

Sickle Cell Disease Patient and Caregiver Preference Study

What did this study look at?

Pfizer worked with Sick Cells to develop a survey to understand how much different features of potential sickle cell treatments matter to people with sickle cell disease and caregivers of people with sickle cell disease. The results from this survey will help Pfizer:

- Design clinical trials that measure what matters to patients and caregivers
- Make sure they are developing treatments that meet the needs of people with sickle cell disease and their caregivers

Pfizer partnered with Sick Cells to gather this information. Sick Cells is a community-based advocacy organization dedicated to elevating the voices of the sickle cell community. The survey was open from February 21, 2022, to April 21, 2022.

What information did the survey capture?

In this survey, people were asked 6 different questions called point-allocation questions (see example question on next page). In each question, people were shown 5 or 6 items that could be associated with a treatment for sickle cell disease. A total of 26 items were shown to respondents. They were asked to give points to each item to show how important they think it is that a sickle cell treatment has that item.

- If an item was **more important**, they gave **more points** to it.
- If an item was **less important**, they gave **fewer points** to it.
- If an item was **not at all important**, they could give **zero (0) points** to it.

They had 100 points to give to items in each question so that the total number of points across all items in a question added up to 100.



Example of a Point-Allocation Question in the Survey



Sickle Cell Preference Survey

Please look at each of the 5 items listed below. Each item is something a treatment can do to help with pain crises. We would like to know how important these things are to you.

Imagine you have 100 points to give across these 5 items. Please assign points to each item based on **how important you think it is that a sickle cell disease treatment address that item**. A higher number of points means that you think the item is more important to address.

Please be sure to assign points so that the total number of points across all 5 items adds up to 100. An item should have 0 points only if you think that addressing that item doesn't matter at all.

Shorten the length of pain crises

Reduce the frequency of pain crises of any severity (whether it is managed at home or at the emergency room)

Reduce the frequency of pain crises that require medical attention (that cannot be managed at home)

Reduce the level of pain experienced when having a pain crisis

Reduce severity of pain crises associated with menstruation or pain associated with priapism (having a painful erection that lasts a long time)

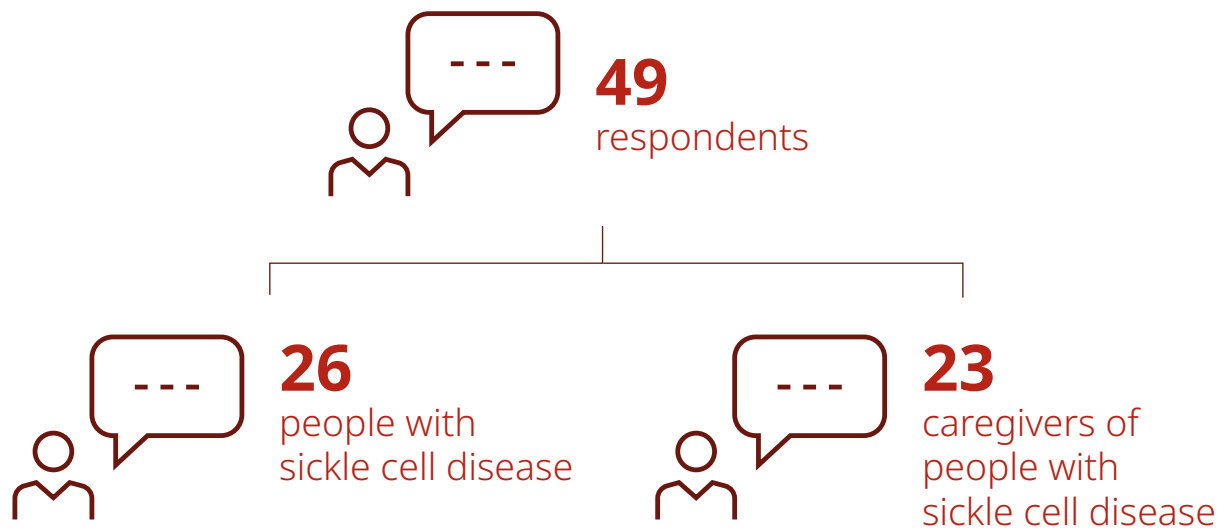
Total



Table 1. Sickie Cell Treatment Items Studied in the Survey

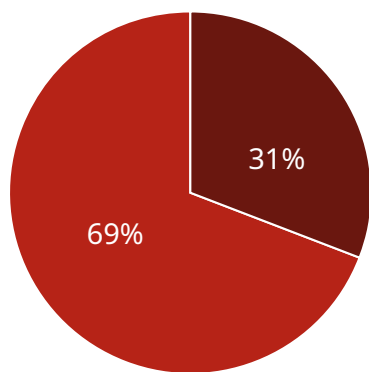
Things a Potential Treatment for Sickie Cell Disease Could Do:	
1	Shorten the length of pain crises
2	Reduce the frequency of pain crises of any severity (whether it is managed at home or at the emergency room)
3	Reduce the frequency of pain crises that require medical attention (that cannot be managed at home)
4	Reduce the level of pain experienced when having a pain crisis
5	Reduce severity of pain crises associated with menstruation or pain associated with priapism (having a painful erection that lasts a long time)
6	Increase hemoglobin levels
7	Reduce the risk of having a stroke (including strokes that don't have any noticeable symptoms)
8	Reduce the risk of having organ damage (including damage to the liver, heart, kidneys, gallbladder, or eyes)
9	Reduce the risk of acute chest syndrome (when sickled cells clump together in the lungs)
10	Reduce the risk of severe joint problems in the hip or shoulder (avascular necrosis/AVN)
11	Reduce the risk of getting a painful wound in your lower leg that won't heal (leg ulcer)
12	Reduce the risk of kidney damage
13	Reduce fatigue (tiredness that doesn't get better after sleep)
14	Reduce risk of having fertility issues (including low sperm count or inability to get pregnant)
15	Reduce need to use opioids to treat pain
16	Reduce risk of headaches and problems thinking or concentrating
17	Reduce the risk of getting a burning feeling, cramps, and weakness in muscles
18	Improve ability to do normal daily activities
19	Increase oxygen while sleeping which can help the body rest
20	Reduce need for transfusions and risk of iron overload
21	Less redness and swelling at the location where the medicine is injected
22	Less chance that the treatment will stop working over time
23	Less chance of having nausea, vomiting, or diarrhea because of the medicine
24	Less chance of having myelosuppression (bone marrow problems cause fewer red blood cells, fewer white blood cells, and fewer platelets than normal and can cause fatigue, shortness of breath, bleeding, and infections)
25	Not needing to take oral pills or tablets every day
26	Not needing to get an injection under the skin at home every 2 weeks

Who took part in this study?

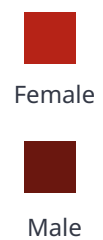
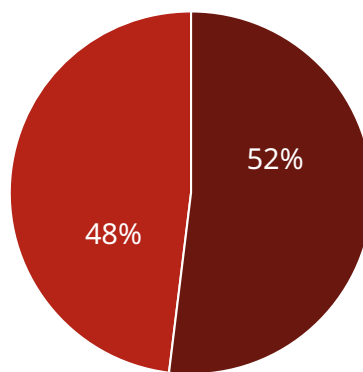


Patient Gender

Self-reported by Patient

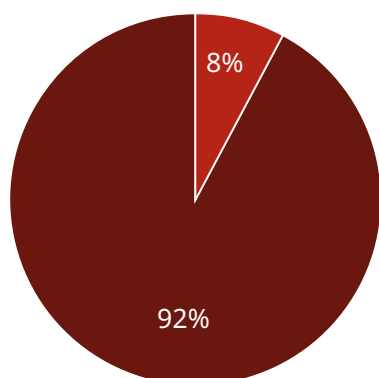


As Reported by Caregiver

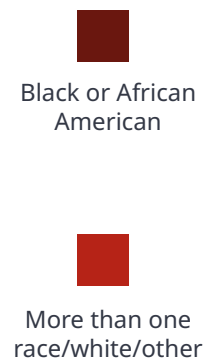
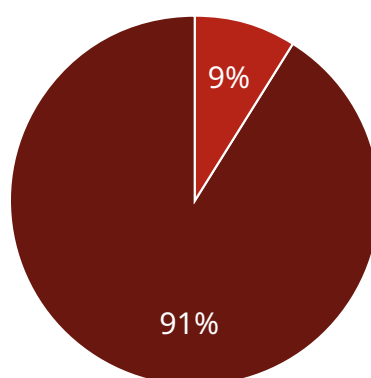


Patient Race and Ethnicity

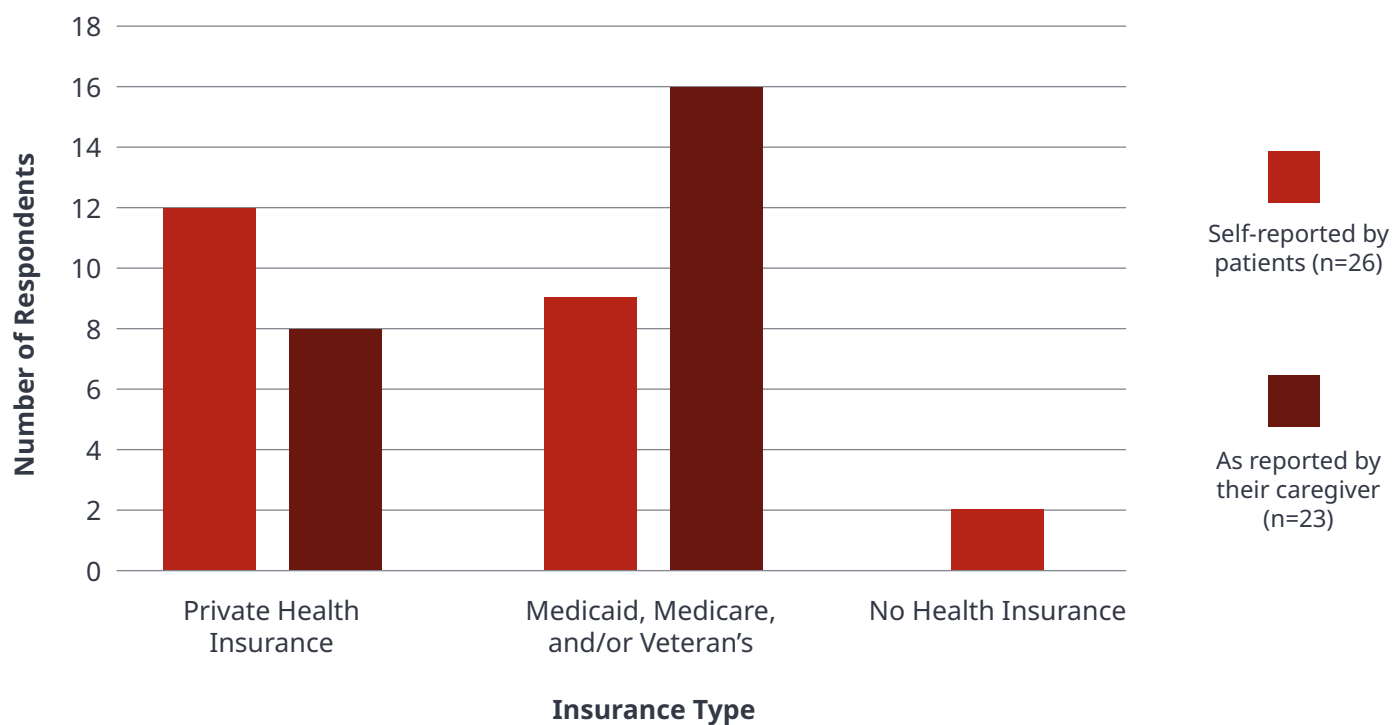
Self-reported by Patient



As Reported by Caregiver

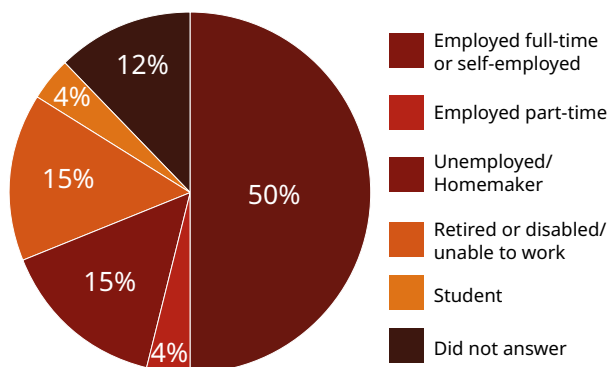


Health Insurance Coverage

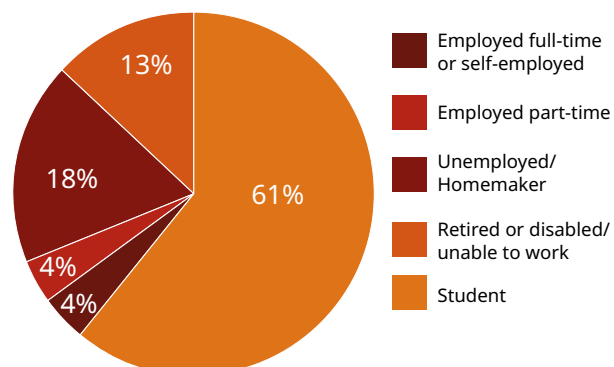


Patient Employment Status

Self-reported by Patient

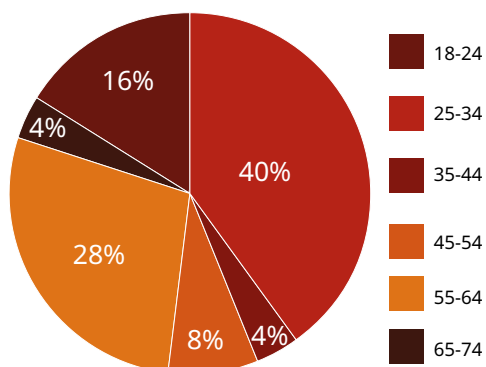


As Reported by Caregiver

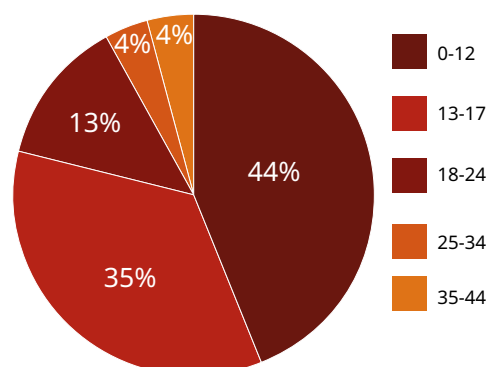


Patient Age

Self-reported by Patient

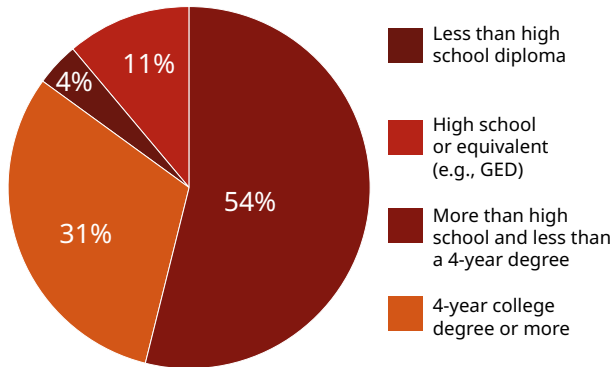


As Reported by Caregiver

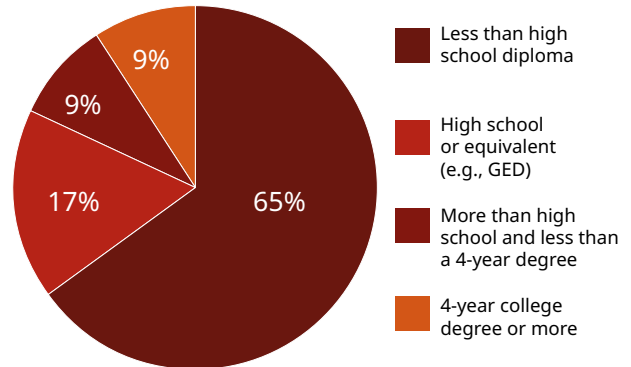


Patient Education

Self-reported by Patient

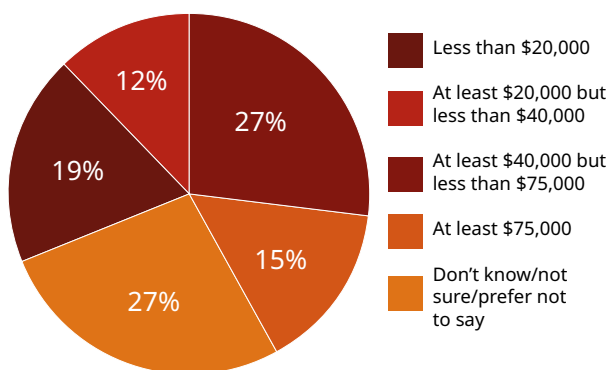


As Reported by Caregiver

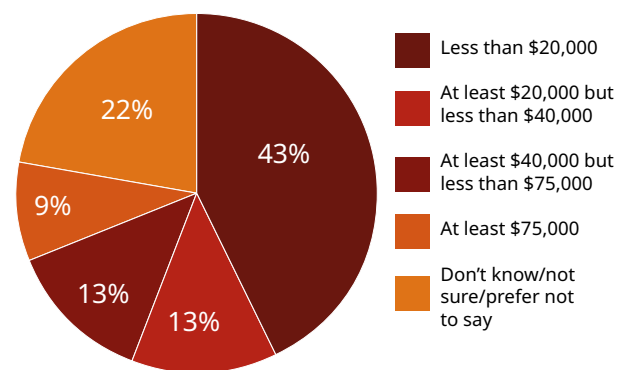


Patient Income

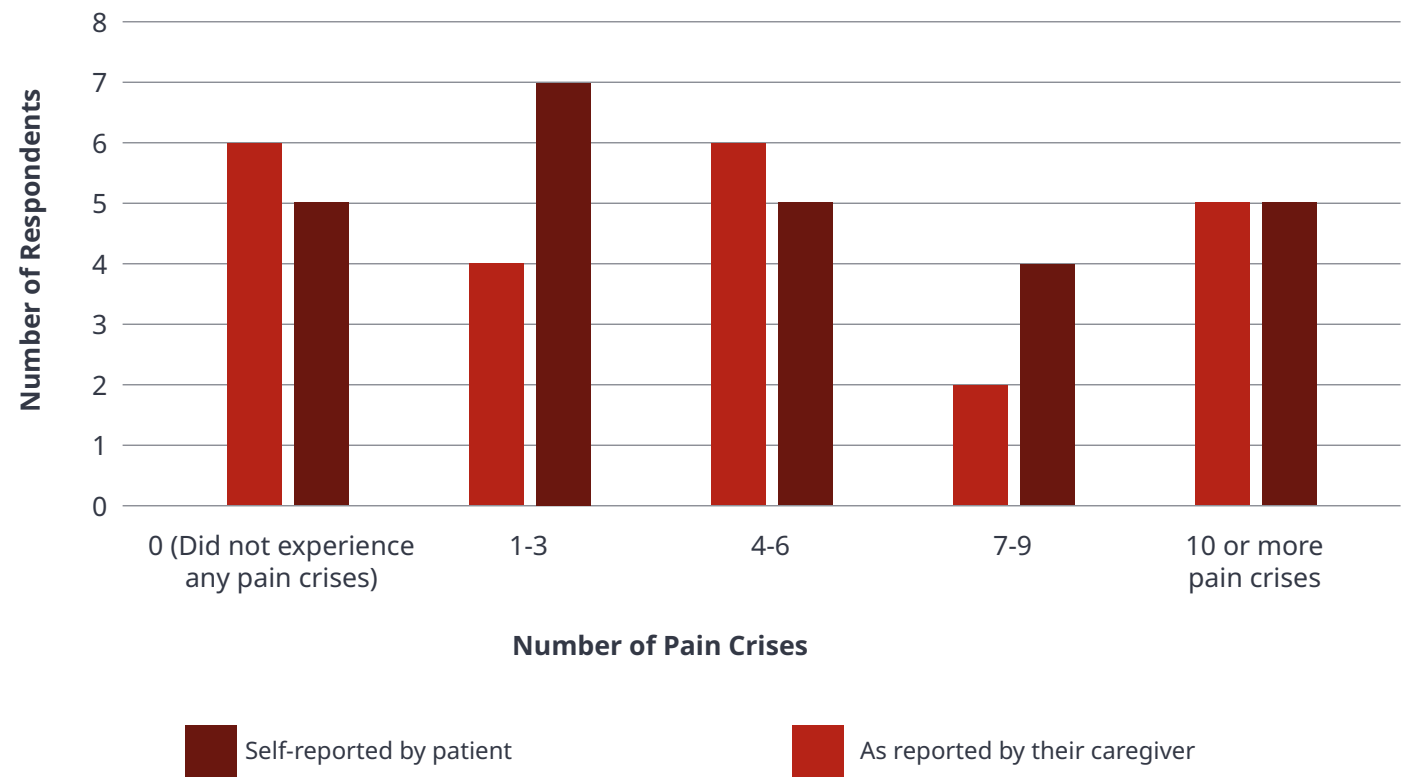
Self-reported by Patient



As Reported by Caregiver



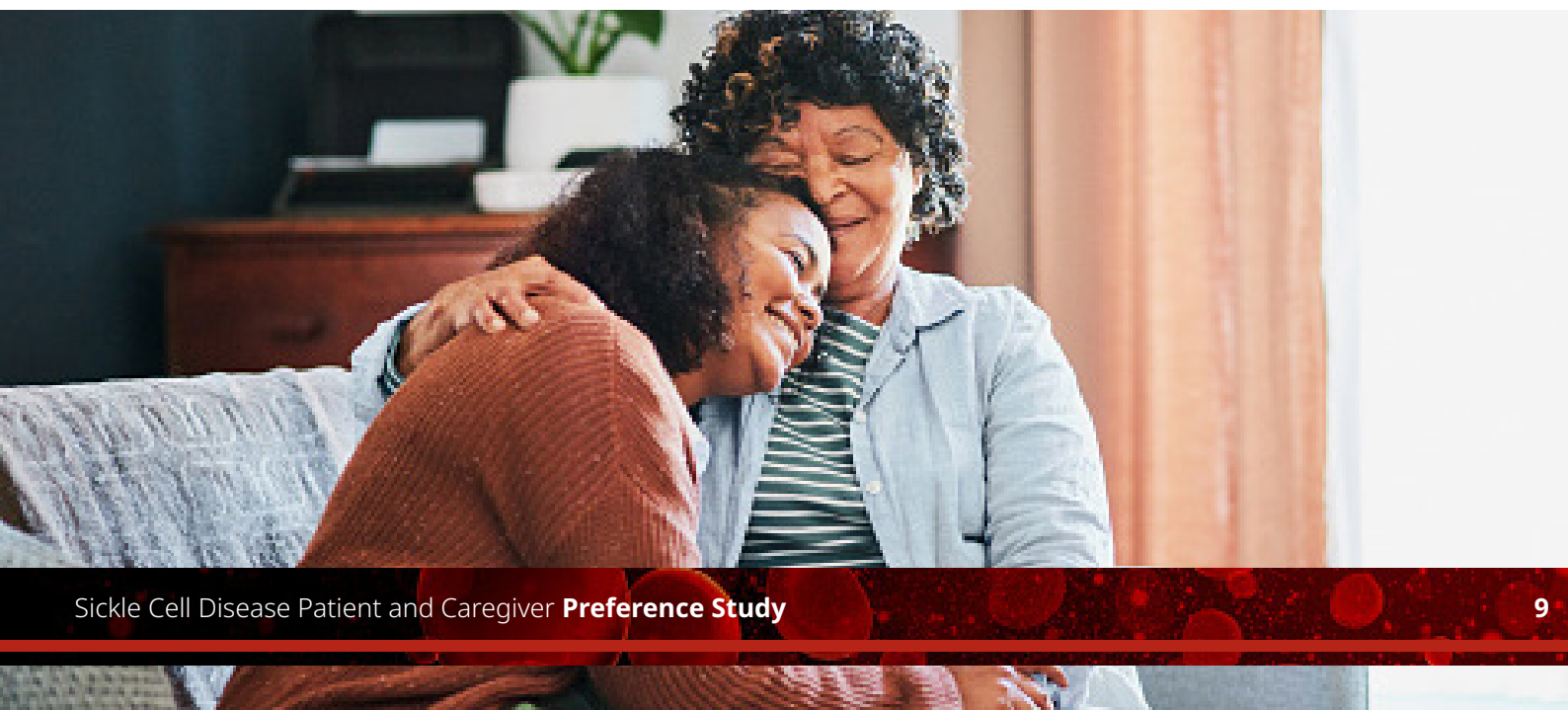
Number of Pain Crises in the Last Year



What were the results of the study?

The top 10 items according to patients are listed in the table below. The importance scores that patients and caregivers gave to those items are shown next to the item name. Higher importance scores are in darker shades of gray.

Top 10 Most Important Items to Patients	Average Importance Score from Patients	Average Importance Score from Caregivers
Reduce the risk of having organ damage (including damage to the liver, heart, kidneys, gallbladder, or eyes)	6.3	5.3
Shorten the length of pain crises	5.6	5.3
Reduce the risk of having a stroke (including strokes that don't have any noticeable symptoms)	5.2	4.7
Reduce the risk of severe joint problems in the hip or shoulder (avascular necrosis/AVN)	4.8	3.7
Reduce the frequency of pain crises that require medical attention (that cannot be managed at home)	4.6	5.8
Reduce the frequency of pain crises of any severity (whether it is managed at home or at the emergency room)	4.6	5.6
Reduce the risk of acute chest syndrome (when sickled cells clump together in the lungs)	4.6	4.8
Reduce the level of pain experienced when having a pain crisis	4.5	5.9
Less chance of having myelosuppression (bone marrow problems cause fewer red blood cells, fewer white blood cells, and fewer platelets than normal and can cause fatigue, shortness of breath, bleeding, and infections)	4.2	3.8
Reduce daily chronic pain	4.1	4.3



Top 3 Most Important Items to Patients



Reduce the risk of having organ damage (including damage to the liver, heart, kidneys, gallbladder, or eyes)



Shorten the length of pain crises



Reduce the risk of having a stroke (including strokes that don't have any noticeable symptoms)

Top 3 Most Important Items to Caregivers



Reduce the level of pain experienced when having a pain crisis



Reduce the frequency of pain crises that require medical attention (that cannot be managed at home)



Reduce the frequency of pain crises of any severity (whether it is managed at home or at the emergency room)

If you want to see the full set of importance scores from patients and caregivers, go to the last page of this document.

What will Pfizer do with these results?

Pfizer will use this information as it determines which items to measure in clinical trials of potential sickle cell disease treatments.

Thank you!

Pfizer wouldn't have learned this information without the help of Sick Cells and the community of sickle cell disease patients and caregivers. We would especially like to thank Ashley Valentine, Maggie Jalowsky, Abby Tainter, and Emma Andelson from Sick Cells, Maryn Smith from Ernst and Young, and all the patients and caregivers who took the time to provide their perspectives by completing the survey.

Importance Scores by Group

