WE CAN CHANGE SCD

OUR PARTNERSHIP IS OUR STRENGTH

Treating sickle cell disease (SCD) is more than just treating acute complications. Treating SCD today means seeing the whole person and considering their lifelong goals.

Abena O. Appiah-Kubi, MD, MPH

Jennifer, **living with SCD**

Together we can improve the care of SCD.

At bluebird bio, we believe that partnership can change the way people think about SCD and feel that partnership is the way forward—partnership between physicians and people living with SCD, partnership between the SCD community and industry, and partnership that includes all of us.

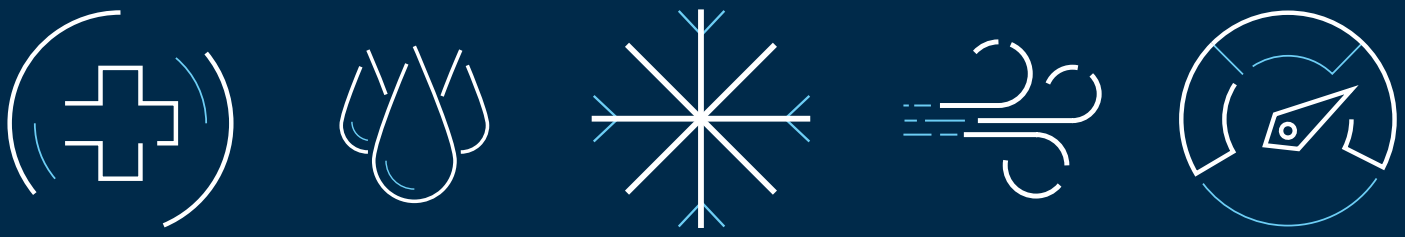
“

Patients want somebody they can trust. We have to hold our hands out. We have to come honestly. We have to come patiently. It’s why we need partnerships based on trust, so we can get to a place where we can ***move forward together***.

WALLY R. SMITH, MD

SCD is an unpredictable lifelong genetic disease characterized by *painful* vaso-occlusive events (VOEs)[1,2]

VOEs can occur without warning and can have a severe impact on quality of life. They can be triggerd by a mutlitude of factors that cause the deoxygenation of hemoglobin Sickle (HbS), such as[1,2]:



Illness | Dehydration | Cold temperature | Increased wind speed | Higher barometric pressure

THERE ARE SERIOUS CONSEQUENCES ASSOCIATED WITH ACUTE, RECURRENT VOEs

- More than 9 *out of* 10 *hospital admissions* for people with SCD are due to acute pain crises (the most common VOE)[2,3]
- 1 *out of* 2 *people with SCD* (50%) will experience cerebral vasculopathy by the age of 14[4,5]
- Approximately 30% *of people with SCD* can or will have acute chest syndrome (ACS)[6]
- Anywhere from 7% *to* 30% *of young children* with SCD will experience splenic sequestration[1,7,8]
- 1 *out of* 4 *adults with SCD* (25%) will experience a stroke by the age of 45[1,5]

It's time to *rethink the standard of care* for people living with SCD by considering the long-term impact of VOEs and refocusing on ongoing, lifelong partnerships in care

Create a *partnership* with people living with SCD to address the pain resulting from VOEs

PUBLISHED GUIDELINES RECOMMEND USING AN INDIVIDUALIZED OR SCD-SPECIFIC PROTOCOL FOR PAIN MANAGEMENT[9,10]

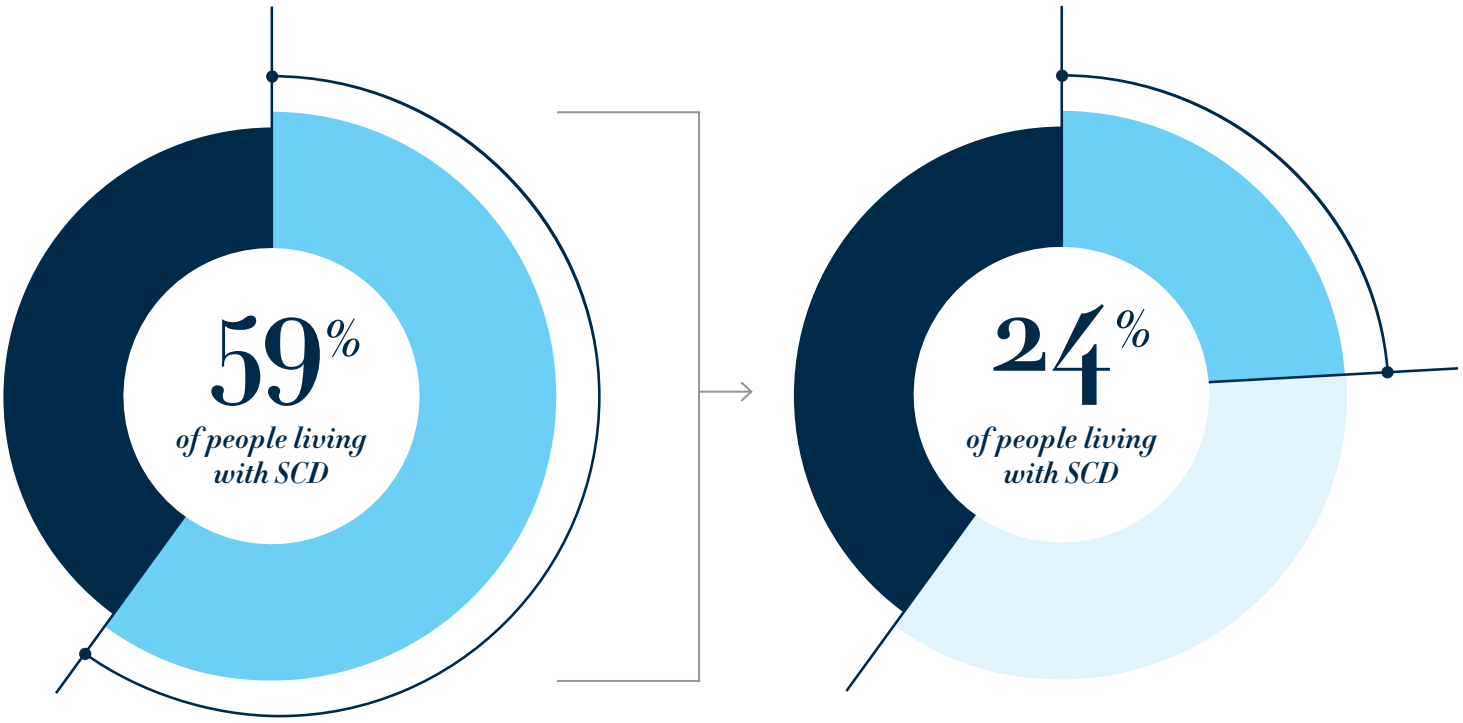
Pain management should include parenteral opioids for severe pain, administered in a timely manner, guided by:

- An individualized prescribing and monitoring protocol written by the patient and their healthcare team
- An institutional SCD-specific protocol

> We try to *form a relationship from the very beginning* and let the families understand that we're here as somebody to help them take care of their child as they grow.
>
> **ABENA O. APPIAH-KUBI, MD, MPH**

Repeated vaso-occlusion, infarction, and chronic hemolytic anemia lead to *organ dysfunction* and *failure*[11]

A MAJORITY OF PEOPLE LIVING WITH SCD HAVE END ORGAN DISEASE DUE TO CHRONIC COMPLICATIONS, AND ALMOST 24% HAVE DAMAGE IN MULTIPLE ORGANS[12]



Have end organ disease due to chronic complications

Have damage in multiple organs

Long-term and ongoing *complications* of SCD

Sickle cell retinopathy	38% *and* 76% prevalence, depending on genotype; can lead to a loss of visual acuity if left untreated. Because it is often asymptomatic, sickle cell retinopathy requires retinal screening.[1,13]
Pulmonary hypertension	6% *to* 11% prevalence, with an increase in severity related to thromboembolic disease, obstructive sleep apnea, hypoxemia, and an increased risk of death. Pulmonary hypertension results from the effects of hemolysis, which promote clinical complications of vasculopathy.[14,15]
Cardiovascular complications	Complications increase with age and are a common cause of death for people living with SCD.[16]
Cerebral vasculopathy	Overt stroke occurs in 11% of patients younger than 19 and in 24% of adults by age 45; prevalence of silent infarcts increases with age from 27.7% before age 5 to 53.5% by age 30 and is associated with neurocognitive dysfunction.[17,18]
Cholelithiasis	23% *to* 66% prevalence in the SCD population.[19]
Renal dysfunction	One of the most common complications of SCD, which includes renal insufficiency, hyposthenuria, proteinuria, and renal failure requiring dialysis; 1 study reported that the mean time to death in end-stage renal disease is 4 years.[20,21]
Avascular necrosis of femoral head	Associated with crippling pain in affected people; 22% prevalence, with 23% of these people requiring a hip replacement at a median age of 36, according to a state-wide analysis of 6237 patients with SCD.[22]
Recurrent, chronic leg ulcer	18% prevalence in adults with SCD; this is a painful and debilitating complication of SCD and is linked to stroke, ACS, thrombosis, renal disease, and elevated tricuspid jet regurgitation.[23,24]
Silent stroke (asymptomatic cerebral infarction)	The most common cause of neurologic injury in adults and children with SCD, associated with diminished IQ scores and poor academic performance; an increase in the prevalence of silent infarcts is highly associated with age.[18]
Depression and anxiety	Affects ~35% of people living with SCD and is associated with worse quality-of-life outcomes; the prevalence is ~5 times greater than that of the general population.[25]
End organ disease	Occurs in 59% of adults due to chronic complications, with multiple organs or multiple systems often being affected.[12]

Treatment of SCD needs to *go beyond pain management*

Proactively *screen and manage* the chronic complications of SCD

GUIDELINES ALSO PROVIDE SPECIFIC RECOMMENDATIONS FOR THE MANAGEMENT OF CHRONIC COMPLICATIONS OF SCD, INCLUDING[26]:

- Avascular necrosis
- Pulmonary hypertension
- Priapism
- Renal complications
- Ophthalmological complications
- Leg ulcers

> Pain is the reason patients come for care. I follow up [treating pain] with, 'Now that I have your pain under control, *can you listen* to me and *can you trust me* to work with you on a more long-term, comprehensive approach to your care?'
>
> **WALLY R. SMITH, MD**

People living with SCD face many *challenges* beyond the disease

SCD AFFECTS THE ABILITY OF BOTH CHILDREN AND ADULTS TO WORK OR TO ATTEND SCHOOL[27-29]

In **ADULTS** with SCD:

UNEMPLOYMENT RANGED FROM

28% to 52%

COMPARED WITH THE NATIONAL UNEMPLOYMENT RATE OF APPROXIMATELY 4%–10% (2002–2010)[27,30]

INCREASES IN SCD SEVERITY **DECREASE** WORK PERFORMANCE AND ATTENDANCE[27,28]

In a survey of **ADOLESCENTS** with SCD:

60%

REPORTED THAT SCD **INTERFERED** WITH THEIR SCHOOL PERFORMANCE[29]

40%

REPORTED **BEING RETAINED** AT LEAST 1 GRADE LEVEL (VS 24% NATIONALLY)[29]

People living with SCD also face *implicit racial bias* in the healthcare setting[31,32]

BLACK PEOPLE LIVING WITH SCD REPORTED:

25%

LONGER WAIT TIMES

TO SEE A PHYSICIAN COMPARED WITH THE GENERAL POPULATION[31]

Black children living with SCD also experienced greater delays in receiving analgesic treatment compared with children with long-bone fractures (15% of whom were Black).[32]

Start a discussion with your patients to understand if they have any difficulties with work or school, are experiencing racial bias, or have ***feelings of medical mistrust*** toward the healthcare community

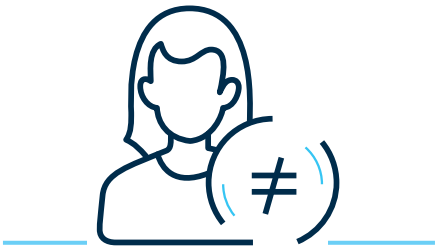
Identify and address *challenges* beyond SCD

ASK ABOUT YOUR PATIENTS' EMOTIONAL MINDSET AS WELL AS THEIR SOCIAL AND EDUCATIONAL SITUATIONS—UNDERSTANDING THESE FACTORS CAN LEAD TO OPTIMAL SCD CARE

Start a **CONVERSATION** with your patients to:

Understand their perspective about their disease, emotional mindset, and social situation

Identify available mental health and community services



Understand if they have experienced health-related racial bias and consider how to address this within the healthcare setting

> Doctors should be thinking about the patient's feelings. Your patient is not only your patient. Your patient is a *regular person* just like you.
>
> **JENNIFER, LIVING WITH SCD**

We can bring change to SCD care by seeing the whole person and by considering their circumstances

- Take a proactive approach to pain management by working with patients to develop an individualized pain protocol
- Consult the published guidelines and recommendations to actively screen for and manage the long-term complications of SCD
- Open up a dialogue with patients about any struggles they face beyond the disease and look at setting long-term goals for treatment and building a partnership
- Get to know your patients in order to build a partnership based on trust and open communication

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Let's change the way we view SCD.

Visit ChangeForSCD.com

Despite all the complications sickle cell disease can cause, our role and our goal is to *partner with patients* to try to prevent as much from happening as we can. The future looks bright.

ABENA O. APPIAH-KUBI, MD, MPH

Actual adult and pediatric physicians who treat SCD (as well as their patients who are living with SCD) helped inspire and inform the content of this brochure. Some of these patients and physicians are depicted throughout.

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