness and Value
Comments: Keith Quirolo, M.D.

- Page 2
 - There are not two, but four, currently approved treatments for sickle cell disease
 - Red Cell transfusion
 - For acute events
 - For prevention of complications
 - Hydroxyurea
 - To increase fetal hemoglobin, decrease leukocyte count, NO donor
 - For individuals who have any complication of sickle cell disease
 - Occasionally for children to prevent complications of sickle cell disease
 - Progenitor Cell Transplant
 - Currently from an HLA matched sibling
 - In study: other sources of progenitor cells
 - L-glutamine
 - Recently approved for sickle cell disease
 - Decrease vasoocclusive events
- Page 2
 - Voxelotor
 - Mechanism of action is to bind hemoglobin in the oxygenated state which prevents polymerization of sickle hemoglobin
 - Increased hemoglobin by decreasing hemolysis
 - An increase in hemoglobin was the primary endpoint of the Phase 3 study
 - About 25% of the hemoglobin appears to be fixed in the oxygenated state in the Phase 3 study
- Page 4
 - The population studied for both treatments was greater than 2 years old
 - Voxelotor: 12 to 65 years
 - Crizanlizumab: 16 to 65 years
 - Unlikely they will be approved for young children by the FDA
- Page 4
 - Markers for hemolysis and hemoglobin studied for Voxelotor
 - Hospitalizations and sickle cell crisis were studied for Crizanlizumab
 - Not sure need for blood transfusions was an outcome in either study
- Page 4
 - Not sure what “chronic infusions” refers to in the Subgroups box

- Page 5
 - Population
 - The lowest age of the population studied was 12 years
- Page 5
 - Red cell transfusions were an exclusion for both studies
 - Not sure there were any subject who required transfusions in either study
- Page 6
 - Key Outcomes
 - I would have to look at the Investigators Brochure but I am not sure all of these Outcomes were actually included in the studies
 - Pregnancy was an exclusion
 - Hearing and vision loss, not sure this was an outcome
- Page 6
 - Additional intermediate outcomes and surrogate outcomes
 - Hemoglobin and hemolysis were primary outcomes for Voxelotor only
 - Not sure red cell transfusion was an outcome measure
- Page 7
 - Age two and older...

Of some interest, in the Crizanlizumab study almost a third of the subjects did not finish the study.

Novartis appreciates the opportunity to participate in ICER's sickle cell disease (SCD) review. This document summarizes our comments on the draft scoping document. Novartis believes that incorporating the recommendations below will make the evaluation of crizanlizumab more thorough, accurate, and balanced. We are hopeful that this evaluation will support patient access to a much-needed treatment option in SCD, an area of high, urgent unmet need.

There are five key considerations for ICER's value assessment that we would like to highlight:

1. Endari (L-glutamine) is the most appropriate comparator for crizanlizumab.
2. Intermediate outcomes in the draft scoping document are not relevant to crizanlizumab.
3. VOCs are the hallmark of SCD. Measurements of disease severity should incorporate VOCs, with or without health care utilization, and also must account for complications and organ damage.
4. ICER should incorporate SCD-related contextual considerations that can be quantified.
5. The patient population should include individuals with SCD aged ≥ 16 years.

1. Endari (L-glutamine) is the most appropriate comparator for crizanlizumab.

ICER proposes to compare crizanlizumab to "usual care." While "usual care" has not been clearly defined, hydroxyurea (HU) treatment and blood transfusions are mentioned as components. However, hydroxyurea and transfusions are inappropriate comparators for a number of reasons, including:

Crizanlizumab will not replace hydroxyurea (HU) in clinical practice.

In clinical practice, crizanlizumab will be prescribed as an add-on therapy to HU in patients who are resistant/refractory to HU, or as monotherapy in patients who have failed, are intolerant to, or are contraindicated to HU. The MSH trial demonstrated that HU is an effective first line therapeutic option for patients with HbSS and HbS β^0 thalassemia. However, the safety and efficacy of HU has yet to be demonstrated in the other genotypes, including HbSC, which comprises ~30% of the US SCD population.¹ Further, many adults with SCD treated with HU continue to have pain crises.² Others refuse to take HU on the basis of concerns around toxicity, including risk of infertility.³

The SUSTAIN clinical trial reflected expected clinical practice as it enrolled patients of all genotypes with high unmet need for alternative treatment options; that is, patients who experienced 2–10 VOCs in the previous year. More than half of these patients were already receiving HU and continued during the trial.⁴ Those patients not receiving HU could be assumed to have declined HU therapy or be resistant, refractory, failed, or contraindicated to HU. The trial population was stratified by HU use.⁵ Thus, this data cannot be used to compare crizanlizumab to HU across the full SCD population due to this stratified clinical trial design. The SUSTAIN trial demonstrates that crizanlizumab is effective in both the presence and absence of HU treatment and regardless of genotype.⁵ Results of the trial support the use of crizanlizumab in combination with HU or as monotherapy in patients resistant, refractory, intolerant, or contraindicated to HU, not as a replacement for HU.

Transfusions are predominantly used for stroke prevention and are not indicated for VOC prevention.

Transfusions are not recommended for VOC prophylaxis by the National Heart, Lung, and Blood Institute of the National Institutes of Health.⁶ Instead, transfusions are primarily prescribed for primary or secondary stroke prevention in the adult SCD population.^{4,7-9} Further, there is no evidence that crizanlizumab will impact the use of transfusions for VOC prevention/reduction in clinical practice. Consequently, transfusions are not an appropriate comparator for crizanlizumab.

Endari is indicated in clinical practice for VOC reduction—making it the most appropriate comparator for crizanlizumab in this evaluation.

Endari was approved by the FDA for the reduction of acute complications of SCD including VOC.¹⁰ The Endari clinical trial enrolled a similar adult population as SUSTAIN, albeit with HbSS or HbS β^0 thalassemia only: patients with ≥ 2 VOCs in the previous year, stratified by HU treatment status. As with the SUSTAIN trial, these patients can be assumed to be resistant, refractory or contraindicated to HU, or

to have declined HU treatment.^{5,10} Endari was not included as a concomitant medication in the SUSTAIN clinical trial as it was not FDA-approved at the time.^{11,12} Given the similarities in trial design, a network meta-analysis is available that compares crizanlizumab and Endari.¹³ ICER may find it helpful to refer to the clinical review in the Endari drug approval package as well as the FDA ODAC report evaluating the merits of Endari and its own assessment.^{14,15}

If approved, crizanlizumab would provide physicians an alternative choice for patients who are eligible for Endari, and therefore Endari is the most appropriate comparator.

Additionally, Endari is not equivalent to the L-glutamine powders and capsules sold as over-the-counter supplements, which are not FDA approved or indicated for SCD treatment, and are not covered by Medicare or Medicaid.¹⁶⁻¹⁸ Endari is the only FDA approved pharmaceutical-grade formulation of L-glutamine that is indicated specifically for the treatment of SCD.¹⁰ Endari consists of a different isomer than the non-pharmaceutical L-glutamine supplements, which face little regulatory oversight.^{19,20} Furthermore, such supplements would be cumbersome to take: a 150lb adult would need to take 60 500mg supplement pills per day to achieve the recommended Endari dose,¹¹ ignoring purity and efficacy concerns.

2. The intermediate outcomes (hemolysis markers and hemoglobin levels) described in the draft-scoping document are not relevant to crizanlizumab.

SUSTAIN demonstrated that crizanlizumab effectively reduces VOCs, the primary outcome of interest. Crizanlizumab achieved this aim independently of a clinically significant impact on hemolysis markers or hemoglobin levels. These intermediate outcomes therefore appear to be irrelevant for predicting the clinical benefits of crizanlizumab in VOC reduction, and are not necessary to include in the model. Furthermore, to our knowledge, there is no evidence demonstrating that increased hemoglobin reduces VOCs. In addition, a clear relation between reduction in crisis frequency and increased HbF or F-cell levels has not been demonstrated.²¹

3. VOCs are the hallmark of SCD. Measurements of disease severity should incorporate VOCs, with or without health care utilization, and also must account for complications and organ damage.

VOCs are correlated with organ damage and mortality.

VOC pain is the result of tissue damage caused by vaso-occlusion associated ischemic/reperfusion injury and inflammatory consequences.²²⁻²⁴ Greater frequency of VOC is an indicator of SCD severity and leads to increased mortality, as increased contacts with the health care system increase risk of complications such as hospital-acquired infections.²⁵⁻³¹ VOCs are also correlated with an increased risk of multi-organ damage, although causality is currently unknown.²⁷⁻³¹

While *Table 1. Key Outcomes and Harms* in ICER's draft scoping document contains important outcomes, the model should recognize that SCD severity is multidimensional, comprising factors including age, VOCs, chronic pain, and organ damage.³²⁻³⁵ ICER should ensure its model accounts for the different levels of severity of SCD and at a minimum incorporates complications and end-organ damage (Table 1), below.

Table 1: SCD-related complications and end-organ damage^{36,37}

Acute complications	Chronic complications and end organ damage	
Acute chest syndrome	Avascular necrosis	Retinopathy
Acute kidney injury	Cerebrovascular disease	Sickle chronic lung disease
Cerebrovascular accidents	Erectile dysfunction	Sickled hepatopathy
Gall stones	Gall bladder disease	Pulmonary hypertension
Hepatic sequestration	Hearing loss	Thrombophilia
Priapism (re-occurrence)	Hypersplenism	

Pulmonary embolism/DVT	Leg ulcers
Retinal detachment/ hemorrhage	Microalbuminuria
Splenic sequestration (re-occurrence)	Thromboembolic diagnosis/ venous thromboembolism
Stroke	Renal failure

The value of VOC prevention should be measured broadly due to the significant proximal and distal impacts of VOCs on health related quality of life.

VOCs are the leading cause of hospitalization and emergency department use among SCD patients.³⁸ VOCs are more common in the real world than shown in the SUSTAIN clinical trial as the latter measured only VOCs with health care utilization. As described in the PISCES study, the ratio of VOC days with health care utilization (e.g., hospitalization, emergency department visit, physician visit) compared to those not associated with health care utilization was approximately 3.6 to 1.³⁹ Thus, ICER could incorporate this real-world data on non-utilization crisis days into their model by assuming crises with utilization will include a certain number of days without utilization. VOCs, whether associated with health care utilization or not, negatively impact sleep quality, psychosocial wellbeing, family interactions, productivity and activities of daily life.⁴⁰⁻⁴⁶

4. ICER should incorporate SCD-related contextual considerations that can be quantified.

We commend ICER for acknowledging and including the severe productivity impacts of SCD in a separate analysis. Additional contextual considerations have a major impact on patients, their caregivers, and society, and there are adequate data to include them in the model. These include:

- Caregivers experience enormous psychosocial and financial burden.^{47,48} The model should include reductions in caregiver quality of life and lost income due to caretaking.
- Work productivity is significantly impacted by SCD. Not only should ICER account for the fact that patient work hours and employment decrease,^{49,50} but also that SCD patients often receive disability payments and some are unable to pursue the same education as their peers.^{50,51} ICER should model how reductions in VOCs and disease severity affect patient quality of life via career satisfaction and educational achievement, as well as patient income and the probability of receipt of disability payments.

5. The population used in the model should only include people age 16 and older, the population studied in the SUSTAIN trial.

Closing remarks

Novartis would like to thank ICER for the opportunity to participate in the SCD review, and appreciates its consideration of our comments. SCD is a disease with a high, urgent, unmet need with limited effective treatment options. New and innovative therapies are of enormous value to the SCD community. Careful consideration of the unique challenges and contextual issues of developing a value-based model is central to conducting a relevant and informative value assessment. We look forward to partnering with ICER to facilitate an accurate and balanced assessment of crizanlizumab, based on rigorous science and the best available evidence.

Sincerely,

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Vanessa Kirk

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have lived a very painful life for 44 yrs living with Sickle Cell Anemia SS.

Please discuss any experience with blood transfusion:

I've had blood transfusions all my life and it still doesn't make it any better afterwards.

Please discuss any experience with hydroxyurea:

I hate this drug. It makes my hair fall and thin out due to the chemo that used in it.

Please discuss any experience with Endari (L-glutamine supplementation):

Haven't been taking it that long.

Sincerely,
Vanessa Kirk
vanessakirk3@aol.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Marissa Cors

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Diagnosed at birth. 5th generation SCD patient. Hospitalized every 18mos until I was 30yrs old. Bone infarction at 13yrs old spent 3mos in hospital. Rejected blood transfusion at 19yrs old was hospitalized for 2mos. At 24yrs old admitted into ICU for systematic organ failure as a result of rejected transfusion. No more transfusions. Globulin saved me. From 33-39 I was in outpatient care in a cancer ctr with numerous infections due to PICC and PORTS. NO MORE OF THOSE. At 41yrs old I started ENDARI and Medical Marijuana in combination with taking the lines out and I have been hospitalized twice this year. Despite all of these complications I finished university, held jobs and started various companies.

Please discuss any experience with blood transfusion:

Rejected blood transfusion at 19yrs old was hospitalized for 2mos. At 24yrs old admitted into ICU for systematic organ failure as a result of rejected transfusion. N

Please discuss any experience with hydroxyurea:

Been on it for over 20yrs...no problems...it made my hair straighter.

Please discuss any experience with Endari (L-glutamine supplementation):

It works! Since taking my quality of life has vastly improved. Ive been out of the hospital for the longest amount of time since I was 30yrs old..I'm now 42. I have way more energy, I sleep and focus better. My narcotic intake has decreased by 75%.

Sincerely,
Marissa Cors
Corsmarissa@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Dorian Archie

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed at birth spent my first birthday in the hospital and for the most part continued with about one major crisis a year (that required hospitalization) up until I became an adult. As an adult the day to strain I'd experience with sickle cell became worse sometime around my mid to late 20s that requires more frequent hospitalizations. I still deal with day to day pain but have been taking endari which I feel has improved the day to day pain!

Please discuss any experience with blood transfusion:

I have not had many blood transfusions in my life time but the hand full or so times that I have been transfused I experienced no complications. I have had two red cell exchanges which k also found to be beneficial at the time!

Please discuss any experience with hydroxyurea:

I have not taken hydroxyurea I don't know that it would benefit me as I have high fetal hemoglobin levels but my concerns are also in that it limits the contact I can have with another person as well as killing off sperm cells even if they do come back! I also have issue with it having been used as a form of chemo.

Please discuss any experience with Endari (L-glutamine supplementation):

I have been taking endari for several months now and have seen improvements in my day to day pain levels I had taken it as an Over the counter supplement prior in the past but not at the grade I am taking it at now.

Sincerely,
Dorian Archie
dorian_archie@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Reebe Jarrett

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My son was diagnosed at 6months of age, was placed on folic acid/penicillin until the age of 11, where we are at no Hematologist is available, he is 30 years old still on our health care. We have learned that eating healthy is the one of the key factors, Gatorade, plenty of water. Blood transfusions, no complications as of yet. Thanking God.

Please discuss any experience with blood transfusion:

We now have to wait a day or so for a blood type match because of the antibiotics, one day he will be able to get s match

Please discuss any experience with hydroxyurea:

He didn't care for it said he really did not feel a difference, also long term effects

Please discuss any experience with Endari (L-glutamine supplementation):

didn't know of this one?

What do you feel is important for ICER to know about the quality of life for SCD patients?

All that's available is pamphlets from the late 60s or early 70s and there just making you comfortable with blood transfusion and addictive pain meds need updated information and medicine to help maintain a better quality of lifecicer

What is important for ICER to know about the day-to-day challenges of SCD?

Yes, it takes a total on Everyone involved mentally, emotional Your life revolves are their daily challenges, I have a job and health care that's accommodating to my son needs Everyone doesn't have that choice

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Minimum 500.00

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
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I don't take vacation in case I need to support him while he's in the hospital or home recovering.

What other considerations are important for ICER to understand about SCD?

They just want the same opportunity/quality of life as everyone else to plan a trip or event and not have to worry if a crisis will come out of nowhere, that will support daily activities, i.e a job

Sincerely,
Reebe Jarrett
Rctaylor6190@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Alain Emmanuel

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed at 3 and I've been having crisis all my life. SC disease and I've had a hip replacement, blood transfusion, acute chest syndrome and many many other complication! Way too many to name but I'm no different than most of the patients with this illness!

Please discuss any experience with blood transfusion:

I had a couple when I nearly died and they saved my life! Blood is crucial for a lot of us!

Please discuss any experience with hydroxyurea:

Hydroxyurea helped me a Lot!

What other considerations are important for ICER to understand about SCD?

Consider how difficult it is living with this illness and how little you really know about the real struggles of living with this illness. Ask us what we would like versus trying to make decisions about our health for us!

Sincerely,
Alain Emmanuel
Emmanuelconcerts@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Yolanda Scott

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have (3)16 year old SC warriors. They have SC Beta thalassemia. Nothing relieves my children's pain. The remedy the hospital gives them is morphine and Toridol. At home they have morphine 15mg which does absolutely nothing for their pain.

Please discuss any experience with blood transfusion:

Only 1 of my children had to have a blood transfusion. He had no side effects except his blood pressure was high.

Please discuss any experience with hydroxyurea:

My child had a 1 day trial of hydrxyurea and he proceed to vomit for a month straight. Constantly

Please discuss any experience with Endari (L-glutamine supplementation):

Never had it. Never been informed about Endari.

What do you feel is important for ICER to know about the quality of life for SCD patients?

My children have absolutely no relief when they are in a crisis. My kids are limited in their daily activities. They have to be careful about over exerting themselves. They do not live a normal life.

What is important for ICER to know about the day-to-day challenges of SCD?

My kids are failing school because the schools are so uneducated on this disease. The teachers can care less about a 504 plan. Living day by day is a struggle when you're in pain.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

0.. I have medicaid

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: Missed work, transportation, buying food. Gas, bills get behind. If you don't work you can't make money to pay bills and other expenses.

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What other considerations are important for ICER to understand about SCD?

I'm tired of uneducated nurses and doctors with absolutely no compassion. It's a struggle to live with this disease. My kids are sometime depressed, moody and angry. People need to get educated especially teachers and school personnel.

Sincerely,
Yolanda Scott
Scott.yolanda@ymail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Kenneth Carson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I am 59 years old and live with Sickle Cell SC Disease, I have experienced Sickle Cell Pain Crisis since my earliest memories. I was diagnosed with Sickle SC Disease at age 8 and began standard treatments at that age which included IM and IV analgesic pain medication as well as Oral Opioids for use at home. (Oral Opioids began at age 4) I have experienced several complications from the disease including, loss of vision in my left eye from Sickle Cell Retinopathy, Avascular Necrosis, Bone Infarction, Chronic Kidney Disease, Chronic Liver Disease, Hepatitis C from Blood Transfusion, Chronic Pain Issues, Multiple Surgeries and Procedures, two near death experiences, as well as physical and emotional trauma suffered at the hands of Doctors and as a result of living with a disease that every day is killing some part of you!

Please discuss any experience with blood transfusion:

I contracted Hepatitis C from a Blood Transfusion, the procedure is difficult and painful due to the lack of accessible veins and the fact that I had my Portacath removed and cannot get a new one due to Subclavian Vein Occlusion on left and right side.

Please discuss any experience with hydroxyurea:

Hydroxyurea, changed my life and in no small part may be responsible for my still being alive. Before Hydroxyurea I was hospitalized for Sickle Cell Pain Crisis every six weeks and was suffering from many complications that I felt was going to lead to my death as it almost had in 2006 I began taking Hydroxyurea in 2006 and in the next year I experienced 3 pain crises and in the following years no more than two with most not requiring hospitalization!

Please discuss any experience with Endari (L-glutamine supplementation):

No experience

What do you feel is important for ICER to know about the quality of life for SCD patients?

Our quality of life is greatly affected our access to knowledgeable care and the comprehensive nature of that care! Too often Sickle Cell Patients are marginalized, treated with stereotypical idealism and inherent bias that ultimately leads to them avoiding going for help or simply not

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

receiving it in their greatest time of need, during the Vaso-Occlusive Crisis. This leads to many damaging side effects including death but more so the damage taking place in their bodies while they are lingering in an untreated state of on going Necrosis taking place throughout their bodies! Contributing to many long term damages as this process is repeated throughout their shortened adult lives!

What is important for ICER to know about the day-to-day challenges of SCD?

Sickle Cell Disease ultimately leads to Chronic Pain! Studies are just now being done on the affects of pain on the brain as well as the affects of Sickling in the brain! Despite the day to day challenge of living in pain, the process of pain and it's contribution to the Sickling process, untreated pain due to the effects of the opioid crisis; we now must consider the affects of Sickle Cell Disease on the Executive Function of the Brain which ultimately affects every aspect of a person's life including their ability to work and function in Society as well as their family's and day to day living.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Medication,Supplements, medical marijuana, (oil for pain) aproximatly \$1200.00 a year

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

If you count the loss of ones ability to maintain full time employment the cost is significant!

What other considerations are important for ICER to understand about SCD?

Please take into consideration that the greatest challenge to the Sickle Cell Patient is still our treatment in emergency rooms and hospitals where for reasons already mentioned our patients continue to suffer for life changing mistreatment that may only change if we no longer have to rely on them to treat us. This could happen with the development of a medication that "safely" reduces the number of Sickle Cell Crisis our patients experience and there by the number of visits to these facilities! Hydroxyurea works well but our patients don't trust and don't take like they should, (including myself) Endari I can't comment on personally but it seems they want to add it to Hydroxyurea? My hope is that one of these new drugs will provide a safe effective alternative to what we have now.

Sincerely,
Kenneth Carson
Kc.kencarson@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Trent Harville

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I don't know how to answer this short and sweet. I've had many treatments, drugs, diagnosis and complications. They include Demoral, Dilaudid, Morphine, and Ketamine for meds. Hydroxyurea and folic acid for vitamins I guess. Blood transfusions, exchange transfusions, gallbladder, kidney, and spleen surgeries, and more. Been diagnosed with high fevers, pneumonia, acute chest, and more.

Please discuss any experience with blood transfusion:

I ALWAYS ITCH for starters. I've had so many they rarely help now. My antibodies are numerous, and I've had breathing complications and severe pain during pain crisis. My hemoglobin has dropped as low as 3.8! They don't know how I survived it! But there are days I've walked around in the sixes and felt fine. Maybe even lower.

Please discuss any experience with hydroxyurea:

Hydroxyurea has worked well for many, but I am not one. Yet every where I go they want me to give it another go. They think higher doses or lower doses will do the trick, but all I've ever really felt it's done for me is make me feel extremely exhausted. I barely have energy as it is, but even in my 20's it affected me negatively. I couldn't take it when I played sports, such as when I was getting paid as a sparring partner.

Please discuss any experience with Endari (L-glutamine supplementation):

Because I just started it, I can't yet give an honest answer as to how it affects me.

What do you feel is important for ICER to know about the quality of life for SCD patients?

WE WANT TO BE HEARD! That matters so very much! Most of us aren't coming into the hospitals until the pain is at ridiculous levels bc we HATE feeling judged all the time. I don't know what these docs are being taught, but it seems compassion ain't part of the curriculum! When I'm talking to them, I'm so experienced with this I can practically read they thoughts. There are times I just look at them and completely stop talking, bc it's so obvious they not hearing me AT ALL! Most times when describing my pain I don't look at them at all, bc if I do and I see that apathetic or judge-y, doubtful look on they face it makes me instantly regret

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

coming in. It's hard bc they want you to give eye contact, speak clearly and be so detailed, all of which are incredibly hard when you in pain if for no other reason bc you don't care about none of that when you hurting. I've felt like I had to put on a show when I was younger bc if I said I'm a 8, 9 or 10 without crying or writhing in pain, they'd never believe me. It was obvious they didn't believe it by how long it would take to get me medication, or all the tests I'd be forced to take before getting anything for pain. Whereas when they believed me they say things like, "ok just hold on, I'm a get you something right now." Or they'd tell me what they were gonna give me AND ask me what I thought about the order before ordering it. Either that or they'd say, "what usually works for you?" followed by actually ordering exactly what I said or as close to it as they could comfortably go! It's the little subtle things they do that let you know they believe you, like getting closer to you and touching exactly where it hurts as gently as they can, followed by affirmations and nicknames and such. Like, "ok bud, tell me, does it hurt when I touch here? Gotcha, sorry if my hands cold. Ok my man, I know you hurting, but if you could point to where on your back is the pain localized, that would help me out tremendously." Ok, thank you man. I. Now you hurting, so im gonna go write up some orders for real quick, and then we can talk some more after the meds on board, hows that sound? Ok hang n there with me big guy. What usually works for you, u think? -or- You think 1 milligram of Dilaudid to start will help or we need to think bigger? How about a lil toradol to go with it? Ok good let's see what that does and if it doesn't do the trick, we may have to get a little more aggressive with it, right? Ok buddy, I'm sorry you got to go thru this but we gon get you right, alright?" I don't think doctors take into consideration what and how they say whatever it is they say nearly enough! Meaning pain control is vital, but care and concern goes a very long way! Patients from all walks of live with various conditions will agree, it helps with a speedy recovery when you feel like you're a part of your care, and it's personalized for you, rather than feeling like you're at the mercy of strangers. Regardless of if they are doctors, nurses, or techs, healthcare is intimate, so it helps tremendously when it feels like you're more to them than Patient A or the guy in Room such and such. I used to ask my nurses whenever they were talking about me to someone else while in my presence, to say my name versus referring to me as a room number. Such as, "Hey, can you help me start an IV on the patient in room 7 please." You're in room 7, I'm the patient, and I can hear you. So please just say "can you come help me start a IV on Mr Harville -or- Trent in 7 please?" I'm in the hospital a lot and I don't want to be. So help me out by making me feel like an individual, not a subject or science experiment.

What is important for ICER to know about the day-to-day challenges of SCD?

I've said so much already, so I'll just say it's a day to day challenge just feeling like a productive part of society versus a pain in people's side! Oh and we actually do feel pain every day. It's not just something we say! It sucks to know how many ppl still no very little to nothing about our disease, and they don't care.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Depends on what insurance I have as far as meds. But it's important to have a big bed to stretch out in, heating and ice packs, and over the counter meds like Ibuprofen and Tylenol.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Medicaid and SSI help, but are not enough to afford to live on my own. I've always had to split expenses with someone else. Low income housing takes a long time to get into. For instance, I've qualified for it since 2010, and I'm still on the waiting list. I've given up on trying to work as my health has gotten worse and worse, but I long to work again, and earn my own money. I just don't think it's possible. I couldn't even do Uber bc my knees are so bad I couldn't drive long without sickle cell flare ups. So needless to say, saving money has been all but impossible and I don't have enough extra to pay on a good life insurance policy! (Last time I tried that is!)

What other considerations are important for ICER to understand about SCD?

My biggest criticism of docs who treat me is they group us all as one. What I mean is they usually have a standard care package, or protocol for sickle cell patients that they hate to deviate from. I. HATE. THAT! We may share the same disease, but what works for your last patient, or even past three patients, won't necessarily work for me. Here's the thing, I don't care how long they been treating sickle cell patients! I've been dealing with this disease all my life! My opinion should mean way more to my care than it usually is. I LOVED my doctor in Georgia, Dr Sreekanth Reddy, and his sister Silpa, bc they always asked me WHAT I THOUGHT! They may not give me exactly what I asked for everytime, but they ALWAYS took it into consideration and most of the time, deviated from the hospitals normal protocol to cater to me specifically. In other words, my voice was heard, and NOTHING means more to a Sickler than knowing they are honestly being heard. They gave me real reasons for why they couldn't increase my IV Benadryl or Dilaudid, such as low blood pressure or something. They proved it bc if day that blood pressure stabilized but my pain or itching did not, they would give my request a shot. I'll be 40 next month, and I can't name one other doctor who took care of me as an inpatient AND outpatient, who actually made me feel like my voice was heard like they have! Everyone else just wants to go on about the "highs" and "licenses" and I always feel like they just see a drug addict. What I am is a person who knows what works for him! PERIOD! I don't want to get high, I want to get better fast and get back to my life! Plus, if the goal was to get high, it would be pointless because I don't feel the "high" anymore from any of that stuff. It would take an absurd amount of meds to do that to me.

Sincerely,
Trent Harville
trentharville@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Ameny Basil

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed with SCD in my childhood and in my 52 years of life with the disease i can say i have been fortunate enough to have had not so many hospital admissions and not many major crisis es however i have had very many minor crisis es, been transfused only in my 40s and had pneumonia once, most times i have felt tired weak and out of breath, never gained weight beyond 57 kg, had yellow eyes full time and leg ulcers once, i still have the use of all my limbs. i recently started with hydroxyurea as my hb levels keep dropping so fast, though i have not yet notice any of its effects. i have been on folic acid for most of my life and paracetamol as my major drug of pain.

Please discuss any experience with blood transfusion:

My experience with blood transfusion has been good, it has often boosted my energy levels and there has not been any bad or serious side effects

Please discuss any experience with hydroxyurea:

Recently started on hydroxyurea, not yet felt its impact on my life and i have not yet noticed any side effects.

*Please discuss any experience with Endari (L-glutamine supplementation):*N/A

What do you feel is important for ICER to know about the quality of life for SCD patients?

Life is hard when you are sick and cannot plan it and cannot know how you will be tomorrow, next week, month or year or when you are always weak, tired and many times in pain.

What is important for ICER to know about the day-to-day challenges of SCD?

Stigma and ignorance from the community

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Hard to estimate because almost every expense is either for wellbeing or treatment.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Cost of immunizations

What other considerations are important for ICER to understand about SCD?

The fact you have to take care of yourself and yet you have no strength to work and earn a decent income.

Sincerely,
Ameny Basil
basilameny@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Kristin Grace

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My life partner has SCD. This is the worst disease someone can have. With multiple transfusion every month, it's complicated. Sometimes he needs an exchange transfusion or ex-Jade when there is iron overload. SCD has impacted him in every way- can't exercise, horrible teeth problem, constant hospitalizations, depression, etc.

Please discuss any experience with blood transfusion:

Monthly blood transfusions. Sometimes it helps but sometimes not. Then there is the iron overload from all the blood that has to be treated.

Please discuss any experience with hydroxyurea:

Hydroxurea wasn't helpful for him.

Please discuss any experience with Endari (L-glutamine supplementation):

We just got this prescribed and plan to pick it up next week.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Stem cells

What do you feel is important for ICER to know about the quality of life for SCD patients?

The overall quality of life for SCD is diminished. It adds insult to injury when the medical professionals do not understand the disease. Education should be widely disseminated across ER/hospitals all over the country to ensure patients get what they need.

What is important for ICER to know about the day-to-day challenges of SCD?

Some days it's a challenge just to get out of bed.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: Can't have a job so we are a one income family.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What other considerations are important for ICER to understand about SCD? Educate the doctors.

Sincerely,
Kristin Grace
Gracedeafservices@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Jasmine Fields

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:
I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My 5 year old son had SCD and take hydroxyurea, amoxicillin, and a variety of natural supplements. He has had splenectomy and a few pain crisis and 1 hospitalization.

Please discuss any experience with blood transfusion:

We had blood transfusion when he had splenectomy surgery. He had no side effects. The only problem was they had a hard time finding his blood type.

Please discuss any experience with hydroxyurea:

We use it and it works when the dosage is right.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

There are tons of natural supplements used.

Please discuss any experience with Endari (L-glutamine supplementation):

We haven't used this

What do you feel is important for ICER to know about the quality of life for SCD patients?

Painful days does not make a quality life.

What is important for ICER to know about the day-to-day challenges of SCD?

The uncertainty of pain is very annoying and causes anxiety

How much do you spend (out-of-pocket) on SCD treatment or medical care each year?

About \$3k after insurance

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Transportation, parking, missed work, tolls, etc

Sincerely,

Jasmine Fields Jazzydharris@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Kamara Warner

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I am the mom of 3 daughters with sickle cell disease. 13, 9, and 4 years old, each was diagnosed at about 1 week in age. As the caregiver, my experience with sickle cell disease has been spending many sleepless nights trying to comfort my daughters. Some of those nights being at home and some being in the hospital. Ive had a daughter on a ventilator and missed many days of work causing me to lose a number of jobs.

Please discuss any experience with blood transfusion:

2 of my girls have had blood transfusions and they have worked well for them. One had acute chest and the other, her spleen was enlarged. The blood transfusion helped.

Please discuss any experience with hydroxyurea:

My 13 year old has been taking hydroxyurea for about 3 years. It has lessened the number of crisis she has but we do have the side effects with her hair coming out. The dosage was changed in the beginning bc it caused her blood pressure to be a little high, but since the change, it has been normal.

Please discuss any experience with Endari (L-glutamine supplementation): N/A

What do you feel is important for ICER to know about the quality of life for SCD patients?

As children, they are treated with such care and offered medicine anytime they come in with pain. I am afraid as my children become adults because I hear of so many stories where they are then denied medicines and treated poorly. The change in treatment seems cruel.

What is important for ICER to know about the day-to-day challenges of SCD?

Daily they are faced with the unknown as far as when a crisis will take place. I have seen my girls go from what appeared to be a completely normal day to pain on a scale of 10 in a matter of minutes.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Luckily insurance covers almost everything for my girls.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Missed work is the hardest concern as a parent. Due to so many hospitalizations, I have been fired from jobs due to attendance policies. When they are on the opioids, they are unable to go to school or daycare.

What other considerations are important for ICER to understand about SCD?

They appear to look normal, but their bodies are going through all kinds of things.

Sincerely,
Kamara Warner
Kamaralw@icloud.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Vonda Jackson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Son was diagnosed at birth in 2012 via newborn screen. He started penicillin and Hydroxyurea via the BABY HUG trial. First blood transfusion @ 6 months old due to fever. My son has had multiple blood transfusions since then. A majority of his care has been in Germany. He has had to have surgery on his left leg due to osteomyelitis. He has had problems with his stomach and has been diagnosed with having kidney stones as well that we have to continue to watch out for.

Please discuss any experience with blood transfusion:

He has had several blood transfusions without any complications.

Please discuss any experience with hydroxyurea:

So he has been on Hydroxyurea for awhile. He started experiencing abdominal pain and issue with constipation. I made changes in his diet but, that didn't work. So I stopped Hydroxyurea to see if that was the culprit. He was having frequent crisis so he's now back on the Hydroxyurea. His last crisis with hospital admission was Feb 2019.

Please discuss any experience with Endari (L-glutamine supplementation): We have never used this.

What do you feel is important for ICER to know about the quality of life for SCD patients?

My son is only 7 but, I have heard stories from other parents who have adult children that are being accused of pain seeking. I agree that sickle cell patients should be triaged just like a patient who is having an MI/heart attack. Sickle cell patients are also suffering lack of oxygen to wherever the crisis is occurring over time that tissue or bone dies.

What is important for ICER to know about the day-to-day challenges of SCD?

All facilities that provide childcare have stipulations about them being nut-free but, they allow kids who are not vaccinated to attend and will not disclose that information to parents. Our immune compromised children can be hospitalized just like a child who has consumed nuts. No

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

child should be allowed to attend a school, childcare, or any programs where other children attend without immunization.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

In 2018 7,000-10,000 dollars.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

I am currently a stay at home mom but, previously I worked a full time job. Thank God for FMLA or I would be without a job. More companies need to be understanding and provide that to their employees.

What other considerations are important for ICER to understand about SCD?

The awareness about sickle cell disease and sickle cell trait. I had no idea I had the trait until I was pregnant with my first son. We also need continued research to help find an affordable cure .

Sincerely,
Vonda Jackson
Vondajackson@hotmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Roxana Mateo

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Its been a roller coaster ride to say the least. I was diagnosis with SC when i was 3months old. I had my gallbladder removed and i have had acute chest. As a treatment i use folic acid and morphine for pain. Also drink a lot of organic natural juices

Please discuss any experience with blood transfusion:

With blood transfusions i have done well haven't had any side effects so far

Please discuss any experience with hydroxyurea:

I used it for about a year, i feel like it did not work for me. I felt extra dehydrated and sick with no energy. When i took it. Also it did not control my crisis. I was having them same amount of crisis.

Please discuss any experience with Endari (L-glutamine supplementation):

I have never used it

Sincerely,
Roxana Mateo
Roxana.mateo@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Velvet Brown Watts

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Our family began this journey 14 years ago, and it has been a rollercoaster. Though these years, we have experienced many complications and diagnoses, we have lost income and our family has been it hard. One of the many challenges has been education and maintaining grade level. We have an IEP and IHP. The other big issues has been challenges with kidneys, and lungs. We currently use O2. The emotional and mental is overwhelming, our whole family changed within 1 hour, 60 mins of receiving letter in the mail from the state health department. Families need medication that can help give the individual and their family a little bit of peace. It must be affordable and accessible. Life changing therapeutics should not be held hostage due to price or accessibility. Sometimes, I wonder are we asking, what is a life worth? Well, when it comes to our loved ones, my answer is, it's worth everything. My family deserves to have life, liberty, and the pursuit of happiness too.

Please discuss any experience with blood transfusion:

I have seen people be on chronic transfusions and others that have had transfusions. Some have died from iron overload. Many felt trap, as the quality of life was impeded. Some had blood reactions and had to be hospitalized. These times of issues can be mentality and emotionally draining.

Please discuss any experience with hydroxyurea:

As a family we need choices, other disease have choices. It is just for a community to have only 2 drugs. Is it just to have a drug that was not actually for the sickle cell population, but a repurposed drug. I want our family to have choices. Hydroxyurea does not work for everyone. When people tell you it does, that's a lie. It just like a person living with diabetes, not all medications work for everyone, just like there medication are a variety, so should medication for sickle cell be.

Please discuss any experience with Endari (L-glutamine supplementation):

Currently our request for Endari is being denied

What do you feel is important for ICER to know about the quality of life for SCD patients?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Patients are living longer but that does not equal improved quality of life. Many patients and caregivers are suffering. Caregivers health related quality of life is poor. Many, don't know how parents and caregivers are becoming physically sick, and the emotional trauma for both can produce PTSD. The lack of resources in our community is staggering. What others see as normal, creates hardship for us.

What is important for ICER to know about the day-to-day challenges of SCD?

You feel like you live with a certain sentence everyday. That you live in a medical jailhouse. You cannot plan as you would like and when you do, at a split second it can be changed.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I have not calculated this big, but we have expenses.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

I lost over 40,000 right away, and if I had to count how much over thr years with my degrees, the inability to work full time, and missing work, for both my husband and I would would say it is over 300,000

What other considerations are important for ICER to understand about SCD?

The sickle cell community has been marginalized sicker thr discovery of this disease. It is critical that families have access to treatment options. Families must have hope that they can live and not just exist. They should not be punished. They did ask for this. When companies work to give them hope families hope after decades of suffering, it would be an injustice not to have it accessible.

Sincerely,
Velvet Brown Watts
swithsicklecell@att.net

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Crystal Gunby
September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Missed diagnosed in 1982 diagnosed as having the trait And re diagnosed while pregnant in 2007 with SS sickle cell was treated with only flic acid pain meds and blood transfusion oxygen and fluids.. Later as joint started to decaded in 2010 from sickle cell lack of blood flow Doctor add a dia of avasclur norcorsic.. Which caused both shoulders replacements both hips replaced before the age of 34 not to mention many ER visit average 10 a year and doctors appointment average 2 a month. And crisis episode average 4 a month its bed and painful which lable me as no reliable at caused me to loss my job.. And it effectively caused stain to my family as my care giving husband job was not happy he had to leave work for me

Please discuss any experience with blood transfusion:

Its a chance you'll get sick with blood transfusion or antibodies fighting off blood cells and because doctors come to the conclusion that you need it when levels or a 7 and under even tho sickle cell blood levels or already low from 7 - 10 at a max that blood transfusion can actually be held off. not understanding that just because your blood levels are low dosent mean you always need blood transfusion. Ive took so many it caused iron overload which is another issue. And more drugs to add to the list

Please discuss any experience with hydroxyurea:

Its not for sickle cell community...It doesn't work it caused me stomach pain it caused levels to be out of wack. While taking it doctors wanted me to take 4 pills max 5 and when i tried it made me sick. Presser high weaknesses vomiting. It didn't take away crisis as mentioned it effects your fetal.. Meaning you cant have a healthy child or it makes you not want to have children. It can actually stop the circle of life and stop population of children just to be honest.

Please discuss any experience with Endari (L-glutamine supplementation):

Haven't had it Its too expensive even with insurance. With one income because i cant hold a job i cant afford it and I hear it helps alot for ones who take it. I wish i can afford it.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Blood exchange. More pain meds more meds just for SICKLE CELL patients. It's only 4 I believe, which are Hydra (dont know why that is) Endari, Blood Transfusion and Folic Acid for treatment out and one was actually for cancer patients.. Why would they have us taking kemo drug.. Sick cell should be reviewed more more trails.. The numbers dont add up on the population of people having SICKLE CELL in the documentation from research.

What do you feel is important for ICER to know about the quality of life for SCD patients?

If we had the right affordable treatment and drug that either can cure or mantain us from crisis symptoms we could work, live a healthy life, stay out of hospitals less or non at all. We can contribute to society in the way the government wants it to be..

What is important for ICER to know about the day-to-day challenges of SCD?

For me 1. Waking up without pain 2. Not being able to work when you want to. Always being laid off for a reason they companies make up like your postion is no longer available when you have 20 more people doing the same position. Or budget cuts. 3. Family is also effect by my conditions. As my husband experience because of me having to go to doctors hospitals or getting sick at work he is the only reliable person that can drive me or take care of my day to day of feeding or help groom because I'm in excousanating pain he has issue at he's job at the point where its in jeopardy. Meaning income gets lower my children ournt happy because we can provide like usual. 4. Depression accrues because you want to do for yourself and or work but you have a disease that you cant control it controls you and others that doesn't even have it.. And not able to do simple things is the worse feeling ever. 5. Taking pain meds every day is an issue. For now pain meds is all that we have for treatment to manage or crisis pain. And having to take it every day makes are bodie reli on it and when you try not to take it your body goes into shock.. I don't like then when your in a crisis and the meds yoy have at home dont work because your body has gotten use to the dose and you need to go to the hospital to control your symptoms but your automaticly put into a box as a drug seeker which brings me to 6. Dealing with prejudice against sickle cell patients is at a all time high in the medical world. I dont know why but it is.. That is why sickle cell patients dont like going.. Who want to be mistreated and misunderstood while dealing with pain and low blood count among other symptoms like acute chest pain. 7. Being proscribed treatment thar dosent work. Is a waste of time and money. Doctors are giving out meds for sickle cell patients to try and see if it helps happens. Which brings me to 8. Before finding a good doctors finding a great doctor that knows information about sickle cell is finding a needle in a hay stack but by the grace of god I found one. 9. Trying not to get infection or colds having a simple cold or UTI can trigger SCD crisis. 10. I've gained Vascular Norcrosis due to having SCD joints decade symptoms this on top of SCD is the most depressing painful an embarrassing experience ever Having to walk with a lemp having to get replacements before my time having to get checked out under metal detectors all the time having to go broke to get these procedures to have a better life. Because Sickle Cell breaks your body down before its time.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: All home necessities or being jeopardise do to medical appointments and medicine. Missed work one year I made only around 500-600 the whole year from being out of work not much I can do with that.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What other considerations are important for ICER to understand about SCD?

Sickle cell is a blood order disease that has a sickle shape. When the blood is shaped abnormal it blocks the flow of the blood from flowing in your blood stream correctly. This causes painful crisis symptoms. That appears without notice this leads up to other medical issues. We don't have the right treatment as of now that can control our symptoms. Due to lack of hours of work income cant afford high medicine monthly . multiple surgery and hospitals stay doctor appointment and more. Please consider doing more research and trails on this diseases. On more then one ethnic group all ethnic groups are impacted by this diseases. Please consider being knowledgeable about the disease This disease control's your life It control when or how many days or weeks you'll be in painful crisis or when they will appear. Any drugs that's on trail should be research for at least 2 years.

Sincerely,
Crystal Gunby
Crystalgunby@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Andrea Barnes

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have tried Hydroxyurea but it didn't work for me. I'm currently taking Endari but just started so I haven't seen any improvement as yet. My Dr recently put me on Steroids and it causes the red blood cells to increase in production but also more sickle cells thus resulting in more pain crisis. I'm interested in the stem cell research and am waiting for feedback on an available study

Please discuss any experience with blood transfusion:

I am on a monthly transfusion scheduled because I can't maintain my levels. Although it helps, my Dr was only doing 2 units of blood with a hemoglobin at 4-5 it doesn't really get me over the hump. Now I'm doing 3 units every 3 weeks. I'm afraid of iron overload because I can't afford the medication for it

Please discuss any experience with hydroxyurea:

I was on Hydroxyurea for 2 months and experience the worst 2 crisis in my life. I was working F/T and able to manage without any major issues. I was just taking folic acid and pain medication when needed. Ever since using Hydroxyurea, I've been extremely ill, unable to work or function. I've had to stop or adjust most activity. Even cooking and cleaning has become a major chore

Please discuss any experience with Endari (L-glutamine supplementation):

I started Endari 01/2019 and took for 2 months, when I went to refill the Rx I was charged \$700. I stopped taking it because I couldn't afford it. I was finally able to arrange payments and just received a 30 day supply. Hopefully I'll be able to get better results after 3-6 months

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Stem cell research and therapy

What do you feel is important for ICER to know about the quality of life for SCD patients? It depletes your quality of life. You're always worried, depressed, in pain, struggling with your health, finances, family

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What is important for ICER to know about the day-to-day challenges of SCD? There are some good days but mostly bad days. Everything becomes a struggle to start and complete. Trying to cook and clean takes hours because you're constantly having to stop for breaks. Outside activities are almost nonexistent

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?
\$500 - \$1500 + Medicare out of pocket (\$6000)

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance.

Transportation, cost for time spent on research, holistic therapy, healthy food and vitamins

What other considerations are important for ICER to understand about SCD?
We need better representation in Healthcare, Insurance, Research

Sincerely,
Andrea Barnes
Barnesa0314@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Georgene Glass

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My 4 yr old diagnosed with Sickle cell has been hospitalized at least 6 times and has recieved 8 transfusions since she was 18 months old. Due to my employer not have knowledge of SCD prior to my being employed with the company, even with FMLA ,I was let go due to frequent absences. Because we relocated from CA and I took a new job there was 2 weeks when I had no insurance and my child was sick and hospitalized with Acute Chest Syndrome and with no insurance at the time I am stuck with a 21k medical debt.

Please discuss any experience with blood transfusion:

My child has had 8 blood transfusions so far. With no side affects, they have helped to increase her hemoglobin level. She is now taking Endari as well.

Please discuss any experience with hydroxyurea:

We have not elected to use hydroxreua as of yet.

Please discuss any experience with Endari (L-glutamine supplementation):

She has statyed Endari as of the 2nd of Sept. We will go 60 to 90 days to see how it goes . There is increase in cost for buying fresh fruit and veggies to juice to put the supplement in

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Counseling/therapy should be included, meditation, yoga, massages,and health education should be apart of automatic treatments for persons with SCD.

What do you feel is important for ICER to know about the quality of life for SCD patients?

With the current treatment options it helps to improve the quality of life and the new treatments coming will also serve to improve the every day quality of life for people with SCD

What is important for ICER to know about the day-to-day challenges of SCD?

There are challenges associated with a person with SCD in school, work, or the community setting sometimes it is hard to move physically because of the pain, the different temperatures

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

can affect them. They can wake up feeling good and hours later not be able to completed their normal everyday task.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

It can vary from 2 to 5000

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Job loss, the only clinic for kids with SCD relocated 40 mins away so that could be a challenge, transportation, can not send to the average child care due to the large number of kids and potential for germs. Forced to find work from home to be able to also watch child and avoid potential risk.

What other considerations are important for ICER to understand about SCD?

There is no one size fits all, what works for once SCD patient may not work for another. People with SCD have as much pain and complications and people with Cancer or other chronic illnesses. The difference is there are many medical treatments for other diseases and only 2 in the last 30 years for SCD. Any company that is trying to work on curative therapies for SCD should be allowed to and as long as requirements for the effectiveness of the drug are met and a fair price is set those drugs should be considered.

Sincerely,
Georgene Glass
ginaglass@dreamsicklekids.org

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Amy Klug

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My son has sickle cell SS with concurrent alpha thalassemia. He was diagnosed at 8 months. He is now nine. He has been hospitalized for fever or pneumonia many times , parvo virus, twice for pain crisis, and he has had two operations for a hernia and for tonsillectomy (sleep apnea). He take folic acid, he took penicillin when he was younger. he took Endari fo a year, but the insurance has denied it this year.

Please discuss any experience with blood transfusion:

Once for parvo virus, and before each of his two surgeries. He has never had a problem.

Please discuss any experience with hydroxyurea:

None. My son has naturally high fhgb and isn't that sick. Also, he was adopted so we are extremely concerned about the possibility of it impacting future fertility.

Please discuss any experience with Endari (L-glutamine supplementation):

We tried it and liked. He was on it for one year , and now insurance is saying they will not pay for it because he is not on hydroxyurea.

What do you feel is important for ICER to know about the quality of life for SCD patients?

I want my son to lead as normal a life as possible. Where we live the weather is the biggest barrier to that. My son feels very isolated by sickle cell, and I know he thinks he prevents our family from doing many things because so much of the year we have to stay indoors. He loves to visit places where the temperature is nice and he can easily be outside (we have relatives in Southern California).

What is important for ICER to know about the day-to-day challenges of SCD?

The hardest things is the extra level of uncertainty built into everything. You just never know if you will be able to follow through on anything. We never know how my son will wake up feeling. He Is mostly healthy, I can't imagine how it is for families with kids who have a harder time. Also, the bills. My husband's employer has steadily been shifting the burden if healthcare

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

costs in to sick employees- our co-pays have gone up so much in the last 8 years. We try to keep this from the kids, but it is hard.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

We've hit our hospital max the last few years- that's \$1,400. Even when we hit that, we still have to pay \$150 for each ER visit- that really depends on how many fevers he gets in a year. Now that he is older it is probably \$300-600 a year, but when he was younger we would pay more to the ER. Plus specialist visits- \$80-160 in co-pays. Counseling is \$25 a session, which will run us about \$900 this year, folic acid is cheap- \$60 a year or so. The Endari is \$140 a month (about \$1700/year) , but we can't get it anymore. But that's just the SCD care- my son has regular medical needs too- dental issues, orthodontics, etc.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Cost of parking at hospital, cost of food at the hospital, my husband having to use up his sick days so he can't use them for when other people are sick, I don't feel like I can even get a job because I need to be available for my son's appointments and free to stay with him at the hospital (and this is the biggest hit to our finances, having to be a one income house hold- it prevents is from things like saving for our kids to go to college).

What other considerations are important for ICER to understand about SCD?

Even the kids who aren't "that sick" suffer so much.

Sincerely,
Amy Klug
amyk105@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Aisha Braimah

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Sickle cell is very painful I had strokes surgeries and many other complications

Please discuss any experience with blood transfusion:

I have gotten iron overloaded, my body sometimes rejects blood, and at times I feel itchy

Please discuss any experience with hydroxyurea:

I tried it 5 different times on different dosages it never changed how often I got sick and I dealt with many side effects such as hair loss and my white count dropping very low

What do you feel is important for ICER to know about the quality of life for SCD patients?

Sickle cell patients are often ignored and not looked at like drug seekers. Our complaints fall on death ears. Often when we have a new side effect that are undocumented, no one cares to look into the new medicine the patient is taking this causes patients to stay quiet not reporting new discoveries. This leads to suffering and at times death

What is important for ICER to know about the day-to-day challenges of SCD?

It is very hard to sometimes get out of bed let alone go to work. We miss out on family and friend activities and the weather deeply effects us. We can't just up and go when and where we want. If you use oxygen, you need to make sure you have enough for the activity and the trip home. We also need to make sure we are dressed for the weather and have plenty of fluids where ever we are going. Let's not forget that our pain medicine must be on hand in case it's needed.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I don't really have an amount that i spend it all depends on what i need at the time like a nwe heating pad, copays on meds, over the counter meds, and transportation to and from the doctor

Discuss any other financial burdens of SCD outside of medical costs that are not covered by

insurance: Transportation to and from appointments and hospital stays, food when in the hospital

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

and appointment days, snacks, different activities for when you are in the hospital. Sometimes we need to pay late fees on bills you couldn't pay while in the hospital or for missed events

What other considerations are important for ICER to understand about SCD?

Its important to understand that sickle cell patients get mistreated a lot we don't have many medical treatments and we are not all the same because we have the same sickness

Sincerely,
Aisha braimah
aishabrainmah28@gmail.com

Simone C. Eastman Uwan MD

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My name is Simone Uwan MD and I am a Medical Doctor currently unable to work because of sickle cell disease. I dream of going back to work every day. I was educated at the best schools; Columbia University for my undergraduate education in Stanford university school of medicine for my medical school training and residency. It was not a benign process trying to become a medical doctor with sickle cell disease, But when you have lived a life like I have, with people mistreating you and dismissing you because they cannot see your pain, you can become part of the problem or part of the solution. Unfortunately it came at a price because my diagnosis is triggered by stress, and medical school was no cakewalk. I just finished publishing my second book called All Rise: the Sickle Cell Community versus the Medical Establishment, in order to fully share what my life has been like with sickle cell disease. I talk about the 5 hip surgeries, and back surgery from Avascular Necrosis (something Crizanlizumab could have prevented), the countless blood transfusions(over 200 units of blood) that caused antibodies, reducing my chance for a BMT, the loss of function and lost wages, and what it is like getting your courage up to go to the ER for care, reluctant even when I'm acutely sick. I also cannot begin to share about family members who have been impacted by my health whether it was lost days of work, termination of employment because of me, or their depression from watching me struggle. I need both Voxelotor and Crizanlizumab to make it. We still have no actual drug that was made with us in mind(Endari is a concentration of the natural supplement L-glutamate and unfortunately did not work for me.) Please don't abort our promise of a better quality of life before it is born.

Please discuss any experience with blood transfusion:

I initially received exchange transfusions after having acute chest syndrome where a part of my lungs died. To prevent any worsening of my pulmonary function I was put on exchange transfusions for one year. Later I received blood transfusions as I needed it. Unfortunately this caused antibodies to blood products, and a very serious antibody reaction where my kidneys were involved. I now only get transfused around surgeries and I am pre-treated with large doses of steroids, Benadryl and Tylenol.

Please discuss any experience with hydroxyurea:

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Hydroxyurea is probably the one drug that I kept trying hoping that it would work because I saw it work in my friend. I was on it for a total of five years, until the palms of my hands, the soles of my feet and my tongue all became black from increasing the doses to make it work. It did not decrease the frequency of my admissions, which had become monthly, and so after all that time, even the doctor agreed that I was reaping all of the side effects and none of the benefits. He stopped the drug.

Please discuss any experience with Endari (L-glutamine supplementation):

I was eager to try ENDARI but had to quickly stop after starting. Each time I took it I had an acute onset of pain in my spine, my lower back and my extremities. It felt like I was taking a medication that gave me a sickle cell crisis. That was very disappointing for me. I was hoping it would work and lessen my disability, And maybe I could go back to work. Being a doctor was a very fulfilling profession.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

I have used natural supplements the past several years to greatly improve my pain, and it lessens my narcotic medication usage. However, insurance does not cover my healthy body bone and joint pack from youngevity, or my EvenFlo from Healing Blends, and I am unable to consistently pay for them. I don't go to salons, or get nails done or do any fun stuff. I save what little I have to buy disease modifying products for my body. However it is not enough to be consistent.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Right now there is very poor quality of life for most of us, and truthfully for my loved ones as well because they become permanent caregivers and of active roles in my life. They suffer from depression and PTSD from watching the things I have gone through even at the hands of the healthcare system. It would be so nice to be able to have a treatment that would lessen the pain and get us off of narcotic medications. Please do not make it hard to do so.

What is important for ICER to know about the day-to-day challenges of SCD?

My activities of daily living have been greatly compromised and they often need the use of a mobile chair chair in my home. I walk with a cane or use the chair on hard days. I don't go to the store for much. My husband does all of the grocery shopping and I buy pretty much everything else online.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

It's about \$4000 per year out of pocket, some years it was much higher.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

I can't even begin to tabulate: Uber everywhere because I cannot drive anymore, purchasing ready-made food when I am too sick to cook, purchasing my supplements, Paying for doctors co-pays and my share of every bill from every hospitalization, paying for co-pays on medications, the list is endless.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What other considerations are important for ICER to understand about SCD?

it is important to understand that it takes a village to raise both the child and the adult, and so many peoples lives are affected, not just the patient.

Sincerely,
Simone C. Eastman Uwan MD
simoneuwanmd@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Charly Richard

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed with sickle cell SS at birth and some of the complications I experience are pain crises on my legs and hips, fatigued, shortness of breath, avascular necrosis. Sometimes I have to get blood transfusion when my hemoglobin drop from my baseline. My past surgeries that I underwent were priapism(shunt), incision to remove my gallbladder, and ganglion cyst on my left wrist.

Please discuss any experience with blood transfusion:

When my hemoglobin drop, I have to go into the sickle cell Acute care center to get a blood transfusion. It usually takes time to find my blood because I have antibodies. I've never experienced any side affects.

Please discuss any experience with hydroxyurea:

I had took hydroxyurea twice and some of the affects I've experience were feeling groggy and lightheaded. So I stop taken it.

Please discuss any experience with Endari (L-glutamine supplementation):

I'm currently taken 5mg of Endari twice a day. It's actually a natural product that contains amino acid and it's very helpfully receiving results. I don't experience any side affects.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

I'm under narcotics and vitamins that I take daily when I'm at home, such as, oxycodone 10mg, morphine 30mg, ibuprofen 800mg, promethazine for nausea 25mg, folic acid, vitamin d.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Icer needs to know that a patient living with sickle Cell has a life that's abnormal. There's not a day that goes by where this disease has its role in affecting our lives.

What is important for ICER to know about the day-to-day challenges of SCD?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Some of the challenges for me that I've experience are change of weather, over-excretion, prolong sitting or standing that can cause pain and fatigue.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year? 0

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: Prescription medication that's round up to 6 bucks a month.

What other considerations are important for ICER to understand about SCD? Consider the fact that we get stigmatized at regular ER hospitals, consider that our bodies works 3 times more that a regular human being, consider that this nation doesn't make sickle cell as a top priority as cancer, leukemia, or Aids/HIV

Sincerely,
Charly Richard
charly.richard86@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Marie Jean Ipradieu

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Having sickle cell disease is worst illness to have in life, my family have suffered tremendously, my husband of 30 years left because he couldn't deal with it anymore, my children had be my care taker because of that they have to put their careers on hold. As a nurse, I had to stop working because of frequent hospitalization.

Please discuss any experience with blood transfusion:

I had a blood transfusion reaction which lend me in ICU in a coma on dialysis, my family thought they were going to loose.

Please discuss any experience with hydroxyurea:

I take hydroxyurea, it's been a great help at times but I experience upper epigastric pain and a lot of gas with which makes me unable to eat at times.

Please discuss any experience with Endari (L-glutamine supplementation):

I haven't been taking Endari but my Dr mentioned it to me.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

I would like to try the gene therapy but they said that I am too old for it because I am 58 years old.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Sickle cell disease can lead to a wide range of complications, but patients can live a good life if they live a healthy lifestyle and take your medications as prescribed.

What is important for ICER to know about the day-to-day challenges of SCD?

It's a daily challenge, sometimes you wake up in pain or you don't feel good, feeling weak or dizzy at least three out of seven days to me I don't wake up feeling myself.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
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A lot, It depends on the monthly prescribed medications, I buy my medications on a monthly basis.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

I don't work, I am on disability, the money I get can't even pay for my rent, and food and transportations if you don't have family to help out financially you are not going to live long life , my husband left me now some days I can't even eat how can I afford luxury like having cable for tv, a phone and certain other stuffs like medications and clothing ect.

What other considerations are important for ICER to understand about SCD?

It's a hard life for sickle cell patients without the help of family members

Sincerely,
Marie Jean Ipradieu
mjeanipradieu@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Hertz Nazaire

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Diagnosed at 6 months old with Sickle Cell Anemia (SS) now blind in one eye, while we are now living longer the quality of our lives are still extremely painful at times. I travel long distances for my care at least once per month to maintain my days crisis free.

Please discuss any experience with blood transfusion:

I am currently on apheresis treatment 6 bags of donated blood exchanged each month. The difficulties so far have been 3 infected ports having to be removed which is not an easy thing for most of us to survive due to our immune system being compromised. I would rather take a pill a day that had the same affect on my Sickle Cell Anemia to minimize painful crisis.

Please discuss any experience with hydroxyurea:

I did not have a good experience with Hydroxyurea it did not work for me at the time I took it. So it was not something I would have tried again. I see many patients that do well on it but we are all very different in how our bodies react, so we need more out there for Sickle Cell Disease outside of one drug. We need more options to pick from. Having something that works for you is important, not just try and squeeze in something that works a little with lots of side effects. Finding something that fits your body and proves helpful is extremely important and Sickle Cell only had that ONE choice for over 20 years.

Please discuss any experience with Endari (L-glutamine supplementation):

No experience with Endari yet but I have been discussing it with my doctor in recent months it might be a good fit for me in the near future.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

I would consider CRISPR gene editing. Sickle Cell is a killer and when it does not kill you, you suffer your whole life and this is no way for a sane person to live.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

The quality of life for most Sickle Cell Patients is a life of extreme suffering from pain and rejection of medical care. We are stigmatized as drug seekers because there is hardly any tools a care provider can offer us but pain killers. Life is painful and frustrating, and we have few choices in our options for care.

What is important for ICER to know about the day-to-day challenges of SCD?

Day to day challenge of living with Sickle Cell Disease is the same challenges we all face as human beings we try to survive by earning a living feeding ourselves and having a roof over our heads. The only issue is when we are sick we cannot earn a living so some of us like myself had to go on disability because I have gotten too sick to work. I am blind in one eye and the older I get the greater the chances that Sickle Cell will damage more of my organs. I've been homeless 3 times trying to make it while dealing with Sickle Cell. It is never an easy day to day for anyone affected with Sickle Cell Disease. We need more options not less.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I can't really say I am now on disability. The cost in my past was great enough to lose my apartment and lose many jobs, drop out of college. I can't really add up that much in what is cost me to survive so far.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

I can only make so much while on Disability SSI. The cost is great when you have Sickle Cell and having to be forced to be on disability to have healthcare or go into debt the rest of your life from medical bills. It is a difficult choice but your healthcare has to come first. So I have lost a lot of earning potential to just focus on keeping the pain at bay.

What other considerations are important for ICER to understand about SCD?

Sickle Cell Disease has very few options of treatment, pain killers or Hydroxyria. That is not really a wealth of choices. We need new drugs available that was created specifically for Sickle Cell Disease. It would make life much easier to pay for a few expensive drugs that really helps my Sickle Cell than to end up spending 2 weeks of every other month in the hospital. Those hospital bills really add up. 2 working medications as an options would be a better deal.

Sincerely,

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Candice Reed

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have sickle cell disease SC. I wasn't diagnosed until I was 18. However, growing up I experienced intense pain in my body and joints. Doctors told my mother it was growing pains when I was a child. After being diagnosed I didn't have the understanding about sickle cell as I do now. And at 18 during my first year of college I tried out for cross country track. I barely completed the first and my only mile of the race. Later that day I experienced terrible pain which sent me to the hospital. I had a sickle cell crisis and my gall bladder removed at that time. Over a 20 year span my pain crisis became more frequent. The initial treatment I received for sickle cell was with iron, folic acid, toradol and tramadol. Slowly over a 20 year time span my pain medications have been changed. It was a gradual process of stronger medications. Once the toradol and tramadol stopped working I was put on 5&10/325 mg hydrocodone. (Meaning when the 5 stopped working it was increased to 10) I was on it for approximately 8 years. When that stopped working I was on 5&10/325 mg Oxycodone for approximately 8 years. After 8 years of being on Oxycodone my pain was becoming more frequent as were my ER visits. I was then referred to a pain specialist who then prescribed hydromorphone 4mg for break through pain. For about 2 years this was helpful but as i grew older I believe my 12 hour shift job began to be too much for my body. I began having multiply ER visits, hospitalizations, uncontrolled pain, & an inability to complete a days work. One summer I had been hospitalized 4x with pneumonia. Other complications I've had are enlarged spleen, pleurisy of the lung, acute chest syndrome, avascular necrosis of the hips, emergency c-section, blood transfusions, and negligent & or abusive emergency room/hospital doctors and or staff. Which I truly believe is the worst of the complications. In the past couple of years my disease appears to have become more aggressive. As I mentioned before I could no longer complete a 12 hr shift anymore. Between the high pain and frequent hospital stays. I had to leave my career of 14 years and go on disability. One January I had a hospital stay of 18 days. The longest crisis I'll ever had. Oxycodone stopped managing my pain effectively and my quality of life was at a all time low. That when I was taken off oxycodone by my pain specialist and put on a 3 day Fentanly patch for 8 months. When I was put on the patch I could finally wake up in the morning without my whole body in pain. I could function without the up and down pain that came with oxycodone. Today I am being treated with iron, folic acid, a 7x day buprenorphine pain patch, 4mg hydromorphone for break through pain, and just recently Endari. When it rains or when i'm on my menstrual cycle is when my pain is at

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

its worse. My hospital visits average every 4 months. I am hopeful that Endari will lower the visits more. And grateful for my pain patch that helps manage my pain.

Please discuss any experience with blood transfusion:

I've had 3 or 4 in my life. They were helpful.

Please discuss any experience with hydroxyurea:

Hydroxyurea wasn't a option for me. I've been trying to conceive. And I think its more for SS patients not SC. Didn't want the side effects of hair loss or chance of cancer. As it is a cancer drug.

Please discuss any experience with Endari (L-glutamine supplementation):

Just started taking it in the last month. It is expensive. \$70 after insurance paid \$2,590. And because its new, insurance pushes back some on filling it.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Having SCD can be very isolating. When the pain is in control of your body its hard to be "happy". A crisis can happen so quickly. Without notice or triggers. Having a good support system is important. From family, doctors, and friends. We try everything before going into the ER. Increased fluids, rest, Rx, heating pad, ect.. because of how we get treated in emergency rooms. The ER doctors either dont believe you or say your not bad enough. Doctors that take one look at lab work and tell you "it's good" or nurses who manipulate Rx by watering them down with saline or into a I.V bag. Nurses that delay your medication because they don't believe your pain. This leads to increased anxiety at home for us so we delay going. We deal with alot of anixiety when we see people in our community dying. Imagine someone who looks like you and your age range. Dying often!! It makes you anxious as to when its your turn?? Especially when you just spoke encouraging words to them as they sat in a hospital bed. Also know that Pain medication allows us to function in our daily lives. Allows us to cook, interact with family, and manage our disease. Please don't look at us as drug seekers. Thank you for our input.

What is important for ICER to know about the day-to-day challenges of SCD?

fatigue and pain is a challenge when you're responsible for others, anxiety of when another crisis will happen.

How much do you spend (out-of-pocket) on SCD treatment or medical care each year? at least 1,500

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

I'm not sure. But I can say that I have unpaid medical bills from stays that I have no idea how to pay,

What other considerations are important for ICER to understand about SCD?

working together with a medical staff is important. Mental health is also, but also acknowledge our pain too.

Sincerely,

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Candice Reed
reed_candice@hotmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Ashley Jones

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Well my daughter has SC and it be very challenging at times. She is limited to outdoor activities as well as indoor activities. When a pain crisis hit, everything goes down hill with my daughter and myself also as a caregiver. You feel hopeless in the inside because you can not do anything to take the pain away. On the other hand, she does great academically

Please discuss any experience with blood transfusion:

My daughter never had a blood transfusion.

Please discuss any experience with hydroxyurea:

My daughter does not take hydroxyurea.

Please discuss any experience with Endari (L-glutamine supplementation): N/A

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Due to my daughter having pneumonia twice, she had to be put two inhalers.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Because this conditions does not get the recognition it should. So, I feel that every one/person should know about the cause/effect of having SCD.

What is important for ICER to know about the day-to-day challenges of SCD?

Because every SCD patient fight this condition in different ways on a day to day basis.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I spend maybe close to a \$1,000.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Since my daughter was born in 2013, I have missed work a lot and when you are on FMLA you do not get paid it just covers your position at the job. Also, gas costs because the hospital that is close to us that treats my daughter is two hours away. I have to pay copays for her medication, but I don't know.

What other considerations are important for ICER to understand about SCD?

Come up with a better way for parents who have to take or be off work numerous days to care for a SCD patient.

Sincerely,
Ashley Jones
ajones@halek12.org

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Funmilayo Ibidapo

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Was diagnose around 6 month of, no complications, folic acid and paludrine, and also vitamin Bcomplex

Please discuss any experience with blood transfusion:

The outcome has been good to me, never experienced any side effects, my concerns are fear of unknown like having diseases as HIV, syphyllis, hepatitis

Please discuss any experience with hydroxyurea: I have not taken it before

Please discuss any experience with Endari (L-glutamine supplementation):

I have not taken it before, I have never had contact with it before

What is important for ICER to know about the day-to-day challenges of SCD?

Sudden weakness experience by SCD patient

What other considerations are important for ICER to understand about SCD?

Make employers know that SCD patients can be absence from work due to our conditions

Sincerely,
Funmilayo Ibidapo,
Jamesbecky33@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Juvian Richards

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have genotype ss, diagnosed at birth. Diagnosed with AVN. Home remedies are used to treat my sickle cell.

Please discuss any experience with blood transfusion:

When I was receiving transfusions, my body was rejecting the blood, by developing antibodies. I developed mood changes.

Please discuss any experience with hydroxyurea: None

Please discuss any experience with Endari (L-glutamine supplementation):

None

What do you feel is important for ICER to know about the quality of life for SCD patients?

Our pain is real. We should not continue to be overlooked and under heard.

What is important for ICER to know about the day-to-day challenges of SCD?

Pain management is difficult. it's hard to maintain a normal life at times.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?
on the upside of 10,000

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

all of my medical treatment is out of pocket.

What other considerations are important for ICER to understand about SCD?

there is not enough education about sickle cell to medical professionals.

Sincerely,

Juvian Richards, rev.juvianrichards@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Joseph Jones

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Diagnosed at birth. Multiple hospital stays. Pain. Fever. Pneumonia. Blood transfusion. Narcotics. IV Fluids. Failed behind in school. Graduated late.

Please discuss any experience with blood transfusion:

First blood transfusion at age 18. Have had 3 since then. 2 on my last hospital stay. No major side effects from them for me so far. Concern was quality of blood. Very scary moment

Please discuss any experience with hydroxyurea:

Took hydroxyurea for about 3 months. I wasn't consistent so I stopped taking it.

Please discuss any experience with Endari (L-glutamine supplementation):

N/A

What do you feel is important for ICER to know about the quality of life for SCD patients?

We would like the chance to have the best quality of life as others with effective treatments.

What is important for ICER to know about the day-to-day challenges of SCD?

Pain. Discomfort. Not knowing from day to day how your going to feel. Well

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Well over \$8,000 with medications and hospital stays.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: Interferes with keeping a job. Finding a job with affordable medical coverage. To qualify for state Medicaid you cant have any income

What other considerations are important for ICER to understand about SCD?

Sickle Cell should be treated and acknowledged as all other critical illnesses. The time research and effort taken to search for a cure should be extended to SCD as well.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Sincerely,
Joseph Jones
jonesejoseph99@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Deirdra Jones

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My son (20) has SS. Prior to turning 13 years old, he had pneumonia a few times and plenty of hospital visits for high fevers. Unfortunately, at the age of 13 he began to have pain crisis that have become progressively worse over the years. I have AVN in his right hip, and had the ball of his replaced when he was 14. We are hoping that he is chosen for a gene therapy research study in our area soon. Before we head to his doctors office or the ER, he takes his prescribed meds and heat (heating pad and hot showers). If those don't help, then we go to the ER for something stronger.

Please discuss any experience with blood transfusion:

My son hasn't had many transfusions, but he does have antibodies that make it difficult for the hospitals to find blood for him. He has never had any adverse reactions from blood transfusions.

Please discuss any experience with hydroxyurea:

My son has been taking hydroxyurea since the age of 3. He has had no side effects and to my knowledge, there hasn't been any long term issues caused by the medication.

Please discuss any experience with Endari (L-glutamine supplementation):

He just started taking Endari and hasn't been consistent enough at this point for us to provide any information regarding how well it works or any side effects.

What is important for ICER to know about the day-to-day challenges of SCD?

Challenges include attending school/classes and securing and keeping employment. Dealing with ER doctors can be a pain when they refuse to provide meds that will actually help or send you home knowing you're clearly still in pain.

How much do you spend (out-of-pocket) on SCD treatment or medical care each year?

ER/Hospital bills are piling up. We have paid plenty on medicine because they are a necessity.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

My husband and I both have had miss work or leave work early to transport my son to appointment or the ER. My son has been unable to keep a job due to absenteeism.

Sincerely,
Deirdra Jones
deirdraljones@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Kamilah Bailey
September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have SCD type SS with Beta Thalassemia. I have experienced 8 years of continuous hospitalizations a total interruption of my professional and social life. Two lung collapses, pneumonia, and so many vaso-occlusive crisis that I cannot count them all. I have taken Exjade, Long acting morphine, dilaudid, hydration therapy, hydroxyurea and several natural remedies.

Please discuss any experience with blood transfusion:

I have had several blood transfusions and a few blood exchanges. Blood transfusions put me at risk for iron overload and build up of antibodies. If my blood count is not extremely low my doctors will not transfuse me, even if that means prolonging the vaso-occlusive crisis.

Please discuss any experience with hydroxyurea:

I tried taking hydroxyurea 3 times over the past 8 years with 2 different hemotologists. In all three attempts my liver panels increased to dangerous levels and both hemotologists took me off the medication.

Please discuss any experience with Endari (L-glutamine supplementation):

I have not yet been able to take Endari, although I would like to take it. My insurance informed me that it will cost me \$1200.00 per month for the medication and there is no way I can afford that.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Pain management and hydration therapy is effective but is not readily available for all patients. Crisper has the ability to cure sickle cell disease for many patients. Patients should have access to all new treatments available as sickle cell disease does not effect each patient in the same manner. It is going to take a wide variety of FDA approved medications to make the difference for patients nationwide.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Depression and anxiety is common in sickle cell patients. I believe if more treatment options were available that could help patients get back to work, decrease their dependence on family and caregivers and allow them to experience life on their own terms the quality of life for patients would increase exponentially. Access to consistent treatment, and FDA approved medications and treatments that could cure sickle cell disease is critical. We all want to put an end to the continued suffering.

What is important for ICER to know about the day-to-day challenges of SCD?

My experience is that I went to college and earned my MBA while working full time in a management position. That was 8 years ago. Now I struggle in so much pain daily to complete small tasks. I am constantly tired and in pain. My family is exhausted with being my caretaker. I am educated with 20 plus years of work experience, (like lots of SCD patients) and now I struggle to pay basic monthly bills and expenses. I want to work, I want to do so many things but sickle cell disease keeps getting in the way. The constant hospital visits have caused a strain on my friendships and relationships with the opposite sex. I am at war with my own body on a daily basis. Can you please contemplate being at war with your own body? It is inconceivable. I want to find a treatment that works for me, so I can get back to being me.

How much do you spend (out-of-pocket) on SCD treatment or medical care each year?

I have Medicare and GHPP to cover my medical expenses now. In the past my expenses have exceeded \$8,000 per year.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

The largest financial burden is my inability to work. I went from making almost \$54k per year to less than half of that. I cannot afford childcare which has been a challenge when receiving treatment. My daughters health insurance is \$300 per month which is difficult to manage. I have a vehicle that is paid off, but repairs and maintenance is very difficult for me. I still have to pay the mortgage, electric bill, gas, etc...every month and buy food.

What other considerations are important for ICER to understand about SCD?

New medication and treatment for sickle cell patients that is affordable can change our lives. With safe, effective treatment some sickle cell patients may be able to return to work, spend more time at home than in the hospital and see the light at the end of the tunnel when it comes to depression and anxiety. Having the ability to relieve the burden on our families and caregivers would improve our quality of life. Removing financial roadblocks to effective treatment and medication can help sickle cell disease patients experience self sufficiency and the satisfaction that comes from it.

Sincerely,
Kamilah Bailey
baileyko18@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Monica Pope

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My two sons have SS

Please discuss any experience with blood transfusion:

Thank God, no side effects.

Please discuss any experience with hydroxyurea:

No side effects noted. My sons have been using Hydroxyurea for 15 years.

Please discuss any experience with Endari (L-glutamine supplementation):

We started Endari, but they were not consistent in using this medication.

What do you feel is important for ICER to know about the quality of life for SCD patients?

I've seen SS patients in their 50-70's

What is important for ICER to know about the day-to-day challenges of SCD? Very challenging, just don't know from day to day if you're going to be in pain or not. That Alone affects your activities of daily living, such as employer school.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Maybe \$150-\$200

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Phone bill, rent, Groceries, fluids,. One of my sons is now unemployed.

What other considerations are important for ICER to understand about SCD?

Treat the person not the community. SCD Patients are treated as drug seekers because they don't appear to be in pain. Treat them as individuals based on their medical history.

Sincerely,

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Monica Pope
mpope2210@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Pamela Guillory

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I live with sickle cell SS, I'm 41yrs old, I also have AVN, blood clot issues, I'm on oxygen, have a enlarged heart and now deal with iron overload.

Please discuss any experience with blood transfusion:

I had alot of blood transfusion over the years and now developed iron over load.

Please discuss any experience with hydroxyurea:

I been on hydroxyurea for many years it helps but you also gotta know that what works for me don't mean it will work the same with the next person.

Please discuss any experience with Endari (L-glutamine supplementation):

I heard of it but I don't take it.

What do you feel is important for ICER to know about the quality of life for SCD patients?

We go through alot, mentally and physically and is in pain alot and we are not drug seekers we want to be treated equally as the next.

What is important for ICER to know about the day-to-day challenges of SCD?

Day to day is hard you are in pain alot and our energy levels are low, and we just want tonne treated like the next. We are not lazy we want fairness.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I have the medical card so I really don't spend alot

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

I'm on disability and don't cover alot as far as bills and if you work we don't get treated equal and we lose our jobs, we to have family to take care of. Financially it get impossible

Sincerely,

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Pamela Guillory
pamelaguillory77@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Torché

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Caregiver and person with sickle cell trait in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I take care of my dad and cook for him, run errands, and I have the traits. Sometimes I get random pains and fatigue.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Pain, fatigue, dizziness, irritability

What is important for ICER to know about the day-to-day challenges of SCD?

Pain, fatigue, dizziness, irritability

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

\$50

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Transportation, special diet and necessary foods beneficial to SC patients, and child care, missed work, life insurance.

What other considerations are important for ICER to understand about SCD?

That it's just as important and serious, and life altering like cancer, aids, etc.

Sincerely,

Torché

Torchychan@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Jody Johnson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed at the age one I last time I was in the hospital was in 94 I don't like going to the er cus they way they treat scr patients

Please discuss any experience with hydroxyurea:

I love hydroxyurea it really helps my blood account to rise

Please discuss any experience with Endari (L-glutamine supplementation):

Not familiar with it

What is important for ICER to know about the day-to-day challenges of SCD?

There is no way to describe our pain when we our in a major crisis it's frustrating for me every September I have major knee and leg pains

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

50-200

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Paying rent when I can't work gas getting to and from the doctors just to name a few

What other considerations are important for ICER to understand about SCD?

What works for me is staying hydrated and I take Dilaudid at church really helps me.
Just knowing that sickle cell matters just like cancer aids diabetes

Sincerely,

Jody Johnson

dbc_123@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Rowan Procter

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Close family friend/intern for advocacy foundation in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I've interned with Axis Advocacy for three years now and in that time I have assumed the role of caregiver several times.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Chronic pain is a near-constant and disrupts patients' abilities to do many basic tasks. In addition, because SCD is such a rare disease and patients exhibit few/no visible symptoms, doctors and nurses frequently dismiss patients as drug seekers and deny them the painkillers they need. SCD patients are often belittled and stigmatized, in and out of the hospital.

What is important for ICER to know about the day-to-day challenges of SCD?

Chronic pain disrupts a patient's ability to hold most jobs, or create art. The transition from inpatient to outpatient is very rocky, and is something most patients go through at least several times a year. There's a constant balance for SCD patients who must decide when their pain gets bad enough to go back to the hospital and when they can put it off. Patients who are in school have to work hard not to fall behind when they are hospitalized for weeks on end.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Missed work is obviously an issue, as is missed school. When a patient is in college or private school, days spent in the hospital can impact the education they're paying for.

What other considerations are important for ICER to understand about SCD?

SCD patients are frequently dismissed and silenced by medical institutions. There are few SCD specialists, and doctors who are not specialists often haven't heard of the disease at all.

Sincerely,
Rowan Procter
wilhelmgrimsby@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Shelly Taylor

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

My dad has been in pain so many times that he couldn't get up. There's a lot of things I do for him because of the disease. I've seen him during the bad times and the worse. It's hard to see him that way because I can't help him.

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Please discuss any experience with blood transfusion:

Please discuss any experience with hydroxyurea:

Please discuss any experience with Endari (L-glutamine supplementation):

What do you feel is important for ICER to know about the quality of life for SCD patients?

What is important for ICER to know about the day-to-day challenges of SCD?

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Sincerely,
Shelly Taylor
shellytaylor93@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Juanita Hampton

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I'm a mother of twins both born with SCD SS. The hospital was our second home as my daughters were always in the hospital or at clinic for blood transfusions. I've experienced and watched my girls deal with the pain on different levels, from acute chest syndrome, pneumonia, the spleen, and liver increase to the point where the staff knew us on a first name basis and became our extended family. Both my daughters have passed away, one on 2001 at the age of 9. She died two weeks prior to her 10th birthday. The second twin died 6 years later, 2007 at the age of 16. They lived happy lives through all of the pain.

Please discuss any experience with blood transfusion:

The iron overload and once one of the twins broke out in hives after receiving a blood exchange. One of my twins died from her liver increase due to the chronic blood transfusions.

Please discuss any experience with hydroxyurea:

My girls didn't have any side effects from hydroxyurea. It helps them from experiencing crisis more frequently from being hospitalized.

Please discuss any experience with Endari (L-glutamine supplementation):

N/A this drug wasn't out when my girls were alive.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Health care providers not providing the proper care for SCD patients. And this plays a great deal in the quality of life because SCD patients will suffer through the pain instead of going to the hospital for care. Also, they've experienced such improper care and the lack of information about it, more people are dying early and experiencing a lot of other problems, like mental health challenges in addition to the SCD, and some experience drug addiction too.

What is important for ICER to know about the day-to-day challenges of SCD?

Trying to live their best life with the illness without feeling isolated and handicapped. The patients fear dying and caretakers having to bury their patients, as a mother burying both my children at early ages.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Missing work to care for my daughters. I've lost my jobs and quit jobs because I needed to care for them being in the hospital or at home. The funeral costs is another financial challenge.

What other considerations are important for ICER to understand about SCD?

The challenges faced as a caretaker for those with SCD is challenging because we also must be advocates and medical providers too.

Sincerely,
Juanita Hampton
j_hampton7@icloud.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

La Shanna Mosley

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Our son Kaleb is six years old. He was diagnosed at 3 months old. He has had stomach issues since one year old due to sickle cell. He has been hospitalized six times for just that. He has had seven crisis his whole life. We started him on hydroxyurea last year. Since then no crisis to be admitted. Weight gain has gotten better. He has gallstones as of last year. But no issues bc of them. He is doing very well with going to school. We had to get him on a special bus. Because we don't have a car and the only other choice was for him to walk. Which is fine for a normal child but not for kaleb. He has had only four transfusions in his life. He takes folic acid allergy medicine and something for acid reflux. I am not sure if I have covered everything.

Please discuss any experience with blood transfusion:

He has had four in his life. They went fine. But as we learned more. We learned less is better. When it comes to this.

Please discuss any experience with hydroxyurea:

He is doing great on it so far. He has gained weight. He eats more. He hasn't had any crisis in a year now. We try not to give it to him with food. It makes him sick on his stomach. I'm concerned about long term usage on his body

Please discuss any experience with Endari (L-glutamine supplementation): N/A

What do you feel is important for ICER to know about the quality of life for SCD patients?

Treat them like they matter. Listen to them

What is important for ICER to know about the day-to-day challenges of SCD?

Even though they work hard to stay healthy. This disease takes over where and when it wants.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Nothing

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

We don't have a car so we have to pay gas fare each visit.

What other considerations are important for ICER to understand about SCD? Don't pump them full of drugs and their body is used to it. Then take it all away at transition time

Sincerely,
La Shanna Mosley
shawnmosley1977@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

David Hickman

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

As a personal caregiver to a Sickle Cell client my experience is limited to the medical care of and treatment of SC community in general. It is my experience that this community has been delegated to second level care status in comparison to other popular diseases, i.e Cystic Fibrosis, Breast cancer, Cancer, Aids. This includes, denial of pain medication while in ER and limited pain management after admission. Discriminatory adverse actions by hospital personnel, from Nurses to Physicians to ancillary medical services. This must cease in order for the SC population to receive care adequate enough to resolve the first line issue of Sickle Cell crisis: Pain and Dehydration. Resolving these issues upon immediate arrival at the ER will determine the SC patients experience and outcomes. My immediate focus as a first line caregiver is to resolve these issues first and it is my hope that this becomes the standard of care.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

STEM cell transplant has had positive reviews. I am hopeful that in time it becomes more accessible as a form of treatment

What do you feel is important for ICER to know about the quality of life for SCD patients?

Quality of life can be improved only with total community support. That includes, Medical, Social, Spiritual, Economic and Emotional support.

What is important for ICER to know about the day-to-day challenges of SCD?

It is important to understand that SC disease is debilitating on crisis days and that it affects the total social interactions with others.

Sincerely,
David Hickman
blkmnn59@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Pat Corley

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Provider in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Nurse Advocate for 35 years now retired working in community to improve care and develop resources.

Please discuss any experience with blood transfusion:

As nurse I worked for a comprehensive sickle cell center where I was involved intimately with persons with SCD and scheduled transfusion and monitored them post transfusion.

Please discuss any experience with hydroxyurea:

Witnessed positive effects and benefits persons with SCD experienced after being placed on hydroxyurea. Those effects included decrease in pain and crisis. Weight gain, increase in energy and stamina and an overall better outlook related to life.

Please discuss any experience with Endari (L-glutamine supplementation):

Patients on endari shared increased energy and less pain.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Stem cell transplant, bone marrow transplant alternative medicines

What do you feel is important for ICER to know about the quality of life for SCD patients?

Life for persons with SCD is challenging everyday. Coupled with Pain, disrespect and mistreatment in an environment of suspicion and accusation. They often suffer because of poor services and ignorance on the part of HC system. People with SCD have PTSD due to repeated injury. Hospitalizations and neglect. Poor care leads to depression, anger and hopelessness in this underserved population typically consisting of a disenfranchised population.

What is important for ICER to know about the day-to-day challenges of SCD?

Pain, anger, suffering, depression exists in the midst of this community.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Transportation, housing and childcare and meeting family needs

What other considerations are important for ICER to understand about SCD?

Stigmatization. Discrimination and poverty

Sincerely,
Pat Corley
patcorle@usc.edu

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Caleb Boaz

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed at one. I have had multiple complications and multiple hospital stays. I usually experience acute chest syndrome in the hospital. I have avn in most of my major joints. I have experience in using hydroxyurea and Endari for treating my sickle cell.

Please discuss any experience with blood transfusion:

I usually get exchange blood transfusions when I get hospitalized. It's one of the quickest ways to get me better. I was on chronic transfusions for 6 months after suffering a TIA when I was 17 years old.

Please discuss any experience with hydroxyurea:

I tried hydroxyurea on 2 separate occasions. Unfortunately the side effects were not more than the benefits for me. I have many friends that have highly benefited from using hydroxyurea and live fuller lives because of it.

Please discuss any experience with Endari (L-glutamine supplementation):

I am currently on Endari and it's an amazing drug with very few downsides. It has greatly reduced my daily fatigue, keeps my energy more consistent, gives me more room for taking corrective actions when I sense a crisis coming on.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Gene therapy, bone marrow transplant, and any new therapies being developed are very important because they can help transform the lives of many sickle cell patients.

What do you feel is important for ICER to know about the quality of life for SCD patients?

New drug therapies are essential to increasing the low quality of life for SCD patients.

What is important for ICER to know about the day-to-day challenges of SCD?

It's very difficult to live day to day with sickle cell.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

How much to you spend (out-of-pocket) on SCD treatment or medical care each year? 6000.00

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Sickle cell carries with it many burdens such as loss of tuition or jobs because of missing school or work due to a crisis. Emotional toll, transportation, food, for going to multiple doctors appointments, bringing advocates and family along. Paying for special accommodations such as pre check, buying flight insurance just in case you have to miss your flight. The time dealing with insurance companies to reimburse you. This is all time and money taken away from your already limited life. And it all compounds over years where our earning capacity significantly diminishes.

What other considerations are important for ICER to understand about SCD?

It needs all the drug therapies and interventions for us to live a healthy happy life.

Sincerely,
Caleb Boaz
calebboaz@icloud.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Juanita Gougis

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed at the age of 2 years old with sickle cell disease and now that I will be 31 this year. All I know is that it hurts, and noone knows about it so there is no help for me. You can't help what you don't know about. What people/ doctors don't know does hurt me.

Please discuss any experience with blood transfusion:

Blood transfusion. Thank God for it. But where do I get rid of all the iron I have collected over 30 years. When I have another pain crisis, should I keep pretending I have more room in my body to store all the Iron?

Please discuss any experience with hydroxyurea:

I have tried Hydroxyurea. I think it works. It was doing great until my body couldn't handle it anymore. But I think if it works for others then they shouldn't stop.

Please discuss any experience with Endari (L-glutamine supplementation):

I love Endari. It's too expensive tho. I don't know of any side effects.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Pain meds. Like it or not, they are a treatment. Hydroxyurea and Endari can prolong me staying out the hospital and improve my quality of life but we know at some point I will be back in the hospital. Noone wants to go there. It's scary. I just wish there was a cure. Anyways I'm grateful for Endari and Hydroxyurea (when it was working for me.)

What is important for ICER to know about the day-to-day challenges of SCD?

I don't think anyone with sickle cell disease can write in a paragraph how much pain they feel Physically and emotionally (and pain of our loved ones). And how we push through it. It's amazing that we keep fighting.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?
I dont know. Too much.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

the financial burden on me is hard but also on my family that has to take me to the hospital, and get what I need to live. We are all struggling. Heat, water, wash clothes because at 30 years old I pee on myself because I can't go to the bathroom when I'm in pain. Just alot

Sincerely,
Juanita Gougis
djisjonnita@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Wanda Gougis

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I am a family member that have observed first hand the agony and pain suffered by the Sickle Cell patient. I have notice that the amount of pain medication given is not enough to bring down the pain level. The patient do seem to get a little relief from blood transfusions. The patient have to constantly beg for pain medication and is usually given a Norco which I am told do NOT help bring down the pain level.

Please discuss any experience with blood transfusion:

When the blood count is low the blood transfusion help the patient feel better. The concern is iron over load as a result of too many blood transfusions.

Please discuss any experience with hydroxyurea:

Hydroxyurea taken in the pre teen years seemed to help lessen the number of crises. But, as a teenager the patient was no longer able to take the drug because it hurt the stomach and also had a burning sensation. Also, the patient noticed a slight hair loss.

Please discuss any experience with Endari (L-glutamine supplementation):

L-glutamine taken as directed definitely lower the number of pain crisis as well as lowering the number of trips to the hospital by not having an intolerable level of pain. There have been no side effects. (Price is a concern)

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Usual care should be oxygen, IV fluids, IV pain medication as needed, listening to the patient concerns, not lecturing patient about opioids every time pain medication is requested. Help patient to get in and out of bed when needed, and change sheets IMMEDIATELY if patient have accident! Do not put patient in a cold room, or keep patient in a cold room!

What do you feel is important for ICER to know about the quality of life for SCD patients?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

The quality of life for SCD patients would improve if then can get the treatment needed when they initially go to the emergency room and receive pain medication, without a lecture about being a drug seeker/addict and given IV fluids as well as oxygen.

What is important for ICER to know about the day-to-day challenges of SCD?

Patients with SCD cannot predict from day to day if they will have a pain crisis and have to miss work, school, or vacation/travel (with all arrangements paid and loss of money because they had to go to the hospital). This very situation happened again this year.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Bills, eg. car notes (car needed for work & hospital trips) and other obligations! Also, SSI/Medical cut off without explanation and finding out at the hospital.

What other considerations are important for ICER to understand about SCD?

During SCD crisis patient should be able to receive medication needed whether it is an antibiotic, pain medication and it should be affordable because most patients miss a lot of work because of dealing with the crisis at the hospital as well as dealing with the pain at home.

Sincerely,
Wanda Gougis
w.goigis@icloud.com

Casey Gibson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My older sister has sickle cell. It has been an incredibly disruptive and limiting influence in her life. Other than pain management and hospitalization, my sister has taken endari and L-glutamine. She also can no longer receive blood transfusions due to a complication earlier in her life.

Please discuss any experience with blood transfusion:

My sister can no longer receive blood transfusions due to a poorly matched one in her twenties. This very well could have killed her.

Please discuss any experience with hydroxyurea:

From what I have been able to tell, it has been helpful.

Please discuss any experience with Endari (L-glutamine supplementation):

This has definitely helped with energy and fewer hospitalizations.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Pain medication Oxygen Antibiotics Social services Therapy

What do you feel is important for ICER to know about the quality of life for SCD patients?

Sickle cell patients have much less freedom than most people, as they have to take their disease into account when planning trips, finding work, and even just being with friends.

What is important for ICER to know about the day-to-day challenges of SCD?

Plans are often disrupted and have to be changed. The disease is unpredictable and makes life unpredictable.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Due to the unpredictability keeping up a job can get progressively more difficult.

Sincerely,
Casey Gibson
Casey.gibson@me.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

O. I. Oye

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

2010 - year of birth, male 2011 - diagnosis of SCD beta thalassemia, crises and infection routine drugs: penicillin and folic acid 2012 - infections, crises and several hospitalisations. Morphine for pain treatment in the hospital. Paracetamol and ibuprofen for home pain management. 2013 - 2015 - Routine drugs, crises, hospitalisations. Research on Wellness and Wellness regimen started. Home - pressed veggie-fruit juices including ginger, garlic, turmeric and redbeets, 250ml given 2x daily, or more during crises. Stomach pain issues daily during and after breakfast. 2016 - No hospital admission, Evenflo supplement discovered and given in addition to wellness regime. Milk products stopped due to suspected lactose intolerance. 2017 - One hospital admission lasting 10 days, probiotics and natural antibiotics (oregano oil, olive leaf, echinacea, elderberry, vit. C 1000mg) discovered and given when needed in addition to wellness regimen. 2018-2019 - No hospitalisations, several home-managed crises, Evenflo stopped for a while, replaced with Prickly Ash bark, chlorophyll, B12, magnesium and Zinc in addition to wellness regimen. High TCD levels in March 2019, blood transfusion in September 2019 rejected subject to a repeated scan in October 2019. Wellness regimen continues.

Please discuss any experience with blood transfusion: No transfusion

Please discuss any experience with hydroxyurea: Used for about 2 months only due to reactions.

Please discuss any experience with Endari (L-glutamine supplementation): Not applicable.

What do you feel is important for ICER to know about the quality of life for SCD patients?
Difficult

What is important for ICER to know about the day-to-day challenges of SCD? Energy - sapping

How much do you spend (out-of-pocket) on SCD treatment or medical care each year? I don't know, but I spend a lot of time on research and a lot of money on supplements.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

I lose some freelance job opportunities due to the disease and or crises.

What other considerations are important for ICER to understand about SCD?

Food and herbs as medicine should not overlooked or ignored.

Sincerely,

O. I. Oye

Dun2011emerge@hotmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Derana Mathews

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed at the age of 4 years old and there wasn't much information out there about SCD. I had a fairly normal childhood with a bone infection at the age of 5 years old. The infection required surgery. Then I was fine until college. The stress of college was a lot for me and I had a lot of hospitalizations due to it. This was a huge stress and struggle for my family too. I have dealt with many different doctors who do not know about or understand what Sickle Cell Disease is or how to care for us as patients. I experienced a lot of discrimination from health care professionals due to the high levels of drugs that I take. I had to get a core decompression on both of my hips and one of my knees due to having AVN. I have had many port-a-catheters placed due to the difficulty of trying to find a vein. I have also had Infections, gallbladder removal, pic-lines, a non functioning spleen, steroid induced diabetes, and a lot of blood transfusions to the point of now having antibodies and needing treatment before getting a blood transfusion. Treatments I have used are: hydroxyurea, hydration therapy, blood transfusions, Endari (L-glutamine) , Procrit, Lovastatin, and Jadenu

Please discuss any experience with blood transfusion:

My experience with blood transfusions is that lately I have been needing them every 3 - 5 months due to the way my blood level drops. I have antibodies and when given blood it only works with a special IVIG treatment so that my body can accept the blood. Instead of giving me energy right away like it used to, it makes me feel tired and I have to wait a little bit before I feel better. This is a good treatment, but due to antibodies and side effects it can be dangerous for some patients.

Please discuss any experience with hydroxyurea:

Hydroxyurea is a good treatment. It helps raise my fetal hemoglobin so that I can live a normal life. It does increase my appetite and it made me gain weight. It has also thinned out my hair on the sides. Those are the only side effects that I have had.

Please discuss any experience with Endari (L-glutamine supplementation):

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Endari has helped me with having more energy and being able to do more for a longer period of time than before. The only side effect is that if I drink it too fast or take it with food I will have some nausea, but it goes away fairly quickly.

What do you feel is important for ICER to know about the quality of life for SCD patients?

For many patients quality of life is not that great. We all try to make the best of our situation. Unfortunately the frequent hospitalizations, trips to the doctors office and constant testing is very hard. Not to mention that we are what the medical community doesn't treat us very well and we all have to fight for proper health care!

What is important for ICER to know about the day-to-day challenges of SCD?

Many patients deal with depression on a daily basis and don't even recognize it due to the stigma that it carries. We hope that we can get through the day without having a bad crisis. There are people who count on us and we don't want to let them down. We battle being so tired on a daily basis and being in a lot of pain. We are just trying to be positive and live as best we can.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I am not sure what out of pocket expenses I have.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

I am unable to work, and I was unable to finish college due to Sickle Cell. I only make about \$12,000 a year on disability. My doctors and hospital I go to are an hour away so I have to pay about \$20.00 at least for gas every time I go there. Counting meeting for the year it is in excess of about \$300.00. My mom was laid off of work in my college years right before I was to have hip surgery, so that was a loss of about \$70,000.00 dollars for about 7-10 years. I was in debt due to my schooling for about \$25,000.00 I had to get a medical release for the funds. Not to mention the future earnings that I could have made if I was able to finish my schooling.

What other considerations are important for ICER to understand about SCD?

I believe that I stated everything that I can think of. Sickle Cell Disease is a very serious disease that needs to be talked about way more than it is. There are so many people suffering from this disease and education is the key. We need a health care system that will treat us all equally and fairly. There are too many people who have died due to the patient not wanting to go to the ER to be treated like crap, stereotyped, and discriminated against. Something needs to be done for all of us SCD patients.

Sincerely,
Derana Mathews
deranamathews@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Nephritina frierson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease: I was diagnosed at three because I was always sick, had my spleen removed, gall bladder removed, liver bio, P.E which in on blood thinners for the rest of my life and hundreds of hospitalizations for VOC

Please discuss any experience with blood transfusion: Well when I was on hydroxurea I was in the hospital weekly for one thing or another and they use to treat me with book transfusions 1but after a while my blood was getting harder and harder to match, so I eventually told them no more.

Please discuss any experience with hydroxyurea: Hydroxyurea had the opposite effect on my body instead of making me better I was sicker on it then off.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.
I learned that weed has the best effect on my anguish and pain and I think that it should be given more consideration

What is important for ICER to know about the day-to-day challenges of SCD?
It's hard to do everyday things when your body it fighting you and not everyone understands, even in the hospital it's challenging with the attitudes toward us.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year? 200- 500\$

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: Transportation, missed work, marijuana

What other considerations are important for ICER to understand about SCD?
The awareness of people unAffected by it, everyone knows what cAncer is but not sickle cell disease

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Sincerely,
Nephritina Frierson
Nepfrier112287@outlook.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Jordan Wright

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Type SS, 3-4 major crises/year, continued pain. Previously used hydroxyurea, horrid side effects, currently on Endari and its working great.

Please discuss any experience with blood transfusion:

No negative issues with transfusion. They usually help as soon as I receive them.

Please discuss any experience with hydroxyurea:

This did not work for me. Still had crises and painful episodes plus developed stomach issues

Please discuss any experience with Endari (L-glutamine supplementation):

This is my 4th month on sensational and it has been great.

What do you feel is important for ICER to know about the quality of life for SCD patients?

This is my 4th month on sensational and it has been great.

What is important for ICER to know about the day-to-day challenges of SCD?

Daily pain, loss of jobs for me due to the rigors of work and constantly having to take sick days.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year? A lot!!!

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Loss of work, difficulties obtaining SSI

Sincerely,
Jordan Wright
Reneewrightx@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Jessica Gougis

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My older sister is an adult with sickle cell disease. I act as an advocate when she is hospitalized. Her sickle cell causes complications like pain in her back, legs, chest, and arms. During her crises she is treated with pain medications such as dilaudid and norco and other treatments such as fluids, blood transfusions, oxygen, and antibiotics.

Please discuss any experience with Endari (L-glutamine supplementation):

Using Endari L-glutamine has helped reduce the number of times my sister has been hospitalized, as well as the severity of her crises when she does feel like she will experience an episode. She has not stated that she experiences side effects from L-glutamine. One concern I have about this treatment is the limited extent to which it is said to work: While the L-glutamine she takes has been established to help sickled blood cells in her body recover some of their lost oxygen, its effectiveness at preventing the blood vessel blockages created by sickled cells has not been made as clear.

What do you feel is important for ICER to know about the quality of life for SCD patients?

In addition to the large amounts of physical suffering caused by pain crises, SC episodes also interrupt the lives of SCD patients like my sister by bringing on countless trials, obstacles, and hassles they would otherwise not have to deal with. SCD patients are frequently forced to make extensive provisions for dealing with SC outside of hospital visits in order to protect their jobs, family life, finances, and daily activities from being overshadowed by SC or devastated by negative consequences that occur. People with SCD learn to manage their pain and go on with their lives, but they often have to do so overcompensating for their disease.

What is important for ICER to know about the day-to-day challenges of SCD?

Many people with SC struggle financially with getting medications that directly treat the SC blood disorder. Often they find themselves unable to or hard-pressed to pay for these medications, and the number of medications directly modifying SCD (which SC patients are able to purchase) are limited at this time.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

On average my sister misses about 7 days of work when she ends up going to the hospital. Earlier this year, in April 2019, she spent a month in the hospital. Her income suffered severely during this time. In the end, she was responsible for paying for a greater number of expenses with less income.

What other considerations are important for ICER to understand about SCD?

SCD patients have many struggles to deal with that can ultimately be reduced greatly by the continued development of and research into medications that help prevent their crises. The more options they have for medications, the more opportunity they will have to better manage their disease and cut back on the string of physical and situational complications that are likely to follow their pain crises.

Sincerely,
Jessica Gougis
jessica.gougis@ymail.com

Wanda J Williams

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I am a 70 year old woman living with Sickle Cell Disease (SC). I wasn't diagnosed until I was 19 years old after the birth of my son. My OBgyn, Dr. Lampley told me how concerned he was about my health during my pregnancy after my son was born. He said I could have had a stroke or died suddenly. Growing up I was sick every month at least 5 to 7 days experiencing severe pain in my legs, arms or abdominal area. My grandmother took care of me while my mother was working. She would rub whatever area of my body that was hurting and apply warm compresses. In my late twenties I had a core biopsy of my left hip as a result of aseptic necrosis a few years later I had shoulder surgery because of aseptic necrosis. Over the years I had acute chest syndrome on several occasions. I would have hospitalizations at least once every 3 months into my late fifties. I developed an embolism on my lungs on two separate occasions. It became necessary for me to have a blood transfusion every 4 weeks for a year. The transfusions had to be administered through my femoral vein because the veins in my arms would often collapse. I have had to have laser eye surgery because of having SC disease. In my sixties it was necessary to have my gallbladder removed. This lead to scar tissue creating lower bowel obstructions periodically resulting in hospitalizations.

Please discuss any experience with blood transfusion:

It is necessary for me to have a blood transfusion prior to any surgery for me to have a successful recovery. I have had an embolism on my lungs on two separate occasions. Each time I had to have blood transfusions every 4 weeks for a year. Some of the complications were my blood pressure dropping dangerously low, upon standing after the transfusion I would start bleeding from the femoral vein where they had to put in the Quentin catheter to access the vein. The veins in my arms were not strong enough to handle a transfusion.

Please discuss any experience with hydroxyurea:

I have used Hydroxurea for the past 10 years and not had a pain crisis. The side effects have been loss of hair on my legs, arms, discoloration of my finger nails and toenails. I must have labwork every 6 weeks without fail.

Please discuss any experience with Endari (L-glutamine supplementation): No experience

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Voxelotor I feel has great potential to help us living with sickle cell. It has the potential to stop the cells from sickling increase the oxygen. The side effects are minimal and it's a pill taken once daily. It has fewer dangerous side effects than Hydroxurea.

What do you feel is important for ICER to know about the quality of life for SCD patients? A with sickle cell can spend blots of time in the hospital as result of pain an organ damage. The loss of tme from work can make it difficult to keep a job. Because of my condition I can't get life insurance. It is very difficult to find physicians that really know about sickle cell. Insurance companies make it difficult if not impossible to be seen at comprehensive sickle cell centers where there is a wealth of knowledge.

What is important for ICER to know about the day-to-day challenges of SCD?

Finding comprehensive care for sickle cell is a real job. Not knowing when you will have axpain crisis can have you feel like you are living on pins an needles. A pain crisis can happen when you are doing all the right things.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I spend \$2,110 seeing an Osteopath for alternative treatment. My insurance cost are\$7,200 annually.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: My transportation cost are about \$400 a year.

What other considerations are important for ICER to understand about SCD?

It is real important for sickle cell patients to have comprehensive care. For pharmaceutical companies to invest in research for treatment and cures for sickle cell because this is an illness that has been neglected. The drugs that Global Blood Therapeutics and Novartis are bringing to market would make a real difference for people living with sickle cell.

Sincerely,
Wanda J Williams
wandaw10@sbcglobal.net

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Lisa Gougis

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have several family members with scd. The family member that is nearest to me is currently in a crisis. I watched her in excruciating pain only to get small dose of dilaudid via pca pump, and schedule toradol, she can also have norco po for breakthrough pain. Her pain was mainly in shoulders radiating to the elbow. Edema noted to elbows. The dilaudid makes her itch so they would give her benadryl. which contributes to drowsy with the oca pump med. With that being said they initially didn't want to increase her dilaudid, but the eventually did. My question is would atarx help with itching instead of benadryl.

Please discuss any experience with blood transfusion:

Her blood count dropped to 4.5. She was then transfused with one unit of prbc. The next day there was a slight improvement in her pain level and she actually looked and sounded better. I also have noticed clients having and adverse reactions to blood.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Scd patients can have a good outcome if medication is available at a reasonable cost. One other thing that I noticed is once a scd patients becomes an adult treatment is harder for them. Some physicians tends to think they are drug seekers so they don't get the care they deserve and that's with or without insurance, though without is worse.

What is important for ICER to know about the day-to-day challenges of SCD?

As for day to day challenges , they never know when they will have a crisis . My niece is an RN and it affects her job, occasionally she has to work sick so that she would not miss too much work.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Any days missed at this point is non paid for she has used all of her paid time off.

Sincerely,

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Lisa Gougis
lisalavalais@bellsouth.net

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Tristan Lee

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed w/ Sickle Cell Disease SS at the age of 6 month's. At that time I was the second person in my entire city to be found who had the disease. Like many other SCD patients as a child I endured many hospital stays, and E.R. visits. I took penicillin daily to help ward off complications. At the age of 9 I suffered a stroke in the right side of my brain which left me paralyzed on the left side of my body. Through the years I went to P.T. & O.T. to help me regain control & strengthen my left side. I was also put on blood transfusion therapy every 3 weeks until I was 19. Then at the age of 19 I began taking hydroxyurea to treat my Sickle Cell. It has drastically reduced hospital stays, and pain crisis, however it has had some minor complications. Then last year I was put on Jadenu to get rid of excess iron that came from blood transfusions I had in previous year's. I'm very hopeful for a universal CURE for SCD for myself, and everyone else in our community.

Please discuss any experience with blood transfusion:

After suffering a stroke at age 9 I was put on blood transfusion therapy until I was 19. Due to the frequent blood transfusions I was put on Jadenu last year to get the excess iron out of my blood that all the transfusions over the years put there.

Please discuss any experience with hydroxyurea:

I began taking hydroxyurea at age 19. It's been great for me. I have far less pain crisis which result in hospital stays. The only downside is that I have dealt w/ Ulcers as a side effect in the past.

Please discuss any experience with Endari (L-glutamine supplementation):

I was taking L-glutamine(Endari) for about 6weeks, but couldn't continue due to recurring headache's.

What do you feel is important for ICER to know about the quality of life for SCD patients?

I feel that quality of life for SCD patients can be great. If proper care, and treatments are available to every SCD patient. Continuing to listen to us.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What is important for ICER to know about the day-to-day challenges of SCD?

Changes in weather, season, climate/elevation can bring on pain crisis. Lack of knowledge, education, & resources. People not listening to us, and lack of medical professionals treating us w/ professionalism when we are in need or care when we go to the E.R.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Yearly out of pocket \$3500.00 However that is only because I'm on my husband's health care now that we are legally married. If I wasn't it would be 4x that amount.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Transportation back & forth to my weekly Dr.'s visits.

What other considerations are important for ICER to understand about SCD?

I think it's important to understand while SCD is the common bond patients have every case/patient is different. My care/needs are far different than some of my fellow SCD friends. That is why it's so important that every voice is heard, and ICER take every person living w/ SCD into consideration when putting studies, or focus groups together. Education, Awareness, & Understanding go along way as we continue to the fight against Sickle Cell Disease.

Sincerely,
Tristan Lee
divostar28@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Shabreon Howard

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed with sickle cell ss disease at birth. Hospitalized at 1 years old. Acute chest syndrome 3 times. multiple cases of pneumonia. Slight case of pulmonary hypertension. Diagnosed with Iron overload as a result of chronic transfusions. countless hospitalizations as adolescent to adulthood. beginning stages of kidney disease. I have take hydroxourea and endari, folic acid daily antibiotics as child. oxygen therapy, opioid pain medication therapy by mouth and intravenous

Please discuss any experience with blood transfusion:

As a child I received transfusion therapy which ultimately led to me having iron overload. It was a form of treatment that didn't work well for me so my gaurdians stopped the treatment. I still receive blood transfusions when my counts are too low. This only adds to my health problems in the long run.

Please discuss any experience with hydroxyurea:

I was put on Hydroxuearea as a child my dosage was increased to six tablets a day. I felt terrible and it didn't prevent any changes in my condition as it was promised to me and my family so I stopped taking it. As an adult after I gave birth I decided to give it another try. The outcome was pretty much the same I was actually sicker when I took it, it also caused hair loss.

Please discuss any experience with Endari (L-glutamine supplementation):

I started taking Endari last year and it has helped give me more energy and helped me improve my quality of life. I had to cut back on my dosage because it caused me to have migraines.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

deep tissue massages should be included in treatment for sickle cell patients.

What do you feel is important for ICER to know about the quality of life for SCD patients?

It is extremely hard to maintain a quality of life for patients who have a hard time getting the medical community to believe the truth about sickle cell, it's severity is overlooked and people

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

seem to believe that no one actually cares about us or our illness. We are a very intelligent and talented community of patients who deserve fair treatment and proper care. As a patient and the child of a patient with sickle cell. The way that sickle cell patients are treated needs to change. the standard of care needs to change and it needs to be demanded from not just patients and their families but from people of power who can actually make those changes.

What is important for ICER to know about the day-to-day challenges of SCD?

Sometimes it is just difficult to get out of bed. Sickle cell can come on with out warning and consume the normalcy of any patients life. Being in great pain can consume not only the life of the patients but the family's life as well as makes it easy for a patients to feel like a burden. It is a challenge to go to school or maintain a job but most of us push through the challenges. It can cause great financial burdens on not just the patient but their family as well. It's also difficult to deal with insurance and medical professionals.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?300

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: I have to cover transportation costs and childcare for my daughter while I go to doctor appointments and hospital stays.

What other considerations are important for ICER to understand about SCD?

We are an amazing community of people who are valuable in spite of the illness that tries so hard to kill us. just because you cannot see the illness doesn't mean it isn't causing irreversible and severe damage. So take the time to understand who we are and what goes into the individual patient care and treatment.

Sincerely,
Shabreon Howard
shabreon.howard@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Marchell Newton

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:
Sickle cell disease SS

Please discuss any experience with blood transfusion:
I had a bad reaction, hives.

Please discuss any experience with hydroxyurea:
Hydroxyurea is doing well for me, as long as I don't take no more than 300ml.

Please discuss any experience with Endari (L-glutamine supplementation):
Endari seems to do well but my insurance won't pay for the medication.

What do you feel is important for ICER to know about the quality of life for SCD patients?

What is important for ICER to know about the day-to-day challenges of SCD?
Trying to stay healthy and stress free.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?
100 to 200 dollars

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance
I don't want to go there

Sincerely,
Marchell Newton
lilnewt6@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Deniesha Culverson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Friend in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

have seen my friend struggle with this disease for over twenty years. She has been hospitalized more times than I can count. Not all visits are positive. She sometimes gets treated poorly by medical professionals, and they don't always listen to her when she tries to tell them what's wrong. It definitely affects her lifestyle. She is talented, works in the arts but can not always travel to do work.

Please discuss any experience with blood transfusion:

Blood transfusions work for some, not all. My friend ended up in ICU after a transfusion.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Reduced pain and less time in the hospital would be beneficial.

What is important for ICER to know about the day-to-day challenges of SCD?

Some days you can feel great, then others you may feel horrible. Trust the patient when they tell you how they feel.

Sincerely,
Deniesha Culverson
dculvers1979@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Francis O

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I will say my Sickle Cell experience is like passing through the eye of a needle. Nobody understand how painful it is during crisis, all they see is a drug addict. Sickle Cell made loose good job opportunities. I've been using a wheelchair now for over six years due to Sickle Cell causing me to have a chronic leg Ulcer. I've had various treatments that yielded no results. I've used Opioids during crisis but I've totally stopped when morphine almost took my life. I'm using natural organic herbal supplements which has been so great and it works tremendously for me like magic living a crisis free life for decade. I'm only still struggling with the leg Ulcer, which throws me into depression sometimes even though I encourage other warriors often. Sickle Cell struggles is real, it takes only a brave and great heart to conquer from the various complications, stigmatization in the society for being a Sickle Cell, knowing that we struggle every day fighting for our lives. The health care system in my own country is appalling, due to the incompetence in the health sector caused by bad governance. So there's no hope for Sickle Cell patients here. To be honest Sickle Cell has made me attempt suicide before, Yes for real. People don't seem to understand what a Sickle Cell is going through even some family members don't, they only see a drug addict, always comparing your health or physical abilities with theirs. Saying I'm just too lazy without understanding what I'm going through physical, emotional and mentally due to Sickle Cell disease. I rather stop here because when I keep Thinking about the struggle, pains and various health complications Sickle Cell has caused I feel depressed. But I've chosen not to give up but fight the good fight to be alive living Sickle Cell cause it's a life time struggle.

Please discuss any experience with blood transfusion:

I had blood transfusion during my tender age and age sixteen, until MAY 2014 I had another transfusion because my leg Ulcer started bleeding to the extent my pack Cell volume (pcv blood count) was 10%. I had 3 pint of blood since then I've not been transfused anymore.

Please discuss any experience with hydroxyurea:

I used it once no positive signs since then I've never used hydroxyurea again.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Please discuss any experience with Endari (L-glutamine supplementation): I have never used it all

What do you feel is important for ICER to know about the quality of life for SCD patients?

A good medical attention, listening to the patient and taking note of their reactions towards any treatment. Give the right dosage of their medication when needed. Showing love and compassion not to be pitied but understanding our pains and struggles will help a lot.

What is important for ICER to know about the day-to-day challenges of SCD?

We go through physical, emotional and mental struggles daily due to Sickle Cell.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

\$10,000 USD

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Insurance doesn't cover or has ever covered any of my expenses so I do all from my own personal income.

What other considerations are important for ICER to understand about SCD?

It's so complicated that needs intensive medical care.

Sincerely,

Francis O

Meetj4life@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Ron Shapiro

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My adult daughter has Sickle Cell Disease and has spent up to 6 months in a given year in the hospital. She is in constant pain, but only goes to the hospital when absolutely necessary.

Please discuss any experience with blood transfusion:

She had a bad transfusion where the hospital didn't do a thorough comparison of blood type and almost killed her. She can no longer have transfusions.

Please discuss any experience with hydroxyurea:

She uses hydroxyurea but her hemoglobin still drops as low as 3.

Please discuss any experience with Endari (L-glutamine supplementation):

We have noticed a marked improvement in the health of my daughter. She has more energy and has had fewer hospitalizations. Although she's still in constant pain, it seems to be less.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Stem cell research looks to be the one hope for Sickle Cell disease, as well as many other rare diseases.

What do you feel is important for ICER to know about the quality of life for SCD patients?

You can rarely make long term plans, or plan on vacations because you never know how much pain you'll be in day to day.

What is important for ICER to know about the day-to-day challenges of SCD?

Some days you are in so much pain, it feels impossible to get out of bed and live a normal life. It takes longer to do most tasks.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Up to \$1000 some years.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

It's virtually impossible to have a full time job. When you spend months in the hospital each year, nobody wants to hire you, or keep you on a job.

What other considerations are important for ICER to understand about SCD?

It's a complicated disease. It affects each patient differently.

Sincerely,
Ron Shapiro
ron@format3000.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Floyd Willis

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I do not have the sickle cell trait, but my wife does. Our cousin is afflicted as well as her uncle who passed away some time ago. I see first hand what SCD can do...I have donated blood numerous times

What do you feel is important for ICER to know about the quality of life for SCD patients?

The pain is constant, and is felt throughout the body. A cure is obviously ideal, but pain management is important

What is important for ICER to know about the day-to-day challenges of SCD?

Constant pain on most days.

Sincerely,
Floyd Willis
Fwillis44@yahoo.com

Adrienne Shapiro

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My older brother had SCD and my oldest daughter has SCD. My youngest daughter has trait. My eldest has received good care as a child. Once she reached her late teens the disease began to take its toll. She was hospitalized twice a year, had her gallbladder removed, had a poorly matched blood transfusion which resulted in her having too many antibodies and blood transfusions no longer possible. By her late twenties the disease flared up. She became disabled with over 4 hospitalizations a year each lasting over 3 weeks. Her hemoglobin dropping below 3. She was in the hospital, or under home care or in the cancer center for infusion and pain control. I was unable to have consistent employment as I needed to be with her too make sure her doctor's orders were followed.

Please discuss any experience with blood transfusion:

With her third ever transfusion she received blood that was not matched well. As a result she had kidney failure and spent 2 weeks in ICU. The long term effect is she cannot receive blood transfusions as part of her SCD treatment

Please discuss any experience with hydroxyurea:

She is taking HU, there have been effects with her hair and sometime intestinal problems.

Please discuss any experience with Endari (L-glutamine supplementation):

She takes Endari and has had positive outcomes. Higher hemoglobin levels and more energy

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

There are no "usual care" treatment ts.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Sickle cell has long been described as simply a blood disease that causes pain due to blockages in the veins. It is much more complicated and the pain is only a signal that their bodies are being destroyed from within. From the diagnosis families are tasked with keeping their child alive.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Everything is dictated by the disease. For generations parents in our family have watched their children suffer and die. I am the first to have a child who will have access to treatments that will improve her life rather than treat her pain

What is important for ICER to know about the day-to-day challenges of SCD?

An unpredictable chronic illness with acute pain.crisis that require opioids in a world that fears Opioid addiction more than a body being raved by this disease. A life filled with starts and stops due to fatigue, pain, bias, sickness due to side effects of meds, lives where family and friends drop away like layers of lettuce leaves due to chronic illness fatigue

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

When she was a child paid for two insurance plans, I hit cap of 14 grand a year until she was 27 and could no longer be covered. We have paid Thousands on home health care supplies that were required by the home health agency and not covered by insurance. 3k a year for watchers when we had to go to work and the hospitalnrwquired someone to be with her before she could be given her meds.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance

Where to begin, parking at the hospital 30 dollars a week, gas, meals, missed days at work, unemployed periods, etc

What other considerations are important for ICER to understand about SCD?

It is extremely complicated - and it always ends with organ damage or infection

Sincerely,
Adrienne Shapiro
adrienne@axisadvocacy.org

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Traicart

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Sickle beta-thalassemia, avascular necrosis, gallstones. I've used heating pads, folic acid, juicing, light exercise to encourage circulation.

Please discuss any experience with blood transfusion:

I've only had a blood transfusion before/after thr surgery

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

I think people should learn more about juicing and which natural vegetables and fruits are good for our immune system.

What do you feel is important for ICER to know about the quality of life for SCD patients?

At the rate at which people die from sickle cell we strive to make the best of my life or at least try to. The best care means so much to us and we just want to live to see another day.

What is important for ICER to know about the day-to-day challenges of SCD?

It's so frustrating. It's not only physically painful but also emotionally. One day your mind controls your body, the next day a part of your body could control you.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I'm not exactly sure but besides hospital costs and medicine I end up buying topical pain relievers,

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: Missed work

What other considerations are important for ICER to understand about SCD?

Imagine if out of no where your world stops. It feels as though you have smashed a limb in a car door.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Sincerely,
Trai Icart
Icart.art@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Iris Johnson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have seen the pain, the lack of independence these people go through .The lack of understanding they receive during a crisis.when

Please discuss any experience with blood transfusion:

When they receive Blood transfusions iron builds up in theirs bodies and effects all their internal organs.

What do you feel is important for ICER to know about the quality of life for SCD patients?

They deserve the same quality of a pain free productive life as any other American.

What is important for ICER to know about the day-to-day challenges of SCD?

They are in pain all the time.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance.

They are unable to hold down a job be because of pain management meds.

Sincerely,
Iris Johnson
imsj0818@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Toby Robert

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed with sickle cell anemia (SS disease) at birth via newborn screening. I was diagnosed with a retinal infarct at age 11; this diagnosis came about because I had noticed changes in my vision that were caused by permanent loss of vision in the central field of vision in one eye. Because of this serious complication, I was started on transfusion therapy and have been on transfusion therapy ever since, for a total of over 26 years now. Because of my transfusion therapy, I've had 3 mediports (my current one plus two previous ones that had to be removed because they got infected), and I also had to be on iron chelation therapy for a number of years starting ca. 2000 due to iron overload because I had been on simple transfusions for 7 years at that point, and chronic exposure to simple transfusions is known to increase iron levels. (I'm no longer on simple transfusions; I was switched to red-cell exchange transfusions ca. 2000 and have been on those ever since.)

Please discuss any experience with blood transfusion:

See my response above for some info regarding my history with blood transfusions. Outcomes: My physicians and I noted that the transfusions have been effective in keeping me healthy. Side effects: Starting ca. 2007, I became prone to low blood pressure and fainting after my transfusions; heat and hunger increased the risk of fainting. So after my transfusions, I now make sure to stay well-fed, cool, and, of course, seated. I have been very adherent to my transfusion schedule because I know from experience that when I try to go too long between transfusions, I **will** get a sickle cell crisis that sends me to the ED. My transfusions (red-cell exchange transfusions) are every 10 weeks. The transfusions I did from about 1993 to 2000 were simple transfusions, and those were once a month.

Please discuss any experience with hydroxyurea: N/A

Please discuss any experience with Endari (L-glutamine supplementation): N/A

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

FISH OIL. I take fish oil every day. A small pilot study that I read about in 2011 found that fish oil reduced the frequency of sickle cell crises, so I started trying it and I found that it worked. I determined it worked because normally when I go 1 week too long between transfusions, I don't make it and end up in the ED and then get a transfusion the next morning. But on fish oil, I was able to go 2-3 weeks extra between transfusions and not fall into a sickle cell crisis (I did this only once, and it was because my health insurance that year was charging too much for transfusions, but it was the end of the calendar year, so I had to wait just a little bit longer until I could get a transfusion at a lower cost to me after December 31.)

What is important for ICER to know about the day-to-day challenges of SCD?

Stay well-hydrated is a must and it has always been my lifestyle because it is so important when you have this health condition.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

\$275*5 transfusions a year + \$40 copay for hematologist visit + \$40 copay for retinologist visit (to look at my retina & make sure its still stable after the infarct I had) = \$1,455

Sincerely,
Toby Robert
anekwe15@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Talana Hughes

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease: Sickle Cell Disease was discovered over 100 years ago and to date we have 2 FDA approved drugs! This is absolutely shameful. Sickle Cell Disease is has the highest incidence among all tests conducted during the Newborn screening testing. For the approx 100,000 individuals living or that have passed on from this disease there needs to be a variety of treatment options due to the very unique way this disease manifests differently in all patients! My 16 year old daughter with Sickle Cell Disease SS has lived a life of off and on pain, pain meds, transfusions, daily fatigue, hospitalizations, decreased quality of life and depression due to the nature of the disease and the unpredictability of when and how sickle cell disease is going to show up in her life on a daily basis! There technically is not a extensive treatment history because again to date the only option she has been given is Hydroxyurea which due to the side effects which were decreasing her quality of life she can no longer take! There needs to be treatment options now!

Please discuss any experience with blood transfusion: Blood transfusions only place a bandaid on sickle cell disease and long term cause iron overload because my daughters body will continue to produce sickle cells.

Please discuss any experience with hydroxyurea: Hydroxyurea caused my daughter hair loss and constant stomach pains. While on Hydroxyurea her hospitalizations increased and her quality of life decreased.

Please discuss any experience with Endari (L-glutamine supplementation): Has not been offered to my daughter at her institution

What do you feel is important for ICER to know about the quality of life for SCD patients? Treatment options for sickle cell disease need to exist and also need to include a variety of options that address health red blood cell production, fatigue and hemolytic anemia and not solely address pain and hemoglobin.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What is important for ICER to know about the day-to-day challenges of SCD? Treatment options for sickle cell disease need to exist and also need to include a variety of options that address health red blood cell production, fatigue and hemolytic anemia and not solely address pain and hemoglobin.

Sincerely,
TALANA HUGHES
talanahughes@scdai.org

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

R. Yolanda Johnson

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I am a 69 year old woman with SCD-SS and was diagnosed at the age of 2 by a black doctor in Middlesex County, VA in 1952. However, I was never given any-thing for pain until I was 2 months shy of 20 years old. I suffered severely with pain; I missed a lot of school yet remained on the honor roll. God blessed me with great parents, they would take turns day and night to rub the areas that were paining. When I had a crisis, the pain would start in one place and move from area to area. The only people outside my family that knew of my condition were my neighbors and my best friends in the neighborhood. When I would tell kids in school, they thought it was contagious so I didn't tell people anymore until the late sixties. By that time, Dr. Scott had exposed to the international community the facts about sickle cell anemia. I would like to add that I had a brother with SS who died at the age of 33.

Please discuss any experience with blood transfusion:

After the age of 20, I would have blood transfusions for any surgeries I would have in order to bring my blood count up. I was fortunate that I suffered no adverse effects from them. Prior to that, from 1965, I would be

Please discuss any experience with hydroxyurea:

I started on hydroxyurea in approx. 2006 and have been on it every since. I feel this drug has prolonged my life and it has severely cut down on the number of crisis and hospital visits. About once or twice a year I am taken off the drug for two weeks because some of my counts are off track.

Please discuss any experience with Endari (L-glutamine supplementation): N/A

What do you feel is important for ICER to know about the quality of life for SCD patients? As long as there is continued research, I believe the quality of life will continue to improve and eventually be eradicated. Life is much better for SCD patients and is continue to improve. I am speaking from no treatment in the 1950s to several treatments in 2019. However, there is a long way to go and funds are shrinking everyday. Because of this, I am an advocate for SCD to help bring more awareness of the disease to the public arena.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What is important for ICER to know about the day-to-day challenges of SCD? For me, I have pain everyday. However, with pain meds and hydroxyurea, I continue to push on. I have a high tolerance to pain which I believe comes from the fact for nearly 20 years I just suffered with no pain meds at all.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year? Over \$3,000.00 a year

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: I was forced to retire because of missing a great deal of work in 2000. Therefore, I was not eligible to receive my full retirement. Whenever, I am hospitalized or see other specialist they have cost that the insurance does not pay. I am divorced and after I pay rent, food, medicine and transportation many of those medical fees are not paid and my credit rating is in the dumps.

What other considerations are important for ICER to understand about SCD?

ICER needs to get in touch with the major SCD associations and doctors that specialize in hematology as well as have patient forums to track how the disease is being treated. Each patient has a different story as to how SCD affects them.

Sincerely,
R. YOLANDA JOHNSON
ryolanda1950@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Melinda Sobel

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Therapist in the sickle cell disease community.

Briefly describe your experience with sickle cell disease: I provide emotional support

Please discuss any experience with blood transfusion: I have worked with patients who have had extremely dangerous and harmful reactions to blood transfusions.

What do you feel is important for ICER to know about the quality of life for SCD patients? SCD patients are some of the strongest people/patients I know. They live with daily physical pain, which can suddenly become excruciating without warning. This, naturally, can greatly hinder their quality of life. This includes moving forward with life goals, dreams and hopes. The unpredictability of this disease is constant and unrelenting.

What is important for ICER to know about the day-to-day challenges of SCD? SCD patients are often treated as "addicts" rather than as patients arriving at the ED with excruciating pain and in medical crisis. These individuals have a physical and medical disease. In addition, they have been treated for these crises many times before and know what regimen works for them and what does not. Ask the patient. Listen to his or her story and feedback. Let him or her teach you.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: SCD crises are extremely unpredictable so missing work is an issue. One cannot plan for these days, nor can he or she plan how long he or she will be out. In addition, medical bills add up. This can lead to job and financial strain.

Sincerely,
Melinda Sobel
Sobelm@toweroncology.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Adejumobi Otekunrin

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Clinician or Researcher in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I am a trustee of Genotype Foundation, a sickle cell treatment & awareness NGO founded in 1997 based in Nigeria. I am a physician. Treatment of SCD includes: Hydration, folic acid and essential nutrients supplements, prophylactic penicillin (pediatric patients), hydroxyurea (to induce HbF to reduce incidence of crisis), ciklavir (same effect as hydroxyurea), blood transfusion. Complications have included blindness, stroke, IUFD(fetal death), acute chest syndrome, death. Diagnosis by hemoglobin electrophoresis.

Please discuss any experience with blood transfusion: Blood transfusion usually for patients with Hb < 6g/dL. Outcomes have included resolution of crisis and acute chest syndrome. Concern has been sensitization to minor group antigens.

What do you feel is important for ICER to know about the quality of life for SCD patients? I have seen over the 20years of my involvement with SCD patients that if given adequate treatment and support, they lead normal lives just like everyone else.

What is important for ICER to know about the day-to-day challenges of SCD? Financial and emotional support.

What other considerations are important for ICER to understand about SCD?

It impacts the overall socioeconomic state of the country. Especially if the incidence of crisis is not drastically reduced through better treatment to improve the quality of life of people with SCD.

Sincerely,
Adejumobi Otekunrin
oteks2004@yahoo.co.uk

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Ken West

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

History of AVN, on Hydroxyurea, both hips replaced with revisions. Have had skin ulcers recently.

Please discuss any experience with blood transfusion:

Transfusions only when skin ulcers appear.

Please discuss any experience with hydroxyurea:

Have been on Hydroxyurea since the clinical trial. Has worked well for me.

What do you feel is important for ICER to know about the quality of life for SCD patients?

We the Sickle Cell population still suffer from inadequate medical care. Specifically around how we are treated in the ED and lack of knowledge around Sickle Cell in the medical community.

What is important for ICER to know about the day-to-day challenges of SCD?

It is very hard to plan your life, we want to be successful, contributing members of society but it is a struggle to maintain employment, education and health which would contribute to health and wellbeing.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

The conundrum is you must have a job to have health insurance but you must be reasonably healthy to maintain a job.

Sincerely,

Ken West

kwest918@hotmail.com

Ardelia Aldridge

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My diagnosis has been down since 11 months old. I experienced many different occasions hospitalizations as a result of sickle cell crises as well as times when I have treated myself at home. During college I was prescribed opioids daily in order to get ahead of frequent crisis pain. I didn't start taking hydroxyl urea until my early 30's and have had great success with my drug regimen. I rarely take opioids now.

Please discuss any experience with blood transfusion:

I have had less than five transfusions over my 42 years as a sickle cell patient. While they have most times been successful in raising my blood count I have had the experience of poor nursing care which she did not allow for a total transfusion of the two units I was supposed to get I also get facial cold sores directly afterward every time I am transfused.

Please discuss any experience with hydroxyurea:

I am taking hydroxyurea currently and have been since I believe the age of 31 or 32 and I believe it has been successful in staving off a great deal of sickle cell crises for me.

Please discuss any experience with Endari (L-glutamine supplementation):

I have recently been prescribed Endari as a result of L arginine becoming a another source of facial cold sores but I did take L arginine for roughly 10 years I believe the Endari is working but I wish they would press it into pills instead of the powder mixing it with liquid

What do you feel is important for ICER to know about the quality of life for SCD patients?

As an adult this disease can become offensive because it is debilitating and can literally stop you're alive when you were just trying to do regular normal things and be a normal person. It is anxiety inducing when I becoming fatigued but still have to be working because I cannot live if I don't keep my job that keeps my health insurance which allows me to continue having access to the drugs and I need to sustain my semi normal life.

What is important for ICER to know about the day-to-day challenges of SCD?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

One of my biggest major challenges of life is my living situation. I live alone in a junkie messy apartment because I prioritize resting over actually physically taxing myself to clean up my apartment. And that sucks. I buy more clothes because I sometimes can't get my laundry done. That sucks too. Prioritizing your physical energy is really mentally taxing

How much do you spend (out-of-pocket) on SCD treatment or medical care each year?

I do all I can to keep my job with great health insurance. My cost per year are nominal.

What other considerations are important for ICER to understand about SCD?

Prolonged physical pain changes a person mentally and their outlook on life. I try to be positive but the war that goes on between my mind and body spills over into my personal and professional life often. Most people that don't know we cannot understand why I speak with such urgency and aggression because I do not have the time to wait around because I may run out of time here on this planet

Sincerely,
Ardelia Aldridge
ardelia.aldridge@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Susana Rendon

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease: Avn, pneumonia, acute chest ,
SickleCell ss, spleen removal, gallbladder and asthma.

Please discuss any experience with blood transfusion:

My daughter has received blood transfusions at several times and it at times has caused rashes no iron overload yet.

Please discuss any experience with hydroxyurea:

First time my daughter got hydroxyurea as it increased she was becoming more ill almost bed ridden years later her avn was discovered. We stopped for awhile now we restarted and there is only side effects of upset stomach, tiredness and loss of hair and appetite.

Please discuss any experience with Endari (L-glutamine supplementation):

She just started taking endari up to now we have had no side effects .

What do you feel is important for ICER to know about the quality of life for SCD patients?

Quality of life I have a different view. My daughter has come along way but we still struggle for us quality of life would be good quality care without preconceived notions or assumed conditions.

What is important for ICER to know about the day-to-day challenges of SCD?

My daughter lives daily with pain and she struggles to do the daily things that I take for granted such as taking a walk or doing things that are active due to the affects of sickle cell on her body.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Too much to count. I have had to consolidate my bills because it was becoming so hard to keep up with my own normal electric etc.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Missed work so her papa looks after our little one at night he works nights so he losses work and parking cost when I stay overnight and he goes in the morning.

What other considerations are important for ICER to understand about SCD?

It is not an understood disease that people that are clueless assume that parents can take care of all the financial as well as emotional drain this disease affects the whole family.

Sincerely,
Susana Rendon
Sfordeli@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Liza Howell

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have always has problems with my fatigue and lungs I've had Pneumonia pneumonia many times and blood when I pee. Pains come and go when on a regiment but my doctors really don't know about the disease.

Please discuss any experience with hydroxyurea:

It made me feel better but also made my hair fall out

Please discuss any experience with Endari (L-glutamine supplementation):

I would like to try it

What do you feel is important for ICER to know about the quality of life for SCD patients?

That if you are taken care and have people in you corner or professionals that can help life quality will be good.

What is important for ICER to know about the day-to-day challenges of SCD?

That everyday is different last night you might not have had any problems but this morning I feel like I got hit by a train. Or last week I had work and it was the same type of work but this week I feel the difference and he hits hard.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

2,000 for meds doctors appointments and other treatments that are herbal that might help and not to mention insurance monthly

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

I miss a lot of days of work going to appointments my boss so what understands but it's still hard to get it through to some people. I dropped out of college classes because it became to much I'm trying to become a dental hygienist and this has been a long ride but scd will not stop me.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What other considerations are important for ICER to understand about SCD?

That everyone is affected by it co workers family friends spouse kids foster kids parents. I think people shouldn't have counseling for this disease and try to see if it can help just a little then maybe you stopped an person with scd not committee suicide or hurt them self.

Sincerely,
Liza Howell
willowhowell@yahoo.com

Francesca Valentine

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Our son is HgSS diagnosed at 6 months of age . The disease complications include 6-10 episodes of acute chest , one episode requiring mechanical ventilation , others ICU admission with BiPaP. All episodes of acute chest with hypoxia and impending respiratory failure require red cell exchange . The other complications include heart failure , splenectomy , cholecystectomy , pneumonias , osteomyelitis, restrictive lung disease, lower extremity wounds, total hip replacement , knee allograft , stroke , seizures, depression , operating room for incision and drainage of wounds , central line insertion , port a cath placement , lymph node removal for blastomycosis, and every six weeks he goes to interventional radiology for Quinton catheter placement to receive the red cell exchange.

Please discuss any experience with blood transfusion:

Currently complete red cell exchange is the only available treatment for life threatening complications such as acute chest , also to prevent stroke . The concern for transfusions are the development of antibodies and iron overload. Treatment for iron over load is mandatory to prevent organ damage.

Please discuss any experience with hydroxyurea:

espite adherence to hydroxyurea there are still hemolytic crises , pain and the infection risk is elevated . Hydroxyurea can contribute to devastating chronic leg ulcers increasing infection and amputation risks.

Please discuss any experience with Endari (L-glutamine supplementation):

Endari is helpful with energy levels and minimal side effects but Does not stop a crises in progress.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Each treatment for SCD is individualized depending on what part of the body has interrupted circulation. There currently is no usual. Each treatment which are all temporary is unique to each persons level of Sickling and what organs are involved.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Living with SCD is like an internal war , not always visible to the eye. It wages war as it kills the persons body a piece at a time. The entire family is effected. Any time circulation is stopped abruptly or decreased pain is excruciating. Wondering if there is another day of life ahead can consume a persons life in all aspects. Imagine being crushed to death as your blood circulation ceases to exist. This is SCD then add the stigmas of being drug seeking for the horrible pain , limited standards of care despite its 110 year history.

What is important for ICER to know about the day-to-day challenges of SCD?

No one wants to be disabled , in horrible pain and struggle to complete their activities of daily living. Persons with SCD are the strongest humans on the planet they live each day to the best of their abilities grateful for each sunrise. But just think I will have no idea when my circulation decides to become blocked and stop me from keeping a job , finishing school , marriage , family, or even wearing regular shoes if the feet are so swollen from wounds . Most people take for granted the shoes they wear not with SCD.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

We spend over 10,000\$ each year. Wounds alone costs 2000\$ in foot ware and another 7500\$ in supplies not covered by

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

In 2016 I used an entire 480 hour FMLA and my husband has lost jobs trying to keep our son alive. We have been in foreclosure 5 times and between the 2 of us we have worked four jobs at a time. There are additional out of pocket expense , care givers 11-19\$ / hr so we keep our jobs. We have equipment that is not covered by insurance and our handi cap bathroom was 15,000\$. When sickle cell kills your bones you can not walk. We have expenses related to life lines , tutors , service dog training , and the biggest cost time lost from work. We have lost upwards of 50,000\$ in regular salary in any given year.

What other considerations are important for ICER to understand about SCD?

Please understand SCD is long overdue for a treatment and cure. It is buried in years of racial discrimination and to this day health care Professionals treat based on assumptions not science. We need new drugs and treatments. SCD is like apples and oranges and its about time we matter. You can not make general comparisons. Our health care landscape is different there is poor transitional care and not enough adult hematologists taking on the post pediatric population. You can not determine a quality of life years for this disease that is in its infancy for recognition of needs and care.

Sincerely,

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Francesca Valentine
gradgirl52@yahoo.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Kevin Wake

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I was diagnosed with sickle cell thalassemia at 9 months of age two days after my older brother passed away and his autopsy determined that he had scd as well. My younger brother has also passed away from scd. My complications with scd include kidney disease, iron overload, gall stones, spleen removal, 2 diagnosed strokes, leg ulcers, and multiple infections due to the disease. I have had iron elation therapy, take hydroxyurea, folic acid, supplements, aspirin, and red blood cell exchange therapy every 5 weeks.

Please discuss any experience with blood transfusion:

I have had multiple blood transfusions over my life, and now am on chronic red blood cell exchange therapy every 5 weeks. The exchanges are able to maintain my sickle cell percentage below 30% and stabilizes my hemoglobin around 10. Complications from transfusions have included iron overload, antibody development, and most recently uncontrolled bleed from the port after the therapy

Please discuss any experience with hydroxyurea:

I take hydroxyurea. When I first took the drug, the optimal dose for my bodyweight dropped my white cell count and I had to discontinue treatment, have a washout period, and restart therapy at a lower dose.

Please discuss any experience with Endari (L-glutamine supplementation):

No experience

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

I do heat therapy in a hottub to help with pain management in hopes of limiting narcotic use

What do you feel is important for ICER to know about the quality of life for SCD patients?

SCD impacts patients physically with pain and other complications, but there is also a huge impact mentally, socially, it impacted the time for me to complete my education, it has impacted

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

my ability to work full time and hold a job, and the toll it has taken on my parents as caregivers and also their personal struggles with losing 2 children to the disease too is not even measurable in my opinion.

What is important for ICER to know about the day-to-day challenges of SCD?

SCD is extremely unpredictable, even for the most aware patient. There is such a stigma that I feel from having this disease, wanting to do so much and contributing to society and yet I am limited from achieving many of my hopes and dreams.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

The least annual cost has been \$20K

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Other financial burdens includes income loss from not working, childcare, cost of providers completing required documents for insurance/disability, the mental and physical toll on caregivers, home remodeling costs to improve accessibility after my stroke, inability to qualify for life insurance creates a financial burden at end of life.

What other considerations are important for ICER to understand about SCD?

I think the total picture of the variations of symptoms and impact of the disease is hard to fully understand, especially in a rare disease that has had limited exposure and development in the disease.

Sincerely,
Kevin Wake
kevinwake@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Kristine Chieh

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Splenectomy 2009, has had acute chest at least twice. On hydroxurea. Has chronic pain and has a large amount of hospitalizations for pain that's not always a crisis. Did aphaeresis for 3-4 months through a double port, but stopped due to antibodies. Had a prior single port removed due to infection.

Please discuss any experience with blood transfusion:

Developed antibodies from aphaeresis this year.

Please discuss any experience with hydroxyurea:

Has been taking it for year, but recently non-compliant because he feels it's "not working" has had to be educated a great amount on how it works, but still non-compliant with it. He's 17 years old and having a hard time with his illness.

Please discuss any experience with Endari (L-glutamine supplementation):

Was prescribed but did not take.

What do you feel is important for ICER to know about the quality of life for SCD patients?

Pain and fatigue are constant, and receiving relief from that is frustrating and not always possible. He misses so much school and is failing all of his classes, mainly due to nonattendance from hospitalizations and not feeling well.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Currently California Kids Care pays for all copays, but we have parking lot costs, transportation costs. It is not much.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Transportation for a minor when an adult is unable to accompany him. He is 17. Missed school is the main one, and we have had too much difficulty getting a substitute for that besides after

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

school tutoring. Because he's not 100% homebound with his illness. He misses it in spurts, so he doesn't qualify for a teacher to come to the house.

What other considerations are important for ICER to understand about SCD?

Trust between the patient and the healthcare team can be at risk sometimes because of the lack of adequate pain relief options.

Sincerely,
Kristine Chieh
kristine.chieh@gmail.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Danielle Shorter

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

This disease attacks the mind the spirit AND the body. We deal with horrible pains that pounce on my child randomly. With no cure, few physicians that understand the disease and even fewer treatment option, it is easy to feel doomed. We can only pray that one day Sickle Cell receive adequate funding so that this disease will finally be cured (for all people).

Please discuss any experience with blood transfusion:

We are on monthly transfusions which has lead to iron overload.

Please discuss any experience with hydroxyurea:

We tried HU. My daughters levels didn't not increase, however she did develops a really bad rash, head aches and tummy aches.

Please discuss any experience with Endari (L-glutamine supplementation):

N/A

What do you feel is important for ICER to know about the quality of life for SCD patients?

Not enough ER's or hospital's understand the disease. When a patient goes in for immediate help, that person (in pain) is made to wait in line, while their body slowly dies on the inside. Or, said patient might get turned away for not looking sick enough. Sickle Cell is an invisible monster.

What is important for ICER to know about the day-to-day challenges of SCD?

Cold days lead to a crisis Hot days lead to a crises Rooms with high A/c lead to crisis Back to school germs attack our low immune body. Infections/colds lead to a crisis It's hard.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

With insurance- transfusions cost \$2,000 each. We need those monthly. Plus the cost of Penicillin. Plus the cost of an iron chelator \$190 - \$10 per month. Plus hospital parking every month (\$11). Not to mention cold/flu doctors visits. It's a lot.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

I have to stay home from work, so my husband works 3 jobs. It can be tough on our relationship.

What other considerations are important for ICER to understand about SCD?

We needed funding for a cure, but we also need resources for patients while we wait for a cure. I know of single mothers that have to leave their child in the hospital ALONE, because she has to work. She isn't given enough leave time, so she makes the hard decision to keep the job that pays for insurance rather than spend the nurturing time that her daughter desperately needs. We need help

Sincerely,
Danielle Shorter

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Kalesia Voulgarellis

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

I have hbss. Have had more than my fair share of complications. Lung liver kidney spleen and nerve damage which have affected my eyes ears legs and my ability to balance and walk unassisted. Fibrosis of the lung and countless acute chest syndrome leading to hospitalizations

Please discuss any experience with blood transfusion:

I've lost count of the amount of transfusions I've had. I've had allergic reactions a few times

Please discuss any experience with hydroxyurea:

I was on this drink for a few years and develop fibrosis of the lung was told by my hematologist to discontinue

Please discuss any experience with Endari (L-glutamine supplementation):

I've not been prescribed this drug

What do you feel is important for ICER to know about the quality of life for SCD patients?

We should all be treated as individuals and not be thrown into a group as this disease affects us all differently

What is important for ICER to know about the day-to-day challenges of SCD?

It varies depending on the individual

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

Thousands of dollars even with insurance

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance: If I'm in the hospital four times a year for week(s) at a time. Having to support yourself and or a family this is not doable

What other considerations are important for ICER to understand about SCD?

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

It's painful, expensive, stressful and send anxiety though the roof

Sincerely,
Kalesia Voulgarellis
kalesiawright.kw@gmail.com

Marqus Valentine

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Patient in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

Was diagnosed with sickle cell hemoglobin SS at six months old. Heart failure, epilepsy, stroke, restrictive lung disease. I take medications for each condition. My family support is amazing. With my mom as a nurse we were taught what to do when I got sick.

Please discuss any experience with blood transfusion:

It's hard to count the amount of blood transfusions I have had. Blood has been a key component in keeping my health up. After you get blood you feel refreshed. Over the years I have had a few side effects from blood. I take medication for iron overload and before I get blood they Pre medicate with diphenhydramine and Tylenol. Exchange transfusions shouldn't be grouped together. The exchange transfusions should be separate.

Please discuss any experience with hydroxyurea:

I was given hydroxyurea to replace the transfusions. The hematologist at that time decided to stop my transfusion. After the change my life got very bad. I was back and forth to the hospital. Hydroxyurea takes time to build up before you see the benefits. My outcome could have been different if I was able to keep getting blood while the medication built up. I think others should know that.

Please discuss any experience with Endari (L-glutamine supplementation):

Endari help me my blood counts were good and my body was given a chance to heal up. Endari is completely compatible with my heart medication. I don't know why Endari doesn't effect me like the supplement version. Only down side to Endari is a slower gut motility and bad tummy aches. Outside of that it had helped me.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

I think VR along with pain meds can be used to manage sickle cell.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What do you feel is important for ICER to know about the quality of life for SCD patients?

ICER would benefit by taking time to build a different model for sickle cell disease. We have very little and I know icer has tools they use to build on. That won't work in the sickle cell community. Sickle cell is complex it can attack any body part. I feel that using a preset model on sickle cell is dangerous. ICER need to really build from ground up on a new model. With key focus on sickle cell disease. Trying to use a preset will always fall short.

What is important for ICER to know about the day-to-day challenges of SCD?

How much sickle cell disease effects every aspect of our life's.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?

I'm not sure how much is spent.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

I have seen my dad lose jobs and we had trouble keeping up with bills. My brother and sister were late

What other considerations are important for ICER to understand about SCD?

We have to rely on a lot just to survive every single day. I hope that your review can help the community and not hinder.

Sincerely,
Marqus Valentine
Marqus.Valentine@sickcells.org

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Nicole Gilley

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

It cripples a family. So much is determined on sickle cell. Things such as family vacations. If a crisis strikes the family is left to either go without their sick loved one or not go at all

Please discuss any experience with blood transfusion:

My friend almost died from a bad transfusion

*Please discuss any experience with hydroxyurea:*N/A

*Please discuss any experience with Endari (L-glutamine supplementation):*N/A

What do you feel is important for ICER to know about the quality of life for SCD patients?

Make doctors understand

What is important for ICER to know about the day-to-day challenges of SCD?

Weather exercise and advocacy.

Sincerely,
Nicole Gilley
nicole.gilley@outlook.com

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

Rae Blaylark

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear ICER,

Please see my feedback regarding ICER's Draft Scoping Document:

I represent a Family member or Caregiver in the sickle cell disease community.

Briefly describe your experience with sickle cell disease:

My 23 year old son was diagnosed with SCD at 2 weeks old. Our journey has been a tough one, some wins, some losses, but he's still alive! This is not without a fight and some very close calls. He has had numerous blood transfusions, silent strokes, ICU stays, major depression, anxiety, and cognitive functioning issues that have impaired his quality of life. Ther transition to adult care has been N horrendous, even with a fierce advocate and caregiver as a mother. Everyday is a battle to keep and alive and ro keep him fighting through the pain.

Please discuss any experience with blood transfusion:

My son was on transfusions for nearly a year because of high TCDs and also for uncontrollable pain exacerbations. Finding best matched blood is always difficult and the fear of developing additional antigens is an ongoing concern.

Please discuss any experience with hydroxyurea:

Hydroxyurea has been amazing. He stated this drug well before FDA approval in pediatrics. He usually responds well, however, when he dies have a pain crisis, iit I's always complicated by the severity and complexity of his passion and his responses (reactions) to the pain meds, once even going blind for 3 days, despite the narcan drip.

Please discuss any experience with Endari (L-glutamine supplementation):

His hospital doesn't promote it, we had to ask for it multiple times. Now his insurance won't approve it and we can't afford it on an out of pocket basis.

Please discuss any other treatments that you consider to be "disease-modifying" or treatment that you feel should be considered as usual care during the ICER review.

There are MANY new disease modifying therapies coming. Anything that decreases pain crisis, hospitalizations, de-oxygenation, stickiness, or end organ damage, wil improve quality of life and improve early mortality due to ther King term effects of this disease. Everyone is not the same, therefore having more outings to choose from is most ideal.

Public Comment to ICER's Draft Scoping Document: Sickle Cell Disease
Patient Advocate Responses

What do you feel is important for ICER to know about the quality of life for SCD patients?
QoL is more for MANY reasons, disease state, pain, disease and complication complicity, depression, anxiety, health beliefs, racial and medical stigma, micro aggression and flat out racism, and a multitude plethora of additional issues. To veer a patient, a caregiver, or just a person who cares, is enough to see the anguish of this horrible disease.

How much to you spend (out-of-pocket) on SCD treatment or medical care each year?
Anywhere from hundreds in a good year, to thousands in not so good years.

Discuss any other financial burdens of SCD outside of medical costs that are not covered by insurance:

Transportation, missed work, social engagement, e.t.c.

What other considerations are important for ICER to understand about SCD?
Black people's health and medical choices deserve to master too!

Sincerely,
Rae Blaylark
sicklecellmn@gmail.com

September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear Dr. Pearson,

As key stakeholders in the sickle cell disease (SCD) community, we recommend you postpone the SCD review and reschedule the review for a later date. We agree with and understand the Institute for Clinical and Economic Review's (ICER) overarching mission to ensure SCD patients have access to effective and affordable treatments. After review of the Draft Scoping Document, we have concerns about ICER's ability to accurately evaluate Crizanlizumab and Voxelotor at this time. Please see the concerns we outlined below:

Definition of Value

Historically, efforts to fight SCD have been slow and underfunded. Disparities in research innovation for SCD, when compared to other diseases are well documented, and have consequently led to lack of treatment options and high mortality rates for people with SCD. Recent scientific discoveries have led to the development of new treatments, many of which are currently in the pipeline and have provided the community with hope for improved quality of life in the future. Given the lack of current Food and Drug Administration (FDA) approved medications for SCD, special considerations are necessary to provide greater transparency and to incorporate more nonmedical and indirect costs in the review, and to better contextualize value assessments. We urge ICER to work collaboratively with SCD stakeholders and develop a comprehensive definition of value that can be used to assess this population.

Limitation of Comparators

SCD is defined by a group of genetically and clinically heterogeneous diseases that share biochemical and physiological similarities. This leads to a range of acute and chronic complications, driven by ongoing vaso-occlusion. Currently, we lack a clear understanding of the underlying mechanisms that result in the diverse manifestations (e.g. acute chest syndrome, pulmonary hypertension, renal dysfunction, strokes). Comorbidities differ for each individual with and each genotype of the disease, which lead to unpredictable disease progression.

Although evidence-based guidelines exist, they are not widely used or implemented as standard of care. Therefore, treatments should not be classified as "usual care." Treatments for SCD range from opioid administration, body system evaluations to surgical interventions, and other medical treatments. Moreover, hydroxyurea, blood transfusions, and oral L-glutamine (Endari™) therapy are inappropriate for comparison in this review. These disease-modifying treatments utilize different mechanisms of action and, therefore impact diverse clinical outcomes. Other real-world challenges including treatment access, insurance coverage, and patient adherence need to be

considered in ICER's comparative cost effectiveness analysis. Unfortunately, the aforementioned treatments may be limited based on age, location, genotype of SCD, disease severity, and socioeconomic status.

ICER's assessments rely heavily on evidence from randomized clinical trials (RCTs). We are concerned about the potential lack of an adequate representation of the SCD patient population in these trials. The clinical trials under review may represent a minority of the targeted patient population. Additionally, certain populations such as uninsured, socioeconomically disadvantaged, and rural populations are often under-represented in clinical research. To improve the accuracy of the value assessment, ICER should consider incorporating real-world evidence (RWE), such as cost of multiple treatments to treat comorbidities in SCD patients or diversity in different disease severity, in addition to clinical trial evidence. RWE can be better suited to inform payer decision making. ICER should allow sufficient time to elapse after these SCD products are approved and marketed so that RWE can be developed and incorporated into the assessment.

We urge ICER to delay the analyses of sickle cell disease until appropriate comparators are identified and validated with SCD experts.

Health State Categories

As discussed above, symptoms and complications of SCD are different for each person and can range from mild to severe. The scoping document described the use of two health states: SCD with and SCD without VOC. We recommend ICER to consider the review of SCD in different disease state categories: (1) acute events and (2) chronic conditions. The use of these health state categories may allow for a more accurate measurement of cost and outcome associated with states and transitions.

Additionally, health states must be inclusive of other comorbidities of SCD which require medical intervention and cause significant patient burden. These include but are not limited to stroke, acute chest syndrome, splenic sequestration, asthma, end-organ damage, and avascular necrosis (AVN). While VOCs drastically affect the quality of life for people with SCD, VOCs are not the major cause of morbidity and mortality.

Lack of Patient Perspective in the Value-based Price Metric

Individuals with SCD experience complications in multiple organ systems that begin early in childhood and accumulate across the life course. The debilitating nature of SCD impacts social relationships, employment, and the educational attainment goals of patients. SCD disproportionately affects Blacks, African Americans, and Latinx Americans. On top of disease burden, systemic racism, prejudice, and stigma have crippled the SCD community's access to quality care. Likewise, there are notable financial and emotional burdens on the caregivers and families of patients with SCD affecting various aspects of their quality of life including their social and professional achievements.

These "contextual considerations" are not incorporated quantitatively into ICER's suggested value-based price metric. Thus, ICER's value framework fails to capture the significant burden

SCD places on this community. We recommend the quality-adjusted life years (QALYs) analyses account for these “contextual considerations”, including non-health benefits, in-direct costs, and societal benefits, such as a faster return to work, improved ability to act as caregiver, better school performance, burden on and costs of caregiving, daily functioning, time accessing medical care, income loss, loss of productivity, insurance premiums, out-of-pocket expenses, changes to home and vehicle, assistive devices/equipment, and travel costs. These indirect costs and non-health factors are of considerable importance to SCD and other rare diseases. We believe the absence of these factors on ICER’s value-based price metric significantly limits the review’s real-world applicability.

If data describing these factors are unavailable, we recommend that ICER capture and quantify the patient perspective through qualitative assessments. We recommend that ICER solicit in-depth patient, caregiver, and provider interviews on the topics of 1) disease burden and unmet need, 2) current treatment benefits and harms, and 3) reduction of important health disparities. We urge ICER to prioritize the evaluation of the qualitative data and ensure that value is formally and robustly assessed from the patient perspective.

We welcome further discussion about the concerns and recommendations outlined above. We hope that ICER will take into consideration the request from the community and postpone the review of sickle cell disease until more research and insights are available to inform an appropriate methodology. We hope to see increased transparency in disease state selection in future reviews, and that ICER’s approach includes contextual considerations, patient engagement, and use of qualitative data.

Sincerely,

Axis Advocacy

Cayenne Wellness Center

Cystic Fibrosis Research, Inc.

Kids Conquering Sickle Cell Disease Foundation

Maryland Sickle Cell Disease Association

The Martin Center Sickle Cell Initiative

SCD Forum

Sick Cells

Sickle Cell 101

Sickle Cell Disease Association of America

Sickle Cell Disease Association of America, Michigan Chapter, Inc

Sickle Cell Disease Association of America, Philadelphia/Delaware Valley Chapter

Sickle Cell Disease Association of America, St. Petersburg Chapter

Sickle Cell Disease Association of Illinois

The Sickle Cell Experience

Sickle Cell Thalassemia Patient Network

Sickled Not Broken Foundation, NV

Supporters of Families with Sickle Cell Disease, Inc.

Tova Community Healthy, Inc.

Uriel Owen Sickle Cell Disease Association of the Midwest

International Association of Sickle Cell Nurses and Professional Associates

Individual Community Stakeholders

Lewis Hsu, MD, PhD, Pediatric Hematologist in Chicago

Kim Smith-Whitley, MD, Pediatric Hematologist in Philadelphia

Yvonne M. Carroll, RN, JD, Sickle Cell Advocate



September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Draft Scoping Document for the Treatment of Sickle Cell Disease

Dear Dr. Pearson,

Sick Cells is pleased to have the opportunity to submit comments in response to the Institute for Clinical and Economic Review's (ICER's) Draft Scoping Document on treatments for sickle cell disease (SCD). Sick Cells is a patient advocacy organization that aims to elevate the voices of the SCD community. We would like to offer the following suggestions to ICER for consideration.

ICER should consider waiting until more research and insights are available to inform an appropriate methodology for the assessment of treatments this orphan disease. If ICER decides to move forward with the timeline, the points below outline areas in the scoping document that are (1) unclear and (2) recommendations that ICER should consider in its review of SCD.

1. Current methodology does not consider how differences in health insurance status affect the ability to pay for and ultimately engage in health services. Further, health insurance status of SCD patients may also be related to pre-existing health conditions and the severity of disease. According to the 2017 U.S. Census, Blacks or African Americans and Latinx have the highest uninsured rates.
2. The ICER review does not appear to account for the heterogeneity in treatment options.
 - a. SCD is debilitating, occurring in about one of every 365 Black or African American births and one out of every 16,300 Latinx American births. About one in every 13 Black or African American babies are born with the sickle cell trait.
 - b. Some patients will experience more or less benefit from treatment than the averages reported from clinical trials. The majority of ICER's review will rely on pharmaceutical clinical trials. These averages are somewhat helpful, but subgroup analyses are needed, especially for diseases that disproportionately impact specific populations. If the clinical trials for Crizanlizumab and Voxelotor SCD are not adequately powered and weighted to account for the disproportionate number of people of color impacted by the disease, the field will be unable to determine if these new medications will be beneficial for those most impacted.
 - i. We recommend the routine use of sensitivity analyses in modeling to explore heterogeneity of treatment effects and avoid an overreliance on methods based on averaged estimates. The accuracy of analyses that change the age distribution or are specific to the Black or African American population would bolster confidence that the estimates and model assumptions are correct.
 - ii. ICER should consider adding qualitative data in the form of patient and provider interviews and a stakeholder survey to help inform the sensitivity analyses. These qualitative data along with the quantitative will help test the robustness of the model.
3. ICER should clarify how the review methodology uses usual care (hydroxyurea and transfusions) as the control group.



- a. SCD is a syndrome of diseases. Therefore, treatment cannot be classified as “usual care.” Treatment is based on clinical manifestations of the disease and its comorbidities. Defining usual care as hydroxyurea and transfusions is inappropriate and further reasoning why it may be more appropriate to review SCD at a later date.
4. Quality-adjusted life years (QALYs) have significant limitations including ethical considerations, methodological issues, theoretical assumptions, and context or disease specific considerations.
 - a. QALYs analyses fail to account for non-health benefits and indirect costs. Non-health benefits and in-direct costs and in particular, societal benefits, such as a faster return to work, improved ability to act as caregiver, better school performance, burden on and costs of caregiving, mental health challenges, daily functioning, time accessing medical care, income loss, loss of productivity, insurance premiums, out of pocket expenses, changes to home and vehicle, assistive devices/equipment, and travel costs are not factored into QALY calculations despite being of potentially considerable importance to SCD and other rare diseases. If these data are unavailable, ICER should clearly define how sensitivity analysis will be performed. ICER can also use Work Productivity and Activity Impairment (WPAI) index for indirect costs.
 - b. QALYs utility scores fail to account for a variety of additional health-related factors such as severity of the initial health state and disease prevalence.
 - c. ICER should clarify the estimation of capital costs in computing hospital costs. Current estimates use what the hospital billed to the payee, not the patient. Other direct costs include emergency department visits and medications.
 - d. QALYs analysis currently include a population average. However, SCD generally affects the very young and younger adults. Therefore, ICER should consider analysis using at least two different age groups that reflect the ages SCD typically affects 25% under age 18; 75% 18 years and older. Furthermore, there is an aging population of patients with SCD. It is unclear how the ICER review will address patients over age 45.
 - e. The average population analysis of QALYs also does not account for Blacks and African Americans being disproportionately affected. It would be best to review averages within Black and African American populations.
 - f. Current QALYs methodology does not account for the impact of those with fewer treatment options. QALYs gained could be considered alongside other quantified aspects of benefit and combined in a way that reflects the trade-offs people are willing to make between an aggregate measure of benefit considered alongside cost. For some patients, a modest, incremental gain may be clinically meaningful and may significantly improve quality or length of life. However, the QALYs analyses are not sensitive enough to measure small but clinically meaningful changes in health status or utility.
 - i. QALYs could be ‘weighted’ to reflect any differences in the value society places on QALYs gains by some patients that is supported by patient preferences.
 - ii. There are a set of methods available to facilitate understanding patient preferences known as multiple criteria decision analysis (MCDA) that could be used.
 - g. Patients with lower QALYs, whose lives are extended, will have overall higher/unfavorable incremental cost per QALYs than patients with mild disease.
 - i. Currently, it is unclear how the model will incorporate different disease states. The QALYs by disease state could vary considerably.



- ii. It may be best to consider the review of SCD in two different disease state categories: (1) acute events and (2) chronic conditions. Each disease state has different medical interventions.
- 5. It is unclear how ICER will address multiple patient outcomes
 - a. Possible suggestions are to use a health index by combining all the health outcomes with different weights
- 6. The ICER definition of serious health condition is unclear.
 - a. The definition of serious condition should not be subjective but instead based on predefined criteria accepted by clinicians and patients.
 - b. QALY estimates vary depending on the seriousness of the health condition. Therefore, it is important to clearly define serious health condition, and how to account for many comorbidities and side effects of medical interventions patients with SCD experience.
- 7. Highlight the limitations of non-randomized control trial (RCT) data sources.
 - a. If ICER decides to incorporate additional sources of non-RCT data, limitations should be noted.
 - i. Clearly defined outcomes such as survival time or analysis end points that are evaluated by ICER must be pre-specified by the researchers and not the result of secondary or tertiary post hoc analyses. These time considerations are not clearly defined in the protocol and it is important for ICER to consider a time long enough to capture all potential differences in costs and outcomes.
 - ii. ICER should fully disclose all data sources and approaches that inform the contextual considerations sections of ICER reviews to provide increased transparency in the assessment process.
- 8. Considerations for the Markov model
 - a. ICER should clarify the different disease states in the Markov model and how transitions between disease states will impact cost estimates.
 - b. It is also important to perform a sensitivity analyses on the assumptions and parameters used in Markov model. Performing this step allows one to see how sensitive the results are to slight changes to parameter values.

Thank you for your consideration of these suggestions. Sick Cells will continue to engage with ICER throughout this review to ensure that the patient perspective is effectively heard and valued.

Sincerely,

Ashley Valentine, MRes

President and CEO



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September 20, 2019

Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: Stakeholder List for Sickle Cell Disease

Dear Dr. Pearson,

Sick Cells is pleased to submit comments about the Institute for Clinical and Economic Review (ICER) Sickle Cell Disease: Stakeholder List.

The sickle cell disease (SCD) community has a vast reach of community-based organizations. Each region of the United States faces its own, unique challenges. With this in mind, each CBO works within its local governments and healthcare systems to increase access to and quality of care for SCD patients and families. Partner organizations of the SCDA should be evaluated as stand-alone CBOs.

The current list of stakeholders is incomplete and missing key CBOs and professional societies that contribute to the landscape of SCD. Sick Cells encourages ICER to work with all SCD CBOs and professional societies across the United States to better understand and accurately capture disease states. Sick Cells included a list of active CBOs including those already listed in the current Stakeholders List.

Sickle Cell Disease Community-Based Organizations

Alabama

- North Alabama Sickle Cell Foundation – Huntsville
- SCDA-West Alabama Chapter – Northport
- Central Alabama Sickle Cell Foundation – Birmingham
- SCDA- Mobile Chapter, Inc.
- Sickle Cell Foundation of The River Region- Montgomery
- Southeast Alabama Sickle Cell Association, Inc.- Tuskegee
- Cayenne Wellness
- Axis Advocacy
- Sickle Cell 101
- Sickle Cell Disease Foundation of California
- Sickle Cell Warriors, Inc.

Colorado

- Colorado Sickle Cell Association, Inc. – Denver

Connecticut

- Citizens for Quality Sickle Cell Care, Inc.-New Britain/Hartford

California



- SCDAAs Southern Connecticut, Inc.- Bridgeport/New Haven

Delaware

- Tova Community Health

Florida

- SCDAAs of Escambia & Santa Rosa Counties – Pensacola
- SCDAAs Dade County Chapter, Inc. – Miami
- SCDAAs St. Petersburg Chapter
- Sickles Cell Association of Hillsborough County
- Sickles Cell Disease Association of Florida, Inc.-Tampa, FL
- Sickles Cell Disease Association of Tri-County, Inc. – Orlando
- Sickles Cell Foundation, Inc.- Tallahassee
- Foundation for Sickles Cell Disease Research

Georgia

- Sickles Cell Foundation of Georgia, Inc.- Atlanta
- The Sickles Cell Community Consortium

Illinois

- Sickles Cell Disease Association of Illinois – Chicago

Indiana

- Martin Center, Inc. – Indianapolis

Kansas

- Uriel Owen Sickles Cell Disease Association of the Midwest

Louisiana

- Northeast Louisiana Sickles Cell Anemia Foundation – Monroe
- Southwest Louisiana Sickles Cell Anemia, Inc- Lake Charles

Maryland

- Maryland Sickles Cell Disease Association, Inc. (MSCDA)
- Sickles Cell Disease Association of America, Inc.

Massachusetts

- Greater Boston Sickles Cell Disease Association, Inc.

Michigan

- SCDAAs Michigan – Detroit

Missouri

- Sickles Cell Association of St. Louis

Nevada

- Nevada Childhood Cancer Foundation- Las Vegas

New Jersey

- The Sickles Cell Association of New Jersey, Inc. – Newark

New Mexico

- The Sickles Cell Council of New Mexico – Albuquerque
- Kids Conquering Sickles Cell Disease and the Young Adult Sickles Cell Alliance

New York



- Falling Angels Sickle Cell Foundation
- Queens Sickle Cell Advocacy Network – Queens Village
- Sickle Cell Thalassemia Patients Network (SCTPN)

North Carolina

- Bridges Pointe Sickle Cell Foundation – Durham
- Community Health Interventions and Sickle Cell Agency, Inc. – Fayetteville
- Piedmont Health Services and Sickle Cell Agency – Greensboro

Ohio

- SCDA-Ohio Sickle Cell & Health Association – Columbus
- American Sickle Cell Anemia Association

Oklahoma

- Supporters of Families with Sickle Cell

Oregon

- Sickle Cell Anemia Foundation of Oregon – Portland

Pennsylvania

- Children's Sickle Cell Foundation, Inc.- Pittsburgh
- SCDA-Philadelphia/Delaware Valley Chapter
- The South Central PA Sickle Cell Council – Harrisburg

South Carolina

- James R. Clark Memorial Sickle Cell Foundation – Columbia

Tennessee

- Sickle Cell Foundation of Tennessee – Memphis

Texas

- Sickle Cell Association of Houston
- Sickle Cell Association of Texas Marc Thomas Foundation

Virginia

- Sickle Association Inc.- Norfolk

Sickle Cell Disease Professional Societies

- International Association of Sickle Cell Disease Nurses and Physician Assistants
- National Minority Quality Forum
- American Society of Pediatric Hematology/Oncology

COMMENTS to ICER document for Sickle Cell Disease
Julie Kanter

1. Background:

- a. This description vastly over simplifies the clinical manifestations and pathophysiologic process in SCD. I would further increase this section.
- b. Important to note that there is a peak in mortality in young adults (between 18-30 years of age) which results in the average lifespan being 41-43.
- c. Re: Cost this is very important. However, this does not include necessary data on impact on quality of life, requirement that many patients rely on public insurance and disability (and cannot work even part time for fear of losing disability insurance).
- d. Re: current 'standards of care' Another issue is the limits/barriers to these medications and this is woefully neglected here.

2. Stakeholder input

- a. Again-access to care remains a large issue with making sure that patients have a sickle cell specialist. This should be included here.

3. Analytic framework concerns: This framework is not sufficient. \

- a. Crizanlizumab has only been published in patients >16 years not starting at 2 years of.
- b. The majority of the patients treated with Voxeletor are also adults.
- c. These intermediate outcomes are really not optimal as we actually don't know that changing Hb will translate to improving outcomes. We don't know that reducing hemolysis will actually decrease renal dysfunction or other outcomes.
- d. Neither of these drugs has studied the affect on blood transfusions OR that blood transfusions negatively affect outcomes.
- e. THE ONLY data we know at present is that reducing vaso-occlusive crisis that require acute care will both reduce mortality and decrease cost. That is all we have actually proven. The rest is theoretical and this framework is therefore incomplete.

4. Populations

- a. Majority of data only available for adults

5. Interventions

- a. Usual care is NOT just medication. It also needs to include REG visits with hematology/SCD specialist, screening assessments for end-organ disease and aggressive pain medication during acute crisis. These must be included as there

is a lot of fear that more doctors will treat patients with these new drugs but will NOT give full care.

6. Comparators:
 - a. It is also important here to state that the drugs have different endpoints and cannot be compared at all.
7. Table 1: I don't think you can measure outcomes that are not evaluated by either of these drugs. There are several listed outcomes here that have not been tested.
8. Surrogate/interim markers: THESE ARE NOT SURROGATE OUTCOMES THAT ARE PROVEN TO CORRELATE WITH ANY NEGATIVE CLINICAL OUTCOME. THIS MUST BE NOTED.
9. Scope of comparative analysis
 - a. At this time, we only have data that CRIZANLIZUMAB decreases VOC that require acute intervention. This translates to reduction in acute chest (50% occur while in the hospital setting). We know this will translate to decrease mortality.
 - b. We don't have any correlative data for Voxeletor. We know it increases hemoglobin but there is NO correlation for VOC, pain, mortality, organ dysfunction etc. There is no data that says if we change hemoglobin level (without changing the type of hemoglobin, it will result in any other change.

Overall: It is most important that we take a universal approach and that our goal is that ALL Patients have an equal opportunity to receive these medications if approved.