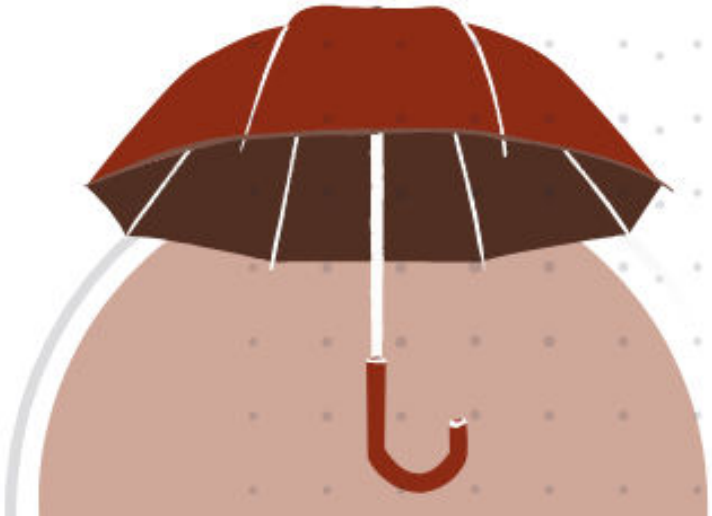
2022 COVERAGE FOR SCD SUMMIT

SICK CELLS

2022 COVERAGE FOR SCD SUMMIT

Improving Equity and Affordability of SCD Therapies

Best Practices in Benefit Strategies and Payer Management



Welcome

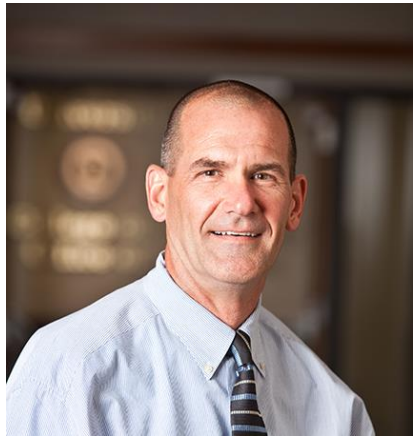
Terry Richardson, PharmD, BCACP
VP, Education Strategy and Development
Impact Education, LLC®

Faculty

Francesca Valentine, MSN, RN
Mother of a Winged SCD Warrior, Marqus

Shivi Jain, MD
Rush University Medical Center

Emily Tsiao, PharmD
Premera Blue Cross



Terry Cothran, DPh
Oklahoma Health Care Authority

Learning Objectives

- Review the evolving SCD treatment landscape and real-world and evidence-based formulary management strategies for appropriate SCD treatments
- Identify where patients with SCD typically incur high healthcare costs and how to reduce the economic burden of SCD
- Discuss how health plans can provide patient-centered care and services that support appropriate treatment coverage and access for patients with SCD
- Apply ethical decision-making and promote greater health equity in the clinical management of SCD

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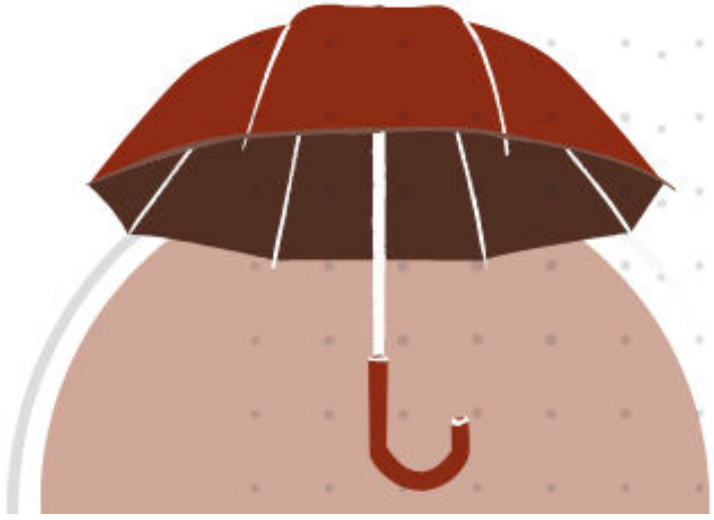
The Patient Perspective

Presented by **Francesca Valentine, MSN, RN**



Introductions

- **Francesca Valentine, MSN, RN**

  Mother of a Winged SCD Warrior, Marqus

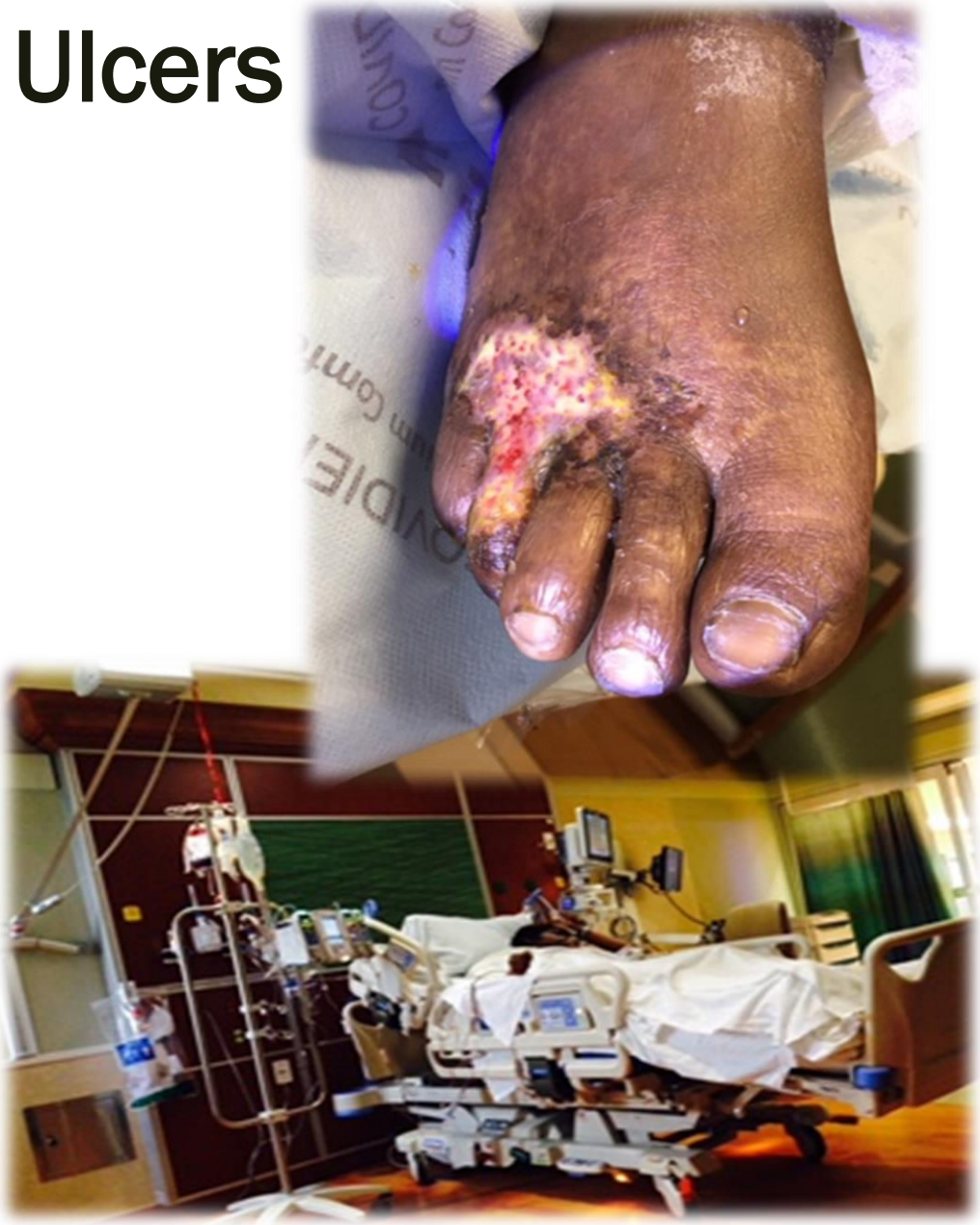
- Marqus was a patient person who devoted his life to understanding others, uplifting his family and friends, and **spreading awareness about sickle cell disease.**

A Look into One Event: Leg Ulcers

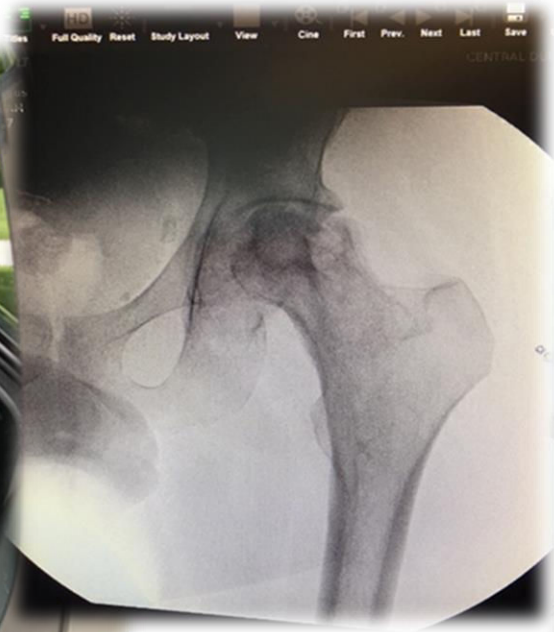
- The pre-session shares about Marqus' journey and highlights one life-debilitating complication of SCD: **leg ulcers**

- In July 2015, Marqus was admitted to the hospital for **sepsis due to infected wounds and sickle crisis**.
  - He spent 30 days in the hospitals
  - Direct medical bills **totaled over $200,000**.

- Complications continued after discharge. Marqus was re-admitted two additional times:
  - September hospitalization **totaled over $60,000**.
  - October hospitalization **totaled over $6,000**.



Debilitating Complication



Leg Ulcers: The True Cost and Impact

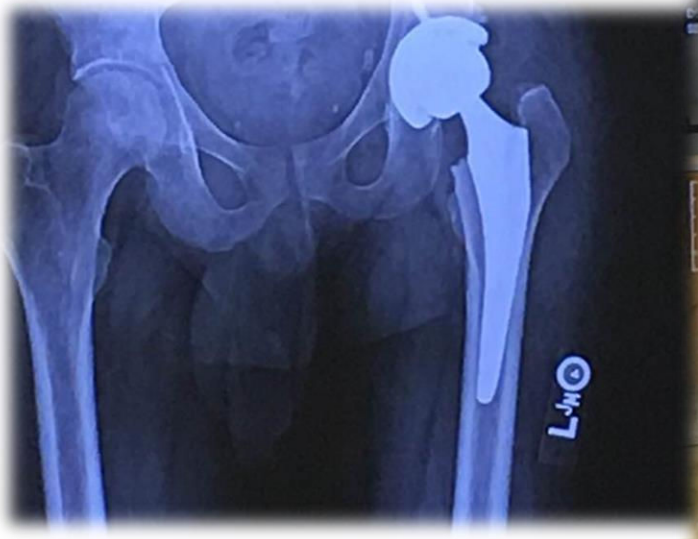
In this one event,

- Marqus had hit his $10,000 maximum **out-of-pocket expenses**.

- $720 each month from his Social Security Insurance benefit did not cover these expenses; **family members had to share costs.**

- **Indirect costs** like transportation (gas), food, medical supplies, shoes and time off from work contributed substantially.
  - Marqus' dad was an electrician and an hourly worker, he didn't **receive paid sick leave**, making any time away from work an instant financial loss.
  - As a nurse, I used **480 hours of FMLA** unpaid leave.

Restoring Dignity and Quality of Life



- SCD is a family affair. Emotional fortitude is required for the entire family.
- Life-limiting complications require intense management.
- Patients must be partners in their care.

In Loving Memory
Marqus Valentine
7/25/1983 – 6/22/2020

"Marqus hopes that the next generation of families living with SCD **will not have to suffer** so much. He hopes that future patients **will have medication to treat their SCD** and can have **access to high-quality care**, access to providers who are educated about their condition, access to **robust coverage for treatments** and therapies, they can live long lives."

-Ashley Valentine, Sister of Marqus

Read her words at: ISPOR Spotlight

"Our Sickle Cell Normal: The True Cost to the Patient of Sickle Cell Disease"

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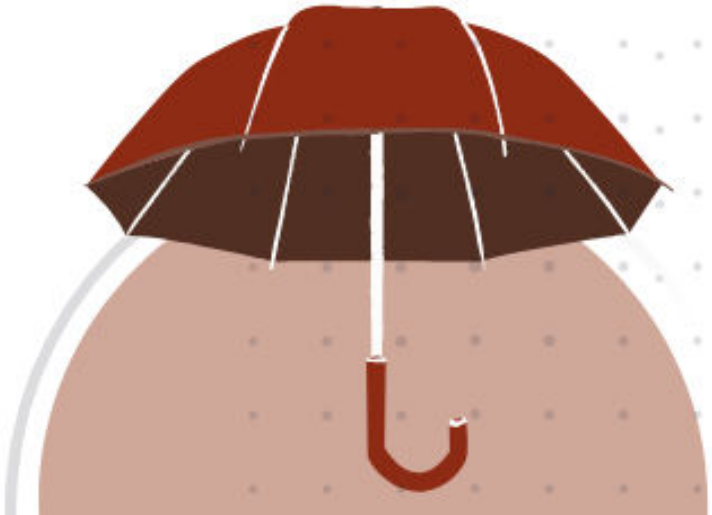
PAYER SESSION

Overview of SCD Treatments and the Importance of Patient-Centered Care

Shivi Jain, MD
Assistant Professor of Medicine
Director Adult Sickle Cell Program
Division of Hematology/Oncology/Cell Therapy
Rush University Medical Center, Chicago, IL



James B Herrick

Ernest E. Irons

EXAMINATION OF BLOOD.

Case Number

Name of Patient Noel

Date 12/31 ,190 X

Room or Ward 7

MACROSCOPICAL AND QUANTITATIVE.

Appearance pale

Coagulability

Erythrocytes per cu. mm. (Thoma Zeiss). 2,880,000

Leucocytes per cu. mm. (Thoma Zeiss). 15,250.

Hemoglobin (Von Fleischl) 50% Dare Corrected

Specific gravity

Color index

Volume index

per cu. mm. 3700, cells very small reproductive nucleated reds?) (red count prepara-

MICROSCOPICAL.

Fresh Specimen.

Erythrocytes—Color

Shape very irregular many elongated forms

Size irregular – ~~average size~~ average size about normal

Rouleaux formation

Leucocytes—Apparent increase in number

Ratio of granular to non-granular

Fibrin

Blood-platelets

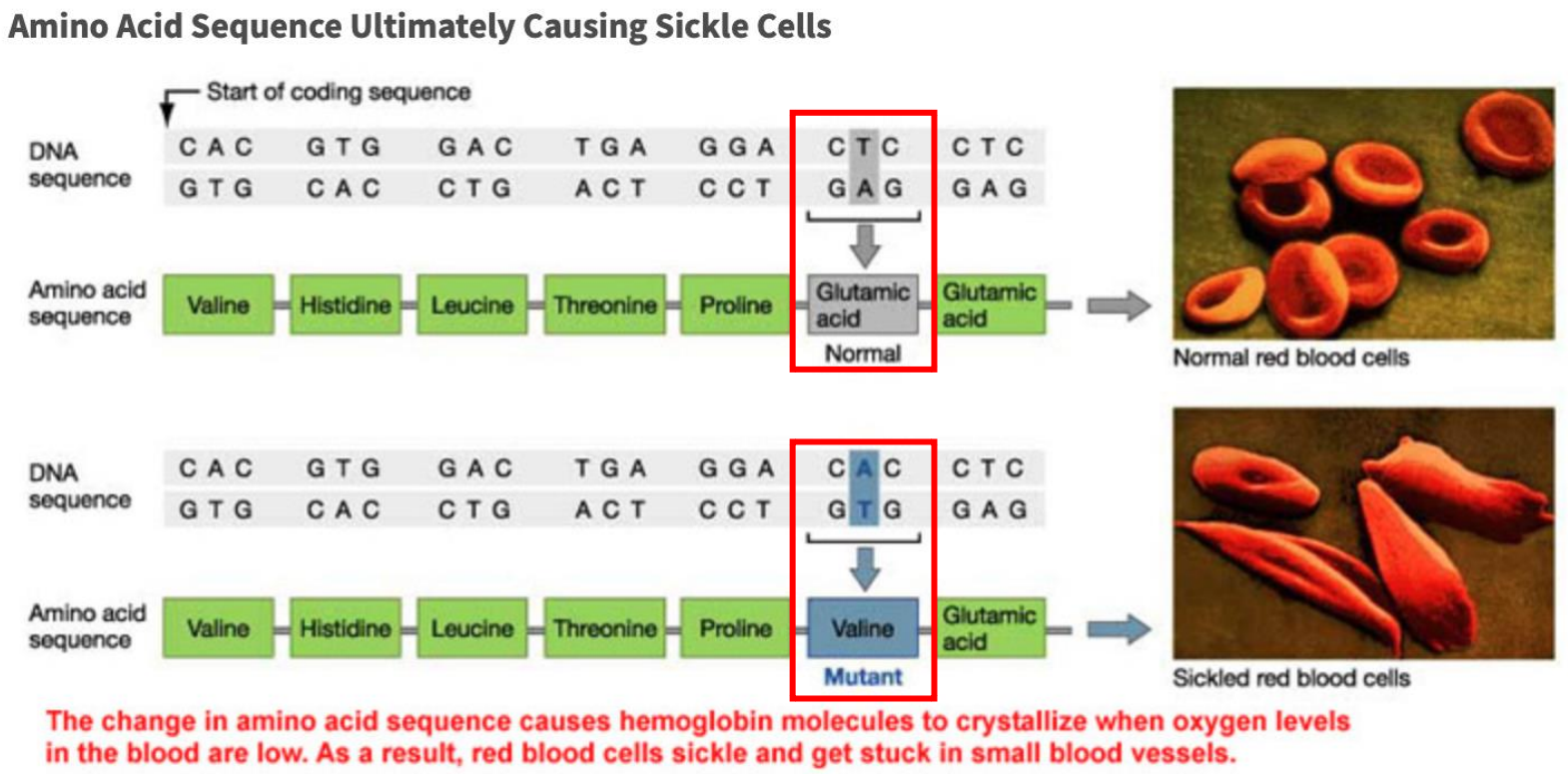
Pigment

History of Sickle Cell Disease

The Rush Connection

Etiology

- Sickle cell disease is an autosomal recessive hemolytic anemia that occurs due to a single point mutation in the beta globin gene (HBB).
- This results in substitution of valine for glutamic acid at position 6 on the beta helix.



Epidemiology

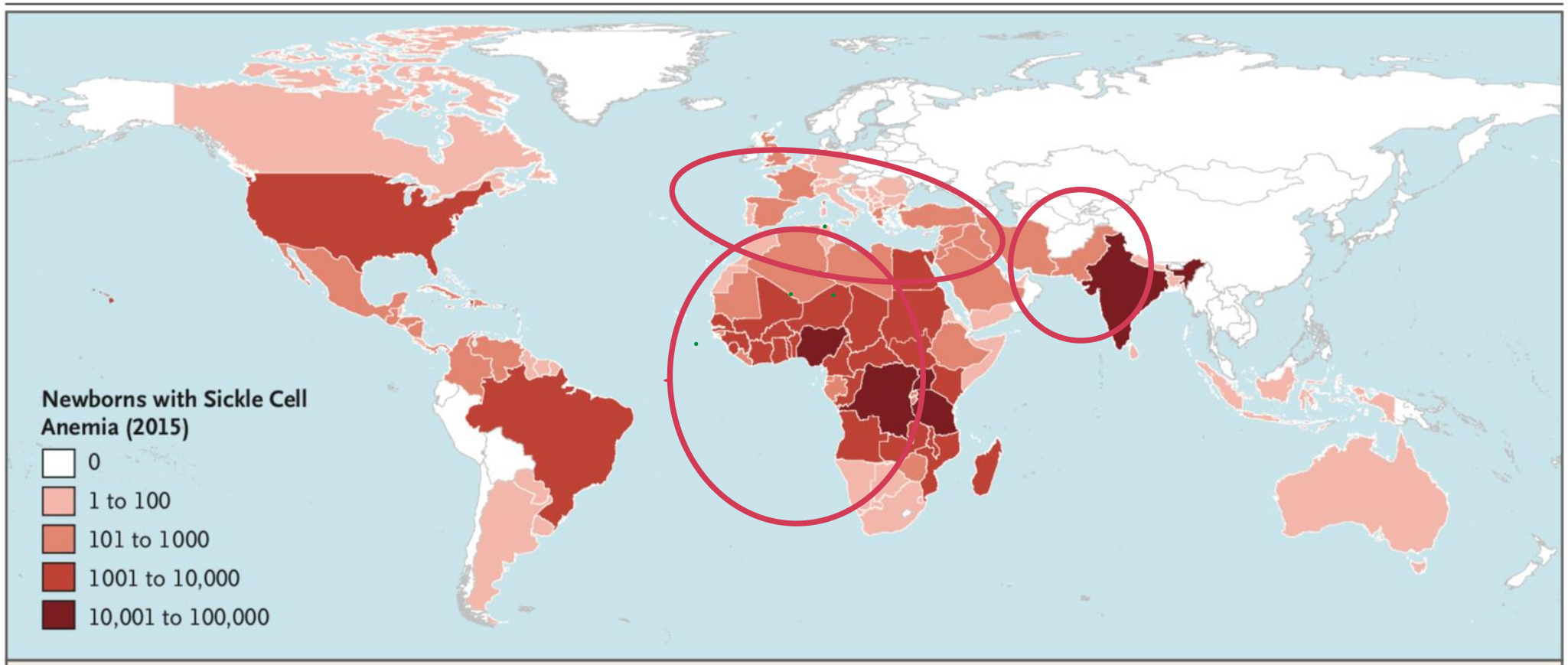
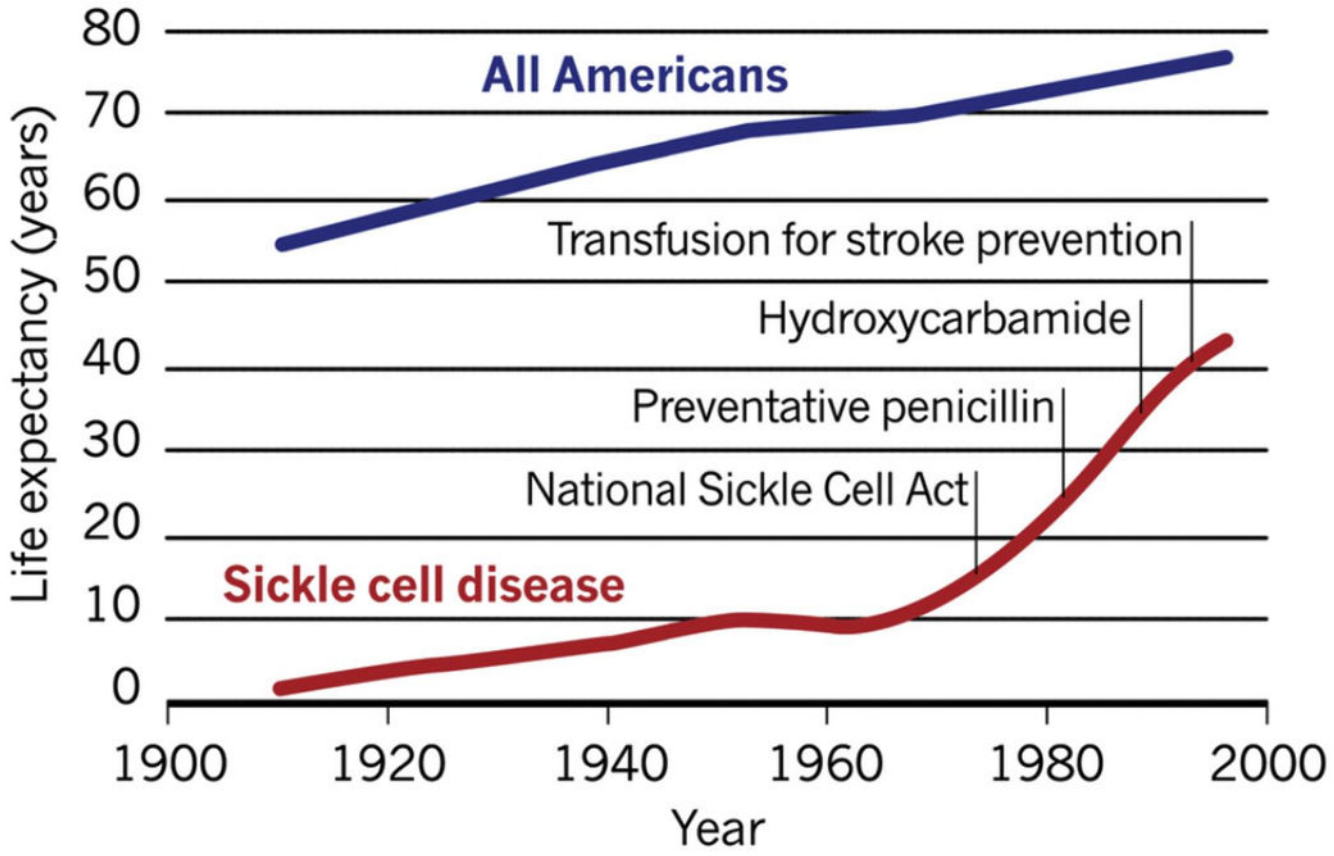


Figure 1. Number of Newborns with Sickle Cell Anemia in Each Country in 2015.

Data are based on estimates from Piel et al.[1] Alaska is shown separately from the rest of the United States.

Frédéric B. Piel, Ph.D., Martin H. Steinberg, M.D., and David C. Rees, F.R.C.P.C.H. *NEJM.* 376;16 April 20, 2017.

Sickle Cell Disease: Life expectancy



Pathology 2017 49, 1-9DOI: (10.1016/j.pathol.2016.10.002)
Haematologica. 2007;92(7): 905-912.
Blood. 2010; 115(17):3447-3452.
Survival in adults with sickle cell disease in a high- income setting. *Blood*. 2016;128(10): 1436-1438.
Factors associated with survival in a contemporary adult sickle cell disease cohort. *Am J Hematol*. 2014;89(5): 530-535.

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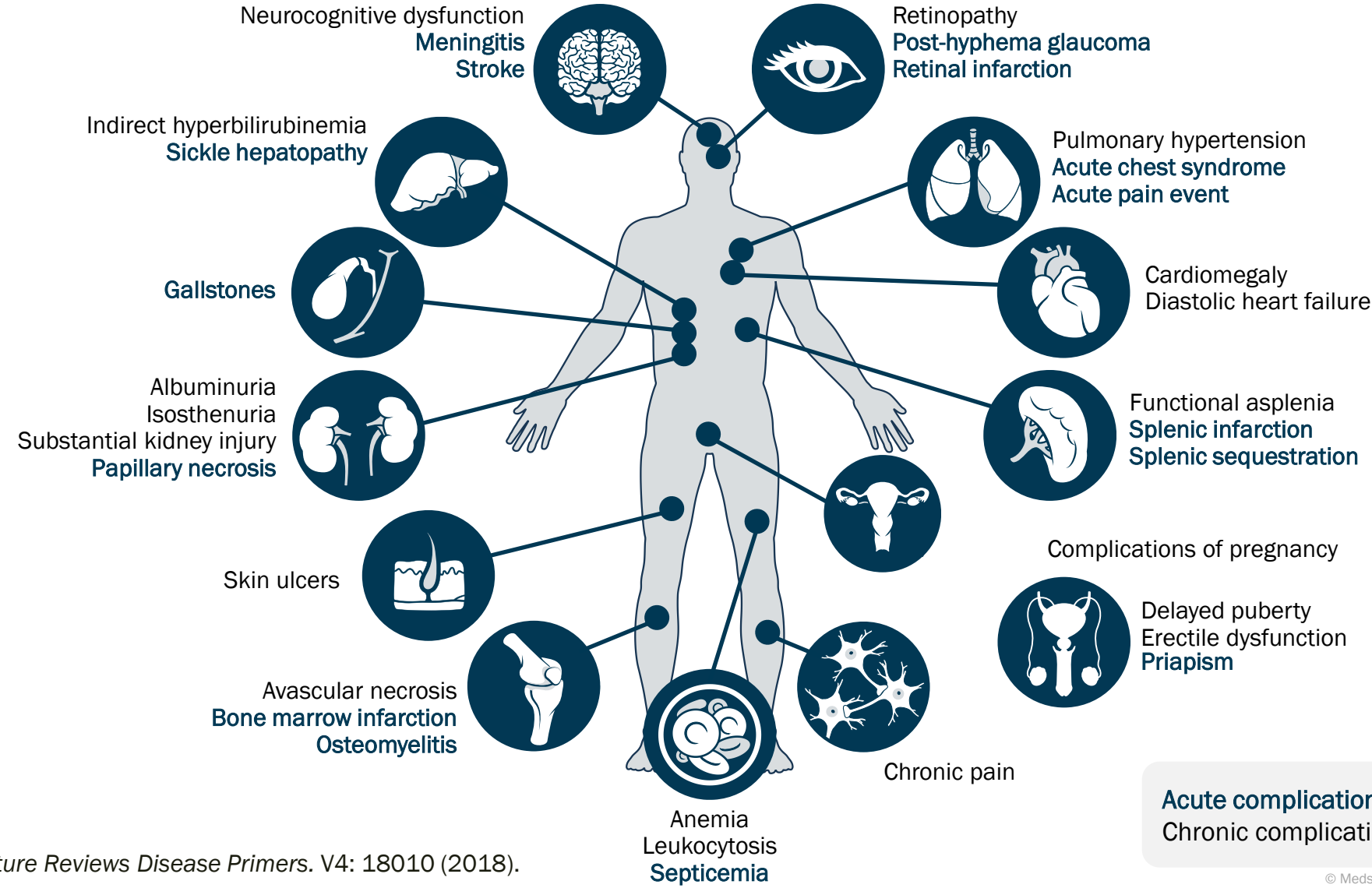
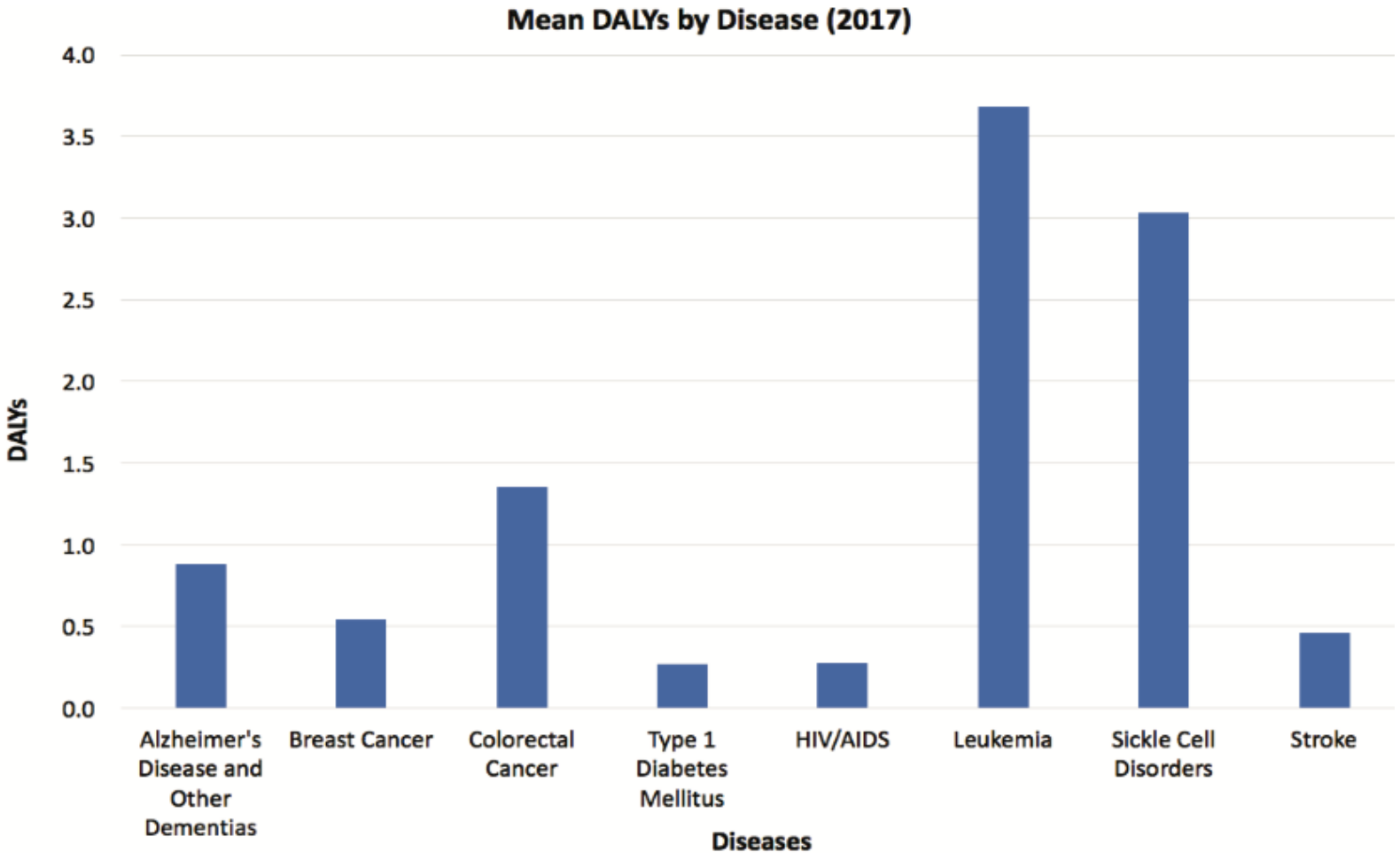
SCD is a Multi-System Disease



Acute complications
Chronic complications

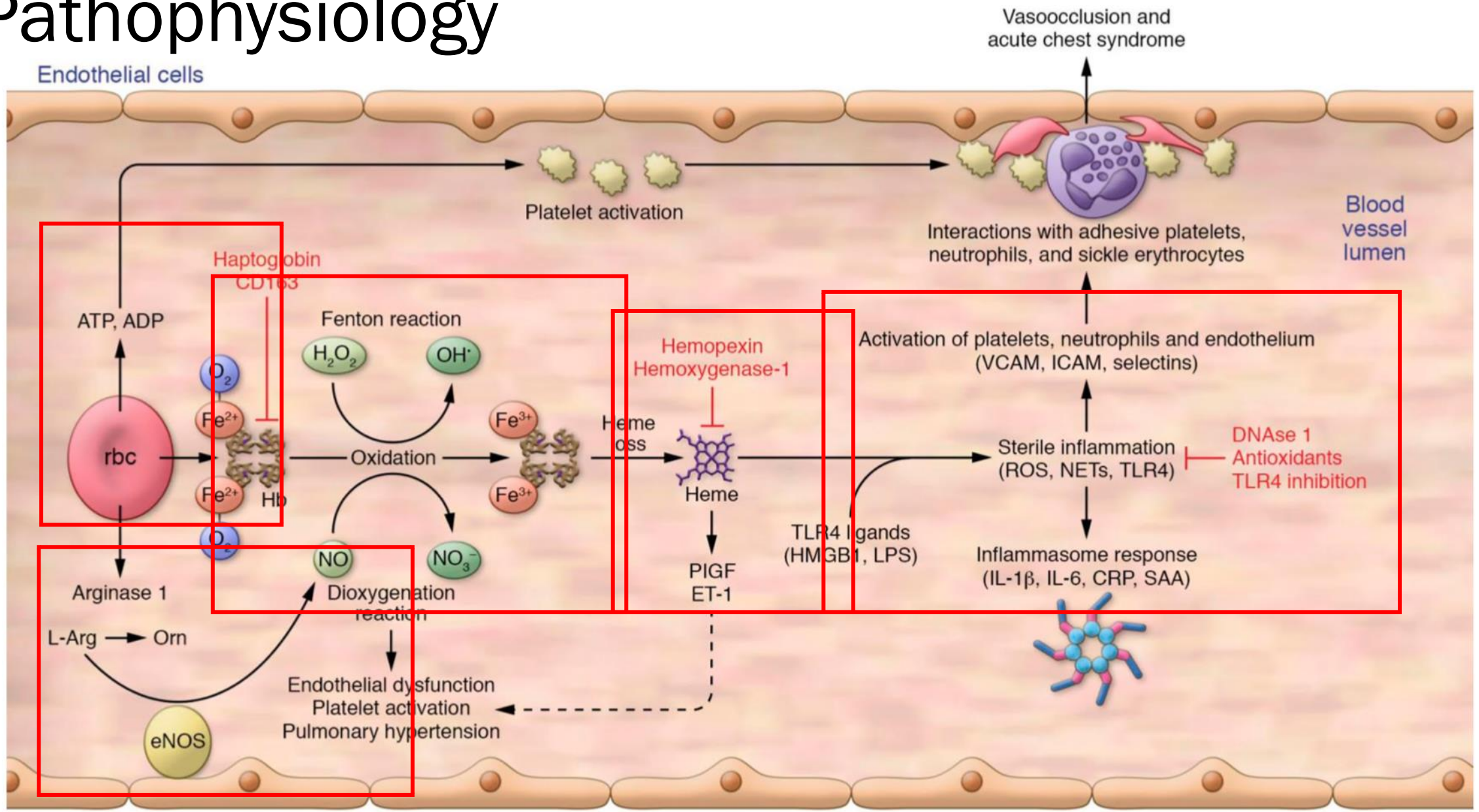
Kato GJ, et al. *Nature Reviews Disease Primers.* V4: 18010 (2018).

Health-Related Burden



National Academies of Sciences, Engineering, and Medicine 2020. *Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action*. Washington, DC: The National Academies Press. https://doi.org/10.17226/25632.

Pathophysiology



Ther Adv Hematol. 2020, Vol. 11: 1–15.

Sickle Cell Disease Treatments

Standard of care

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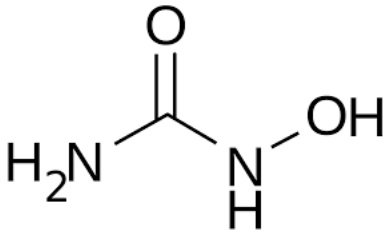
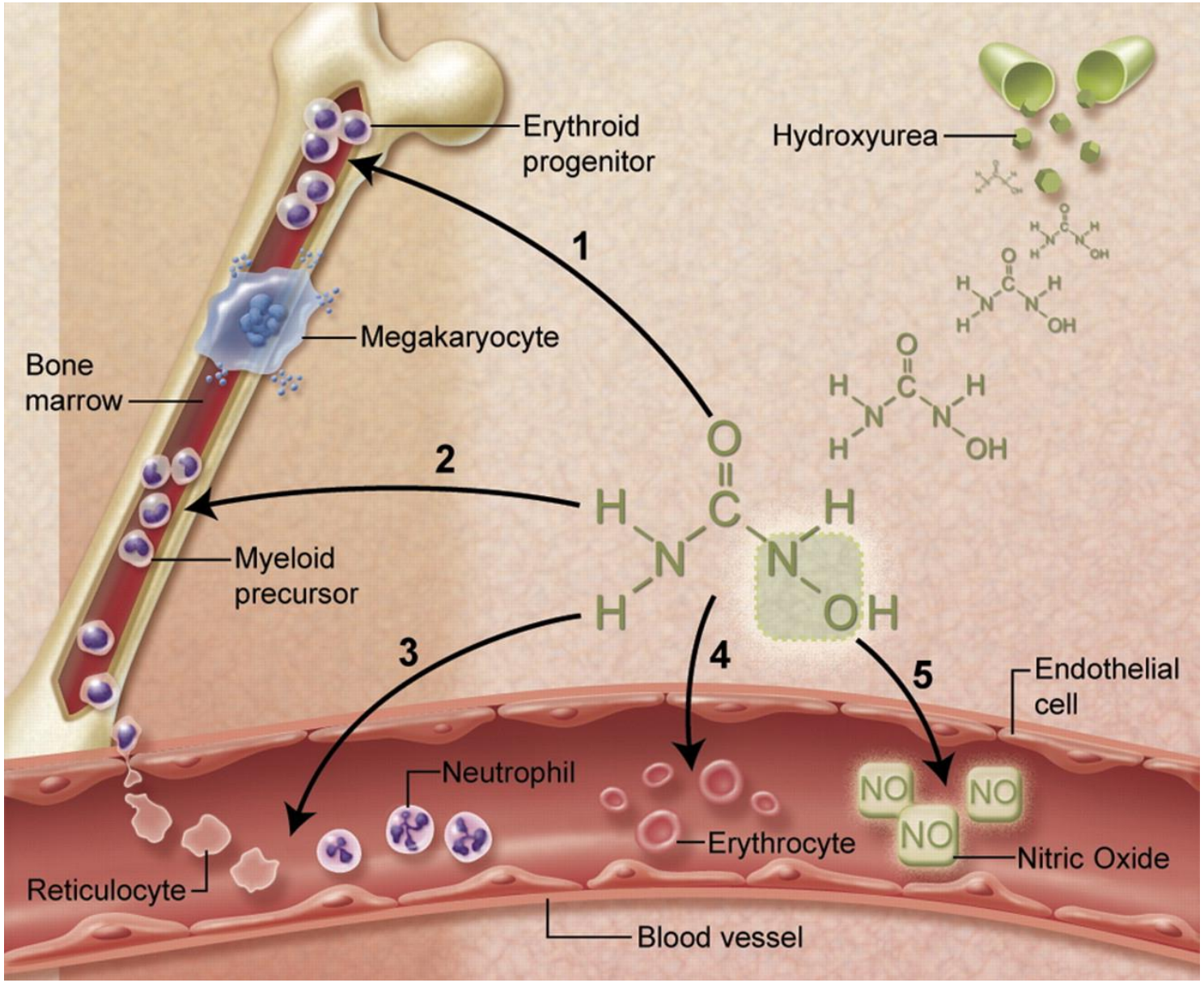
Hydroxyurea





Russell E. Ware, How I use hydroxyurea to treat young patients with sickle cell anemia, *Blood.* 2010.

American Society of Hematology
Helping hematologists conquer blood diseases worldwide

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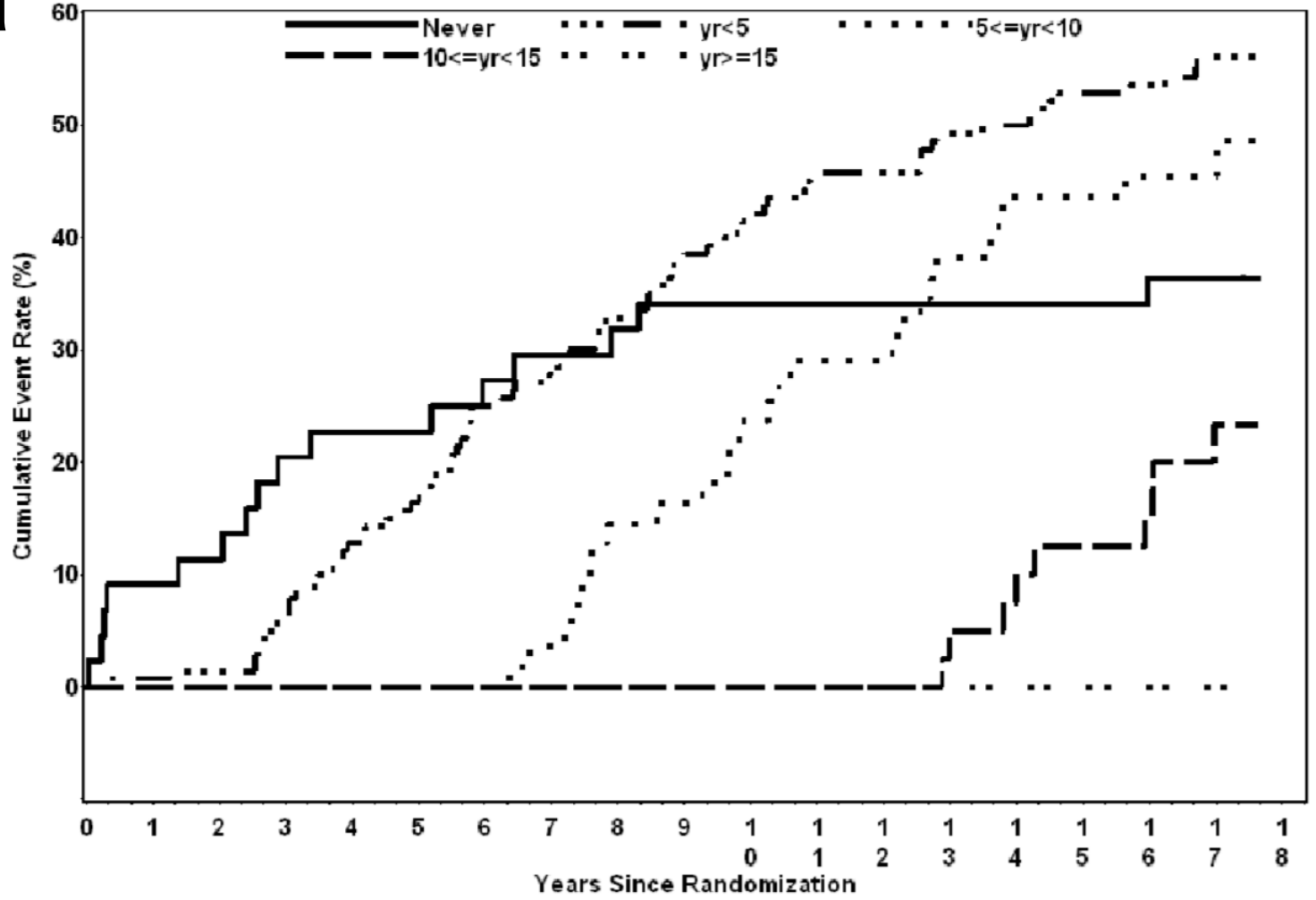
Hydroxyurea



The Risks and Benefits of Long-term Use of Hydroxyurea in Sickle Cell Anemia: A 17.5 Year Follow-Up

Am J Hematol. 2010 June; 85(6): 403–408. doi:10.1002/ajh.21699.

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Hydroxyurea

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Hydroxyurea Treatment Recommendations

Recommendations
1. Educate all patients with SCA and their family members about hydroxyurea therapy. (See consensus treatment protocol on page 145). ***(Consensus–Panel Expertise)***
2. In adults with SCA who have three or more sickle cell-associated moderate to severe pain crises in a 12-month period, treat with hydroxyurea. ***(Strong Recommendation, High-Quality Evidence)***
3. In adults with SCA who have sickle cell-associated pain that interferes with daily activities and quality of life, treat with hydroxyurea. ***(Strong Recommendation, Moderate-Quality Evidence)***
4. In adults with SCA who have a history of severe and/or recurrent ACS, treat with hydroxyurea.* ***(Strong Recommendation, Moderate-Quality Evidence)***
5. In adults with SCA who have severe symptomatic chronic anemia that interferes with daily activities or quality of life, treat with hydroxyurea. ***(Strong Recommendation, Moderate-Quality Evidence)***
6. In infants 9 months of age and older, children, and adolescents with SCA, offer treatment with hydroxyurea regardless of clinical severity to reduce SCD-related complications (e.g., pain, dactylitis, ACS, anemia). ***(Strong Recommendation, High-Quality Evidence* for ages 9–42 months;** ***Moderate Recommendation, Moderate-Quality Evidence* for children >42 months and adolescents).** Note: The panel intentionally used the term "offer" realizing that patients' values and preferences may differ particularly considering treatment burden (e.g., laboratory monitoring, office visits), availability of drug in a liquid form, and cost. Therefore, the panel strongly encourages shared decisionmaking and discussion of hydroxyurea therapy with all patients.

U.S. Department of Health and Human Services
National Institutes of Health
National Heart, Lung, and Blood Institute

Expert Panel Report, 2014

Need for Novel Therapies

- Recurrent VOCs continue to be associated with disease severity and mortality.
- Younger age of death (55.8 years *versus* 66.2 years; $p = 0.04$) higher risk ratio (RR) of death (RR=2.68; p=0.03) among individuals with high rates of VOCs.
- In this study, **41%** of participants reported Hydroxyurea use, and **39%** reported >10 RBC transfusions during their lifetime.

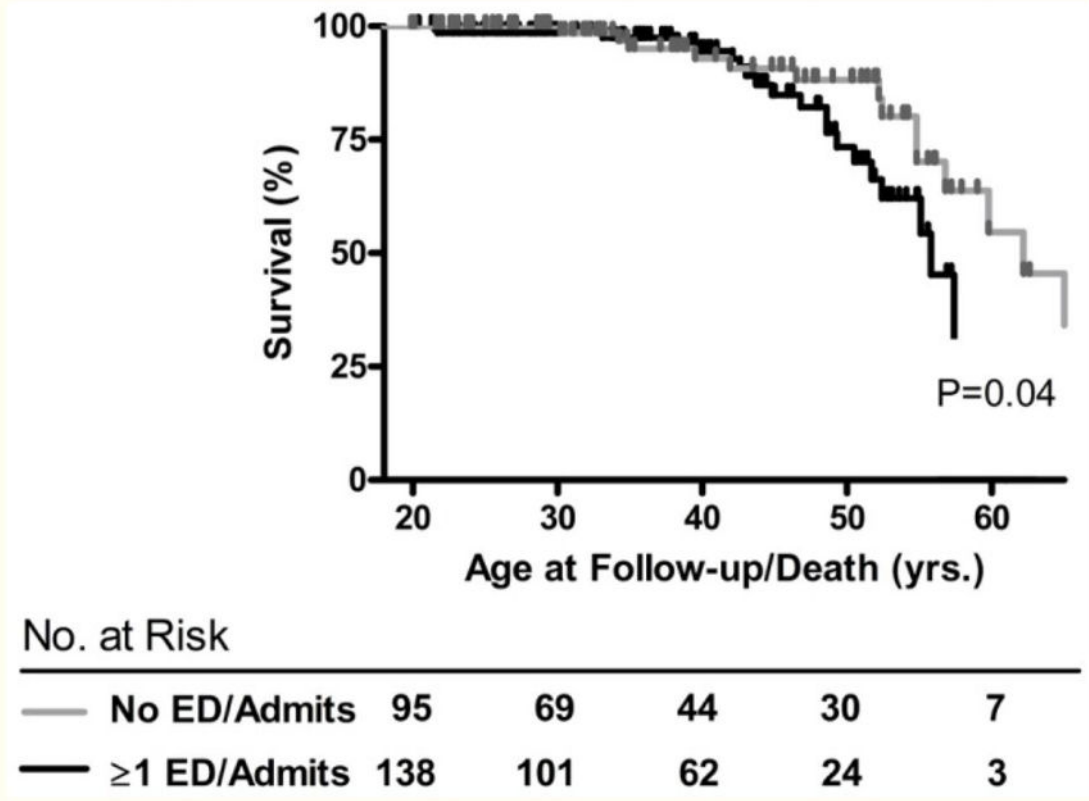

Figure 1

Kaplan Meier (KM) curve showing survival in sickle cell anemia by severe pain crises requiring an ED visit/ hospitalization in past year.

PLoS One. 2013; 8(11): e79923.

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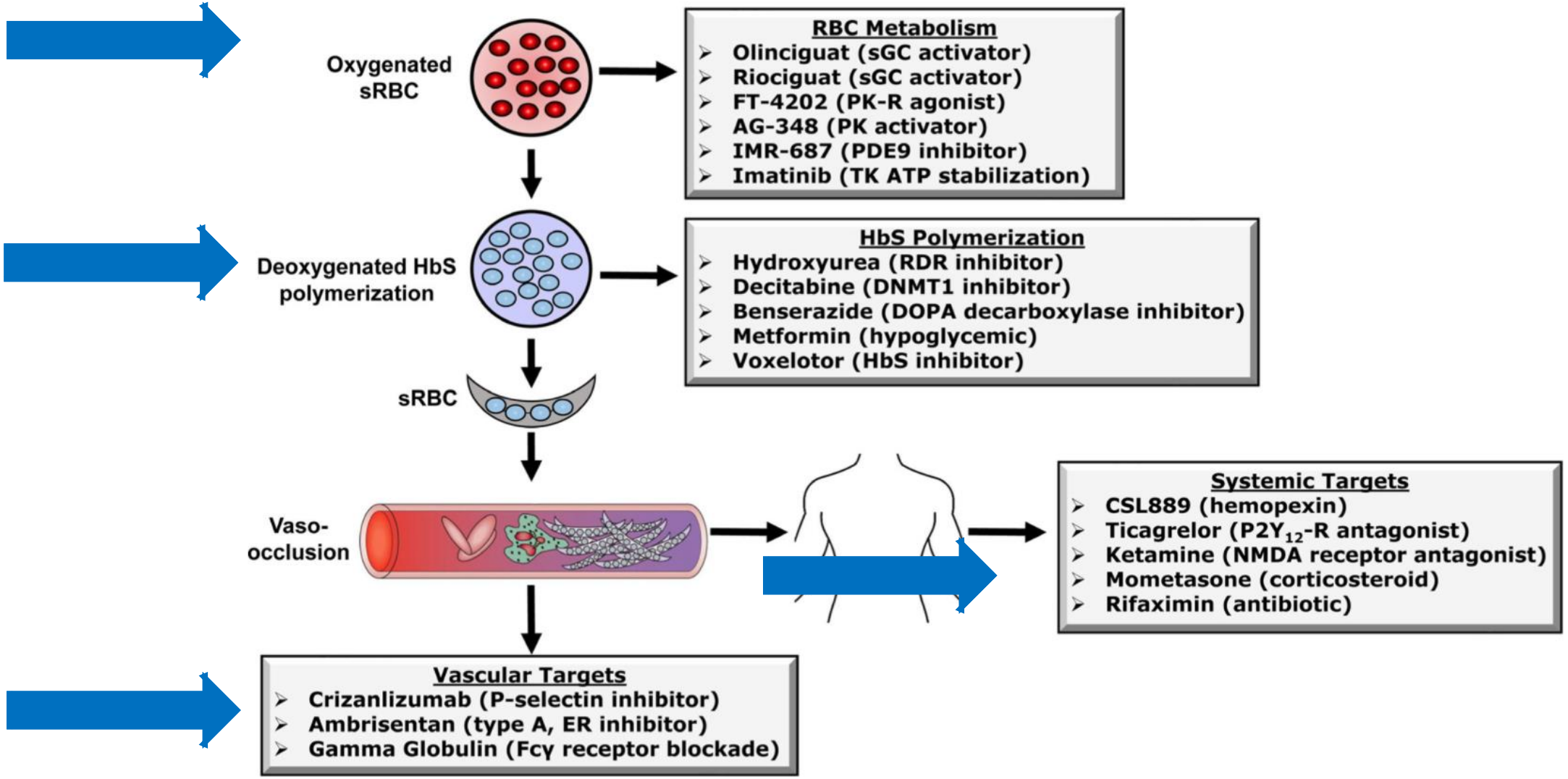
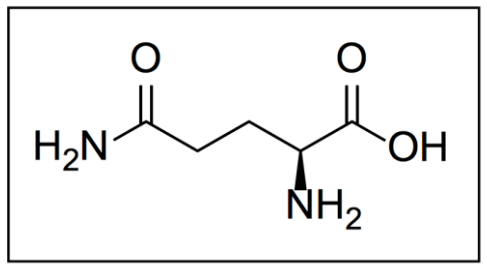
Novel Therapy Targets



Br J Haematol. 2021 Jul; 194(2): 240–251.

L-glutamine

- L-glutamine is an amino acid and precursor for nicotinamide adenine dinucleotide (NAD).
- L-glutamine prevents oxidative damage.
- **Pharmaceutical grade l-glutamine oral powder was approved by the FDA in July 2017** nearly 20 years after approval of hydroxyurea for use in Sickle Cell Disease.
- Not a dietary or nutritional supplement.

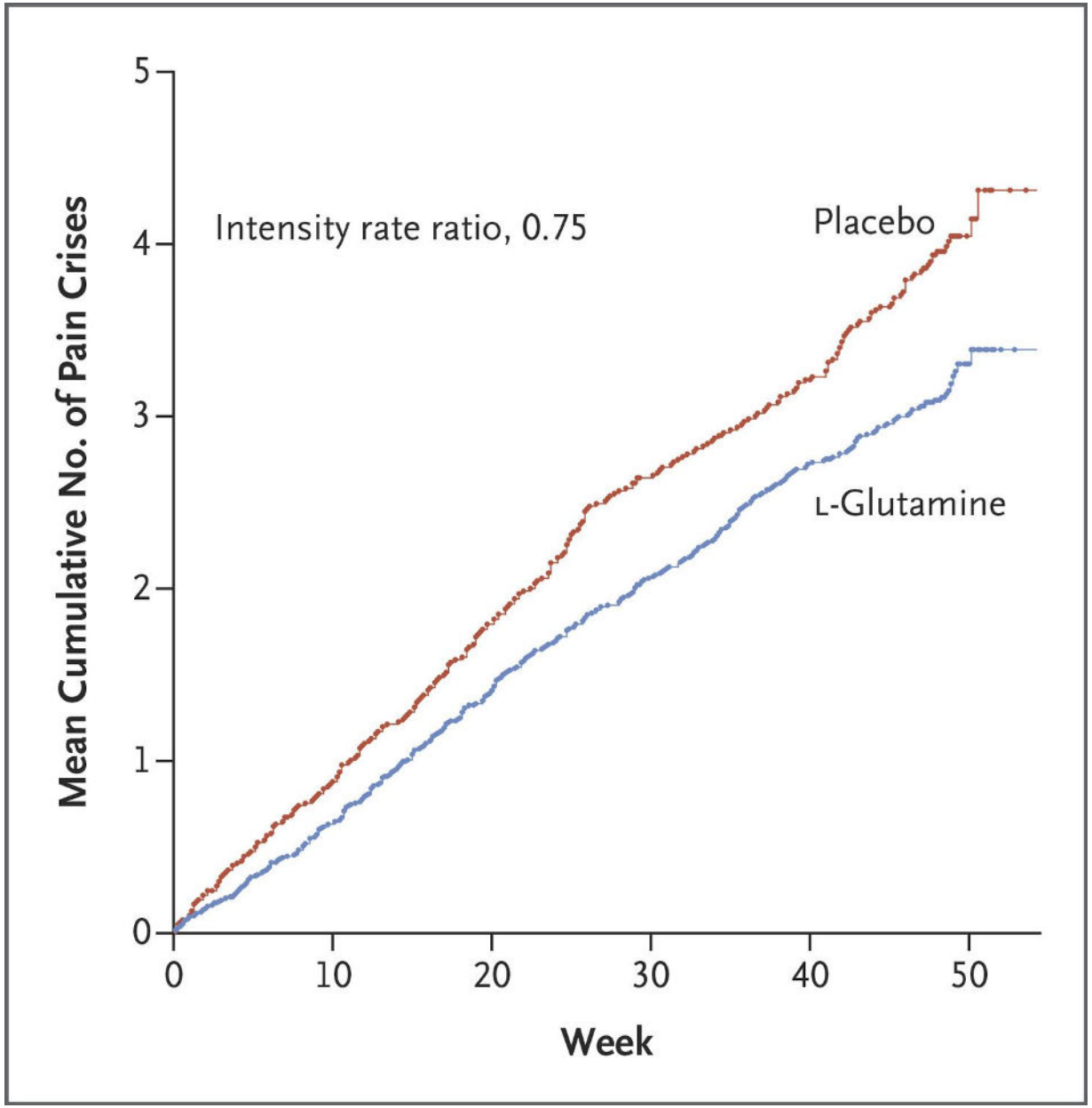


Y Niihara et al. *N Engl J Med.* 2018;379:226-235.

L-glutamine

Phase III randomized, placebo-controlled, double-blind trial showed reduction in the incidence of pain crises in subjects ⩾5years of age who had a history of two or more pain crises during the previous year and have sickle cell anemia or sickle β^0-thalassemia

Cumulative number of **pain crises was 25% lower in the L-glutamine group** than in the placebo group over the entire 48-week treatment period.

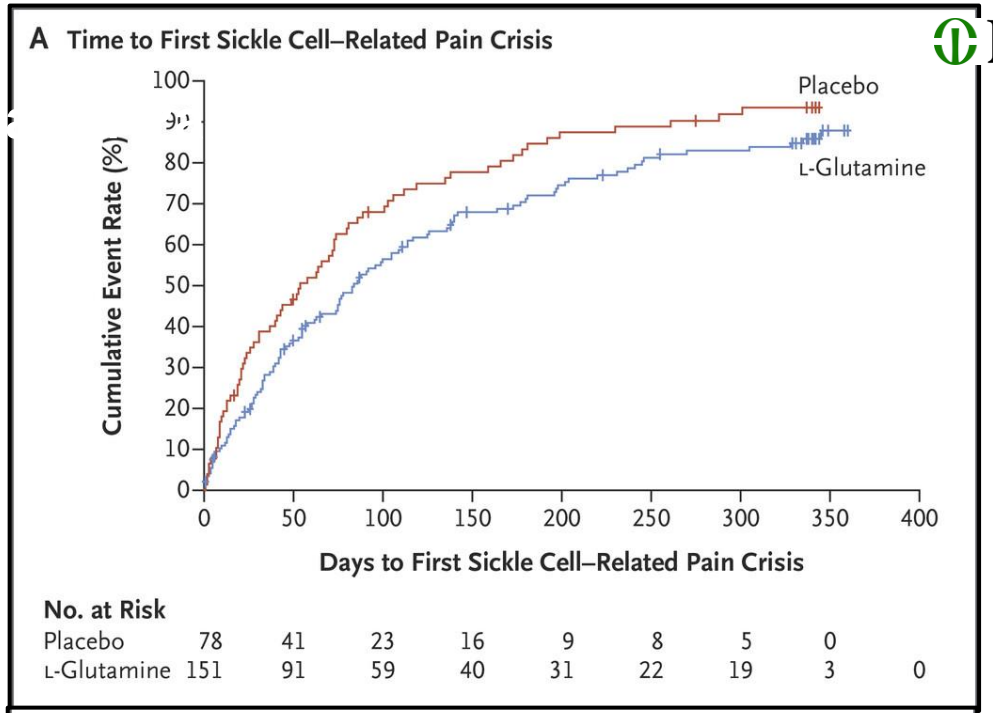


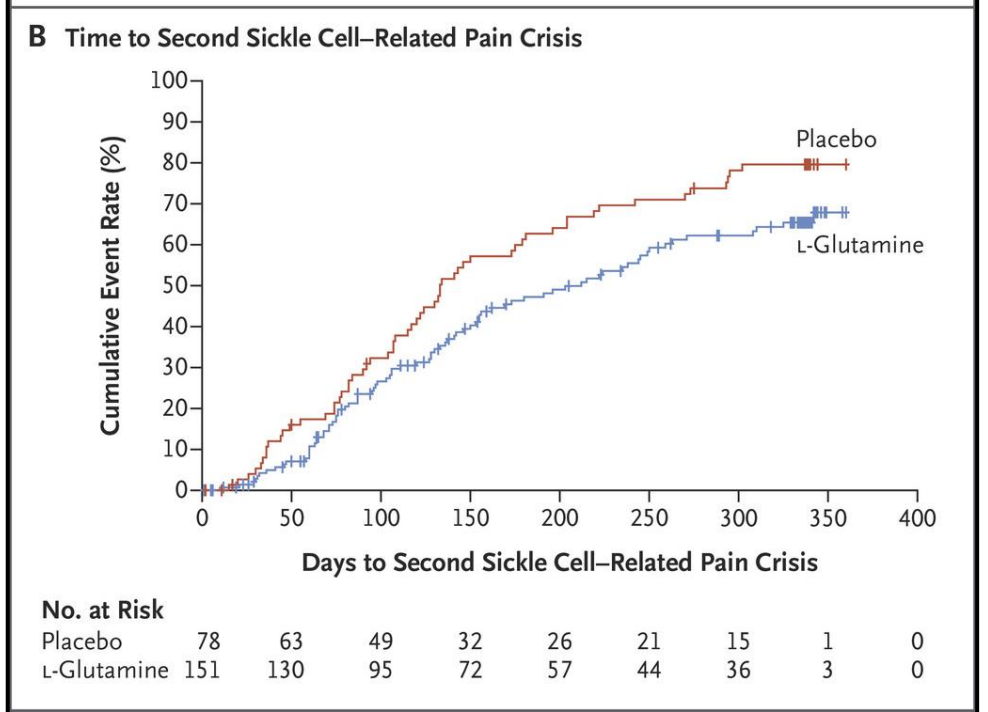
Y Niihara et al. *N Engl J Med*. 2018;379:226-235.

L-glutamine

The **median time to the first pain crisis was 84 days** (95% CI, 62 to 109) in the L-glutamine group, as compared with 54 days (95% CI, 31 to 73) in the placebo group (hazard ratio, 0.69; 95% CI, 0.52 to 0.93; **P=0.02**)

The **median time to the second pain crisis was 212 days** (95% CI, 153 to 250) in the L-glutamine group, as compared with 133 days (95% CI, 115 to 179) in the placebo group (hazard ratio, 0.68; 95% CI, 0.49 to 0.96; **P=0.03**).

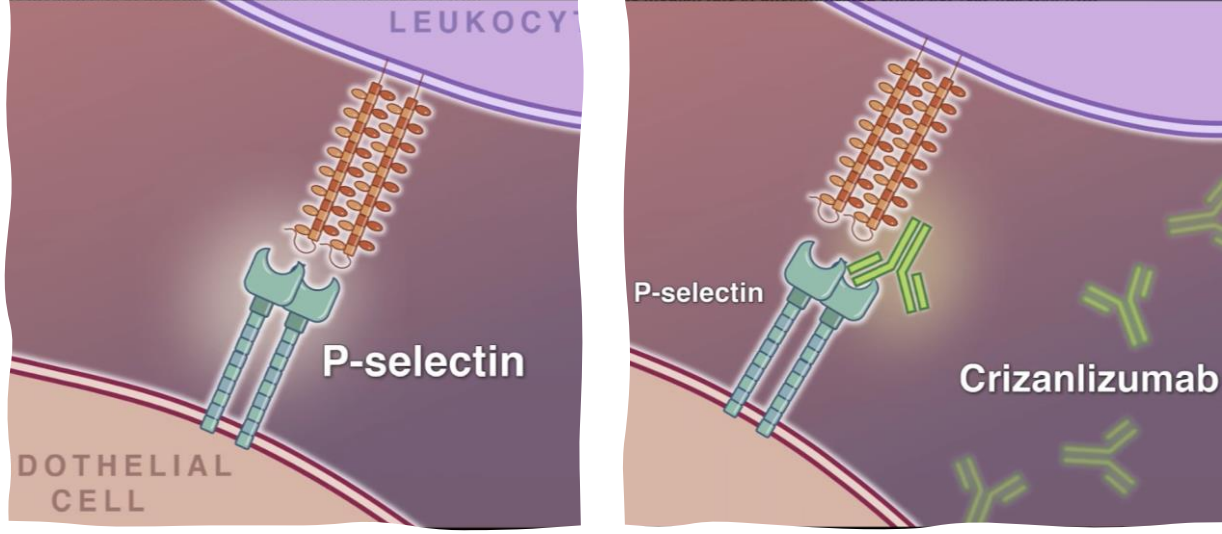


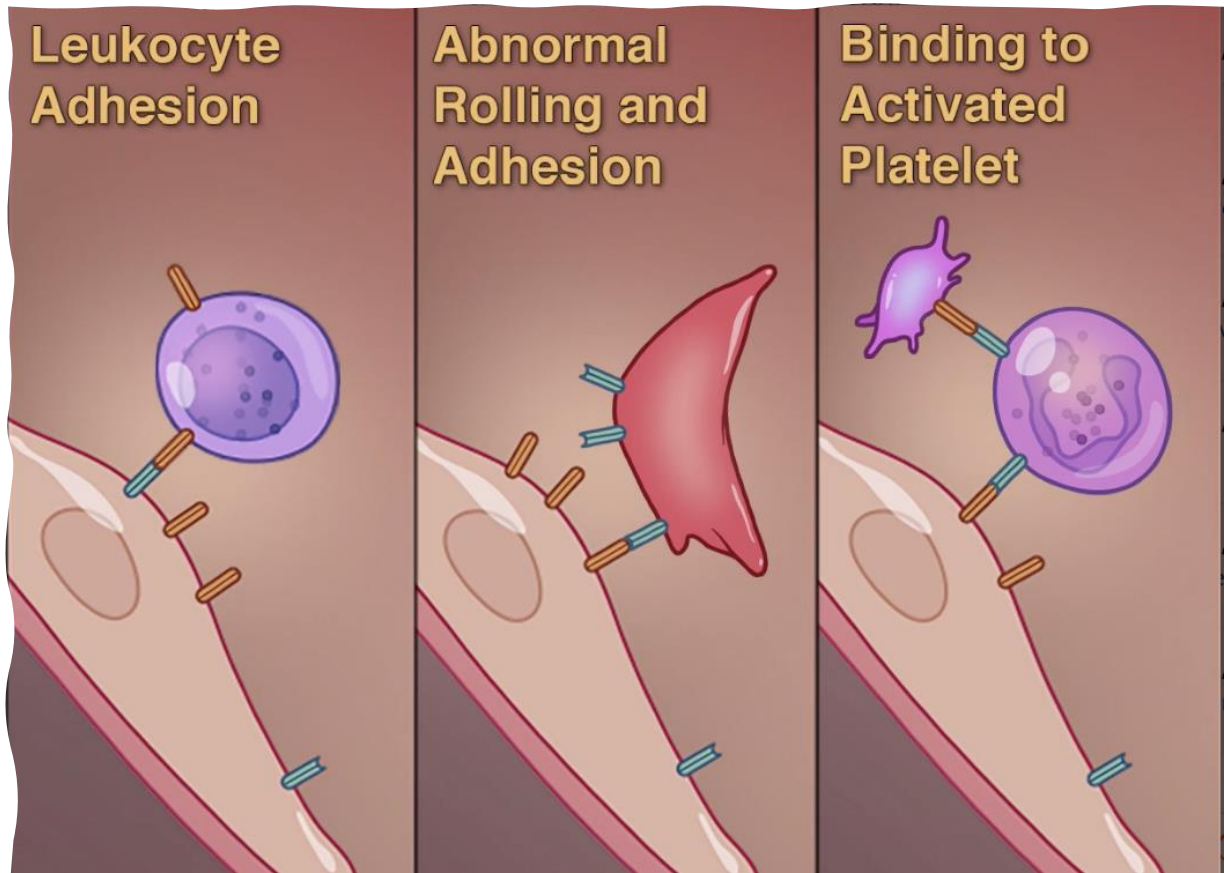


Y Niihara et al. *N Engl J Med*. 2018;379:226-235.

Crizanlizumab

- P-Selectin is implicated in the increased adhesion activity associated with VOCs.
- Crizanlizumab, a humanized monoclonal antibody that binds P-selectin, thereby blocking its interaction with P-selectin glycoprotein ligand-1 (PSGL-1).





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Crizanlizumab

Table 2. Annual Rates of Sickle Cell–Related Pain Crises.*

Variable	High-Dose Crizanlizumab	Low-Dose Crizanlizumab	Placebo
Primary end point: annual rate of crises in the intention-to-treat population			
No. of patients	67	66	65
Median rate of crises per year (IQR)	1.63 (0.00–3.97)	2.01 (1.00–3.98)	2.98 (1.25–5.87)
Difference from placebo — %	–45.3	–32.6	—
P value	0.01	0.18	—
No. of patients with crisis rate of zero at end of trial	24	12	11
Annual rate of crises in the per-protocol population			
No. of patients	40	44	41
Median rate of crises per year (IQR)	1.04 (0.00–3.42)	2.00 (1.00–3.02)	2.18 (1.96–4.96)
Difference from placebo — %	–52.3	–8.3	—
P value	0.02	0.13	—
No. of patients with crisis rate of zero at end of trial	15	7	5

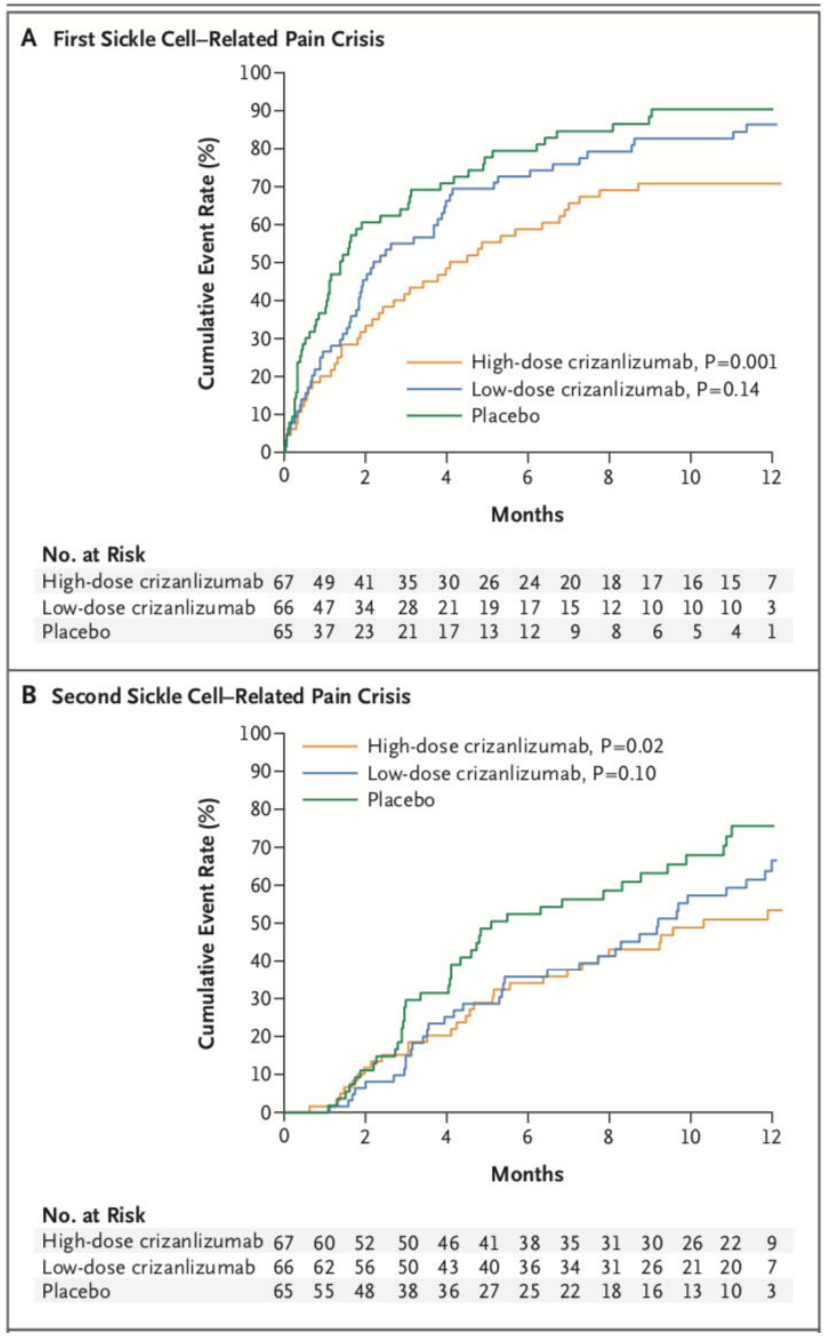
A significant reduction of 45.3% in the median crisis rate/year over the treatment period was achieved with crizanlizumab 5mg/kg (1.63) versus the placebo arm (2.98; p=0.01).

N Engl J Med. 2017;376:429-39.

Crizanlizumab

- **Median time to first** (4.07 months versus 1.38 months; p=0.001) and second (10.32 months versus 5.09 months; **p=0.02**) VOCs **favored crizanlizumab 5mg/kg** over placebo.

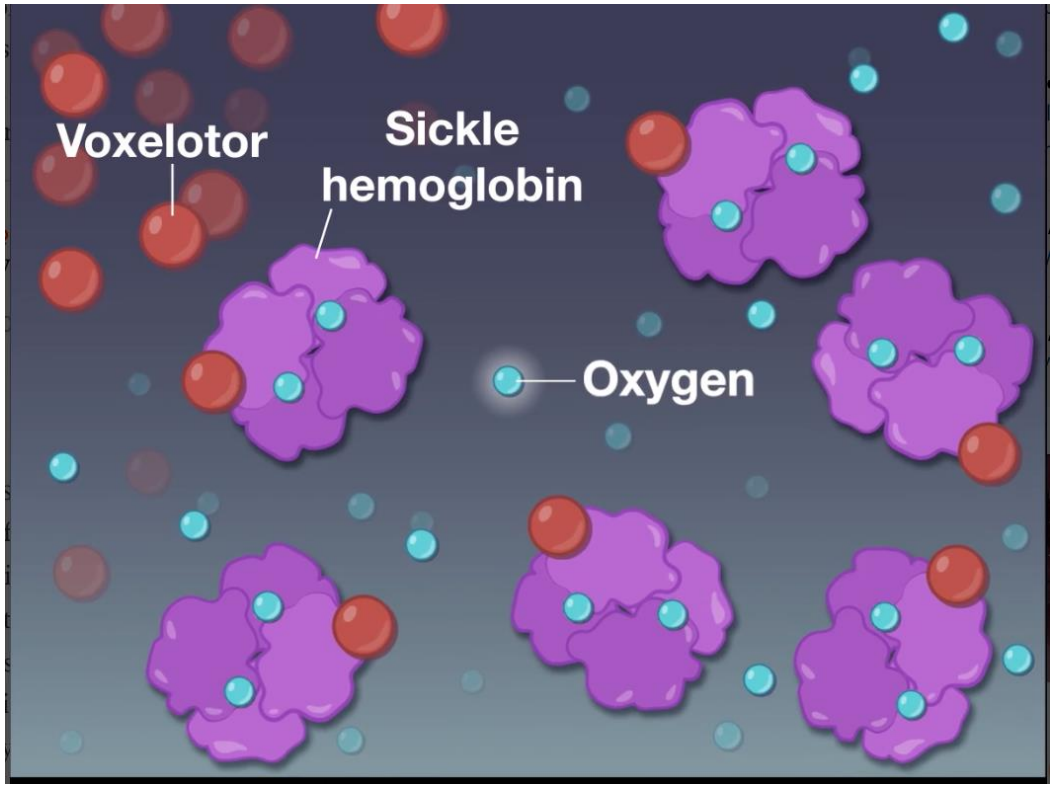
- **The lower crisis frequency** with high-dose crizanlizumab **was evident within 2 weeks** after the start of the 52-week treatment phase and was maintained through- out this phase

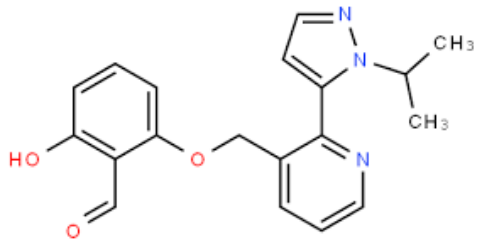


N Engl J Med. 2017;376:429-39.

Voxelotor

- First-in-class orally administered agent that **increases the affinity of hemoglobin for oxygen**, thereby inhibiting the polymerization of HbS.
- The Phase III HOPE trial is an international, multi-center, randomized, placebo-controlled, double-blind, parallel-group trial.
- From January 2017 through May 2018, a total of 274 participants were enrolled at 60 institutions across 12 countries — 90 were assigned to the 1500mg voxelotor group, 92 to the 900mg voxelotor group, and 92 to the placebo group





Voxelotor

N Engl J Med. 2019; 381:509-519.

Voxelotor

Age≥12

HOPE trial

Other Sickle cell disease genotypes included

RUSH

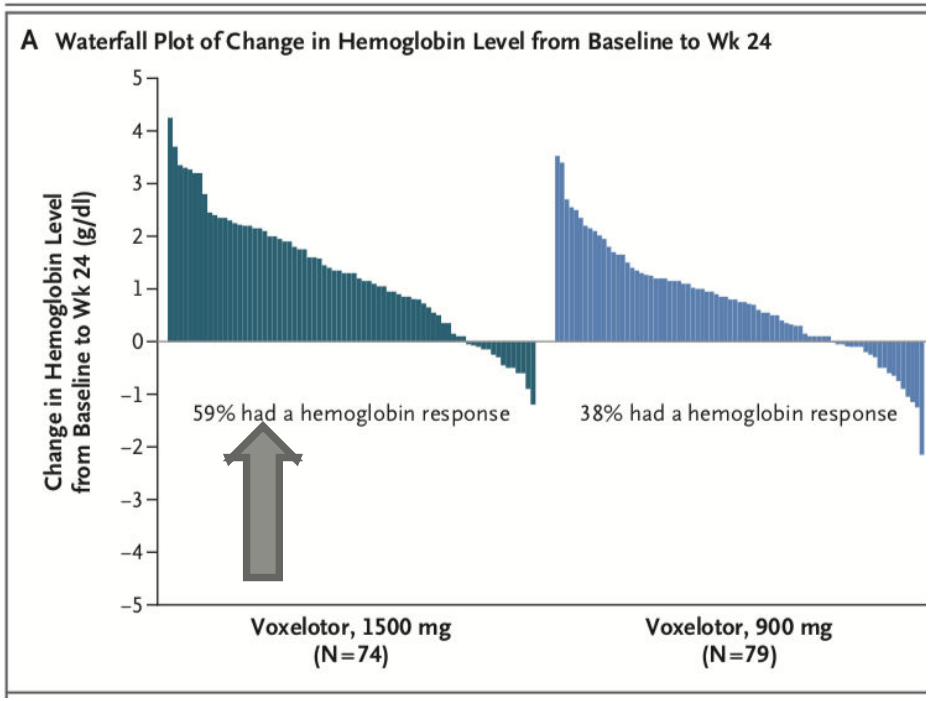
N Engl J Med. 2019; 381:509-519.

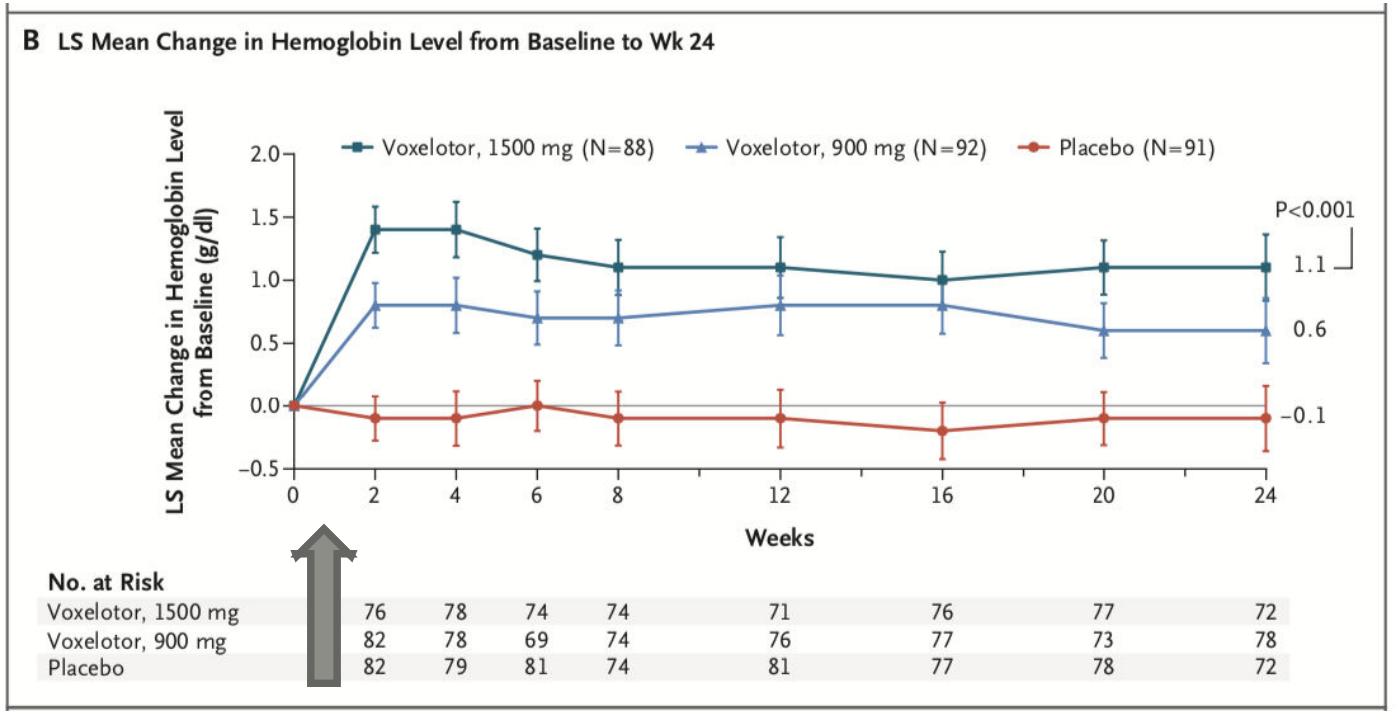
Table 1. Demographic and Clinical Characteristics of the Participants at Baseline.*

Characteristic	Voxelotor, 1500 mg (N=90)	Voxelotor, 900 mg (N=92)	Placebo (N=92)
Age — yr			
Median	24	24	28
Range	12–59	12–59	12–64
Age group — no. (%)			
12 to <18 yr	14 (16)	15 (16)	17 (18)
≥18 yr	76 (84)	77 (84)	75 (82)
Female sex — no. (%)	58 (64)	51 (55)	50 (54)
Race or ethnic group — no. (%)†			
Black	59 (66)	61 (66)	63 (68)
Arab or Middle Eastern	20 (22)	20 (22)	20 (22)
White	12 (13)	7 (8)	5 (5)
Asian	1 (1)	1 (1)	0
Other	2 (2)	5 (5)	6 (7)
Geographic region — no. (%)			
North America	34 (38)	36 (39)	35 (38)
Europe	19 (21)	19 (21)	18 (20)
Other	37 (41)	37 (40)	39 (42)
Sickle cell disease genotype — no. (%)			
Homozygous hemoglobin S	61 (68)	71 (77)	74 (80)
Hemoglobin Sβ^0-thalassemia	18 (20)	13 (14)	11 (12)
Hemoglobin Sβ^+-thalassemia	7 (8)	2 (2)	3 (3)
Hemoglobin SC	3 (3)	2 (2)	2 (2)
Other variant	1 (1)	4 (4)	2 (2)
Baseline hemoglobin level — g/dl			
Median	8.7	8.3	8.6
Range	5.9–10.8	5.9–10.8	6.1–10.5
No. of vaso-occlusive crises in the past 12 months — no. of patients (%)			
1	35 (39)	41 (45)	39 (42)
2–10	55 (61)	51 (55)	53 (58)
Patients receiving hydroxyurea at baseline — no. (%)	58 (64)	63 (68)	58 (63)

* There were no significant between-group differences in demographic and clinical characteristics at baseline. Percentages may not total 100 because of rounding.
† Race or ethnic group was self-reported; participants could be included in more than one category of race or ethnic group.

Voxelotor





- The study met its primary endpoint, defined as an increase in hemoglobin >1.0g/dl at week 24, for patients taking voxelotor (51%) versus those receiving placebo (7%; **p<0.001**).
- This outcome was achieved regardless of concomitant HU use or severity of anemia at baseline.
- HB increase noted as early as 2 weeks after initiating voxelotor.

N Engl J Med. 2019; 381:509-519.

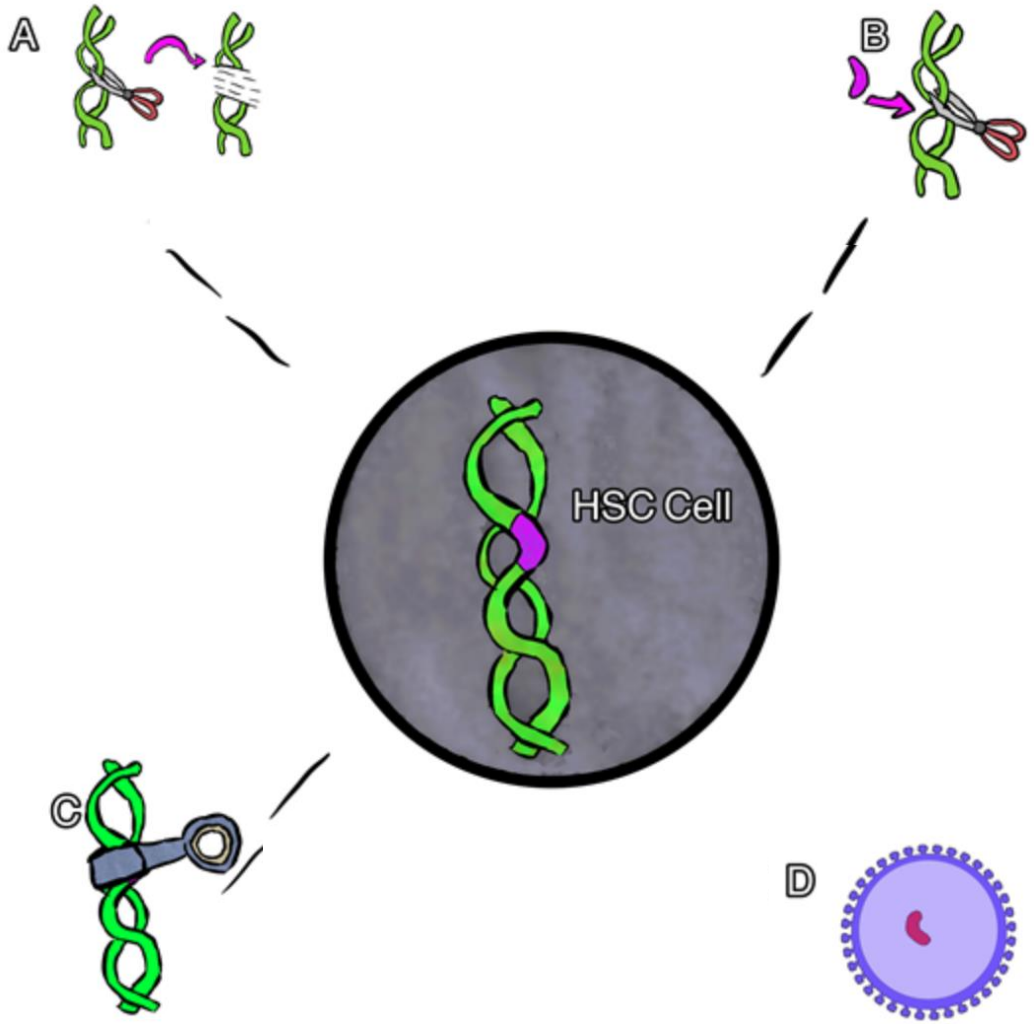
In the Pipeline

Treatment Category	Agent	Comments
Fetal Hemoglobin Inducer	Decitabine and Tetrahydrouridine	Phase1 study shows Improvement in Hb and hemolytic parameters. (*PLoS Med* 2017; 14: e1002382.) NCT04055818
Anti-inflammatory agents	Regadenoson NKTT120 Simvastatin Omega-3 fatty acids	1.Humanized monoclonal antibody rapid and sustained deletion of iNKT cells.(*PLoS One* 2017; 12: e0171067) 2.Improves NO, synergistic effect with HU.(*Br J Haematol* 2017; 177: 620–629) 3.Reduction in VOC
Antiplatelet	Ticagrelor Prasugrel	HESTIA3. NCT03615924 DOVE trail (*Pediatr Blood Cancer* 2016; 63: 299–305)
cGMP-modulating agents	Olinciguat IMR-687	1.Phase II clinical trial NCT03285178. 2.Phae II study shows improved HbF and hemolysis.
RBC pyruvate kinase-R (PKR) activator	Etavopivat	Phase 1 study: Improved Hb and hemolytic parameters. #EP1201:Brown et al.

Ther Adv Hematol. 2020, Vol. 11: 1–15.

Types of Gene Therapy

Gene addition



Gene editing

Gene silencing

Gene correction

SICK CELLS

RUSH

Gene Therapy Trials

Study name	LentiGlobin	DREPAGLOBE	CLIMB	PRECIZN-1	Genetic silencing of *BCL11A*	MOMENTUM	CEDAR
Type of gene therapy	Gene addition	Gene addition	Gene editing	Gene editing	Gene silencing	Gene addition	Gene correction
Editing tool	NA	NA	CRISPR-Cas9 RNP	Zinc finger	ShRNA	NA	HiFi CRISPR-Cas9 RNP
Type of stem cell manipulation	Transduction	Transduction	Electroporation	Transfection with zinc finger nuclease mRNA	Transduction	Transduction	Electroporation
Vector (y/n)	BB305 LVV	DROBE 1 LVV	None	None	BCH-BB694 LVV that encodes a microRNA-adapted shRNA	γG16D LVV	Nonintegrating AAV6 donor DNA repair template
Genetic target (y/n)	NA	NA	Erythroid lineage-specific enhancer of the *BCL11A* gene	11A (*BCL11A*) locus (erythroid enhancer)	*BCL11A* mRNA	N/a	Sickle mutation (adenosine— > thymine [A— > T]
Drug product	LentiGlobin BB305	DREPAGLOBE	CTX001	BIVV003	BCH-BB694	ARU-1801[26]	GPH101
Protein product	HbA^{T87Q}	βAS3, an antisickling β-globin protein (AS3) containing 3 amino acid substitutions in the wild-type *HBB*	HbF	HbF	HbF	HbF^{G16D}	HbA

2022 COVERAGE FOR SCD SUMMIT

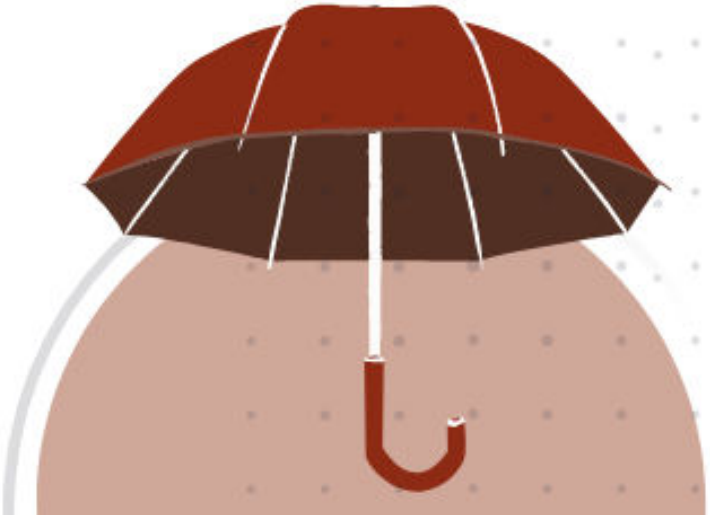
SICK CELLS

PAYER SESSION

Payer Perspectives on SCD Treatment Outcomes and Coverage Policies

Emily Tsiao, PharmD
Premera Blue Cross

Terry Cothran, DPh
Oklahoma Health Care Authority



A Patient-Centered Care Approach SCD Formulary Management

1. Take advantage of ePA and UM capabilities to exclude SCD members from UM requirements when appropriate to decrease the administrative burden for SCD members and their providers
2. Clearly communicate at every opportunity to decrease the administrative burden for SCD members and their providers
3. Have a clear Champion, Operations Lead, and Project Manager to help align goals and support a successful implementation

Key Partners

- Champion
- Operations Lead
- Project Manager

ePA: Electronic prior authorization; SCD: Sickle cell disease; UM: Utilization management

Example: Opioid UM Program

Premera has a history of promoting the safe and thoughtful use of opioids

Prior Authorization

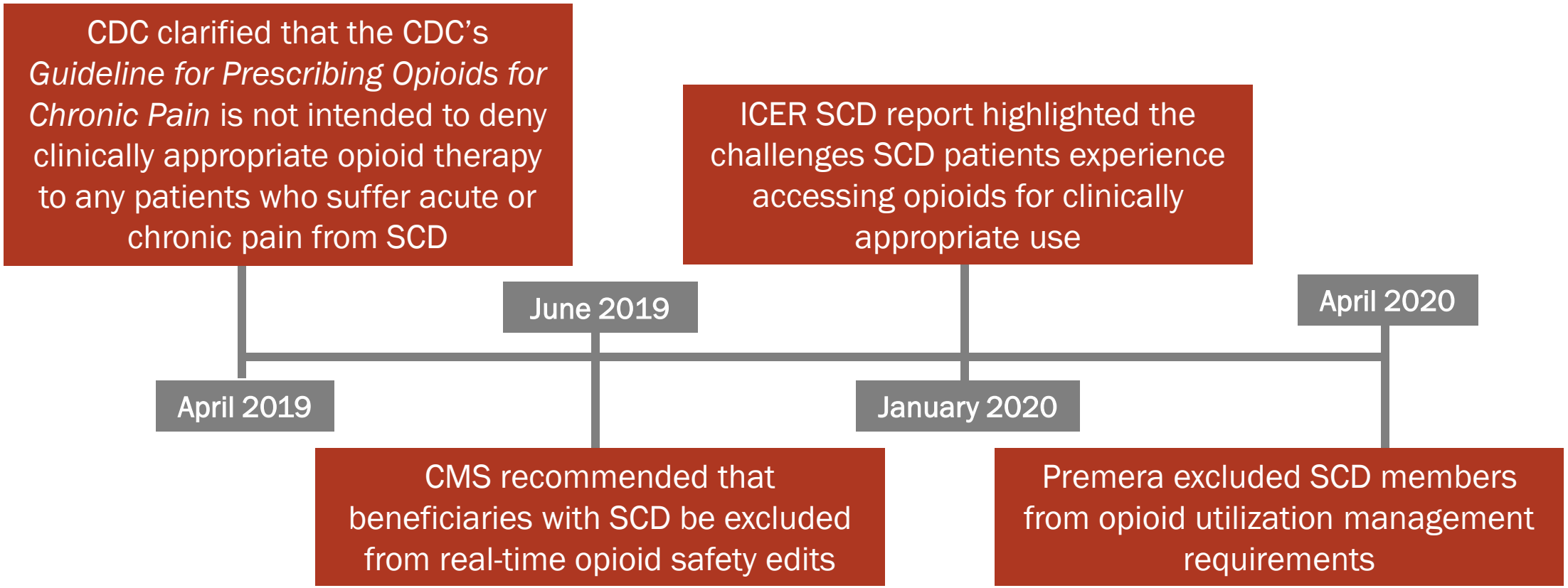
- >Seven-Day Supply for an Opioid-Naïve Member
- Long-Acting Opioids

Quantity Limit

- Short-Acting Opioids
- Long-Acting Opioids

	Commercial Line of Business Members	Commercial Sickle Cell Disease Members
Pharmacy Benefit Membership	1.08M	142
Distinct Opioid Users	115,835	61
% of Members Using an Opioid	11%	43%
% of Members Using a Long-Acting Opioid	2%	8%

Opioid Guidance Timeline



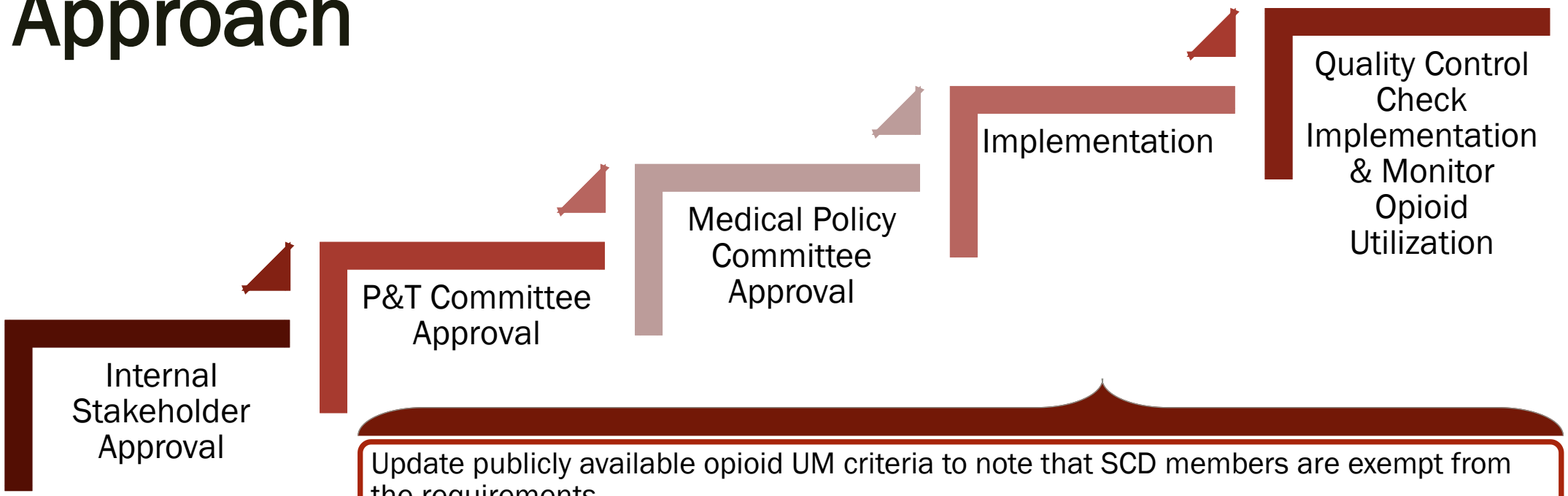
CDC: Centers for Disease Control and Prevention; SCD: Sickle cell disease; CMS: Centers for Medicare & Medicaid Services; ICER: Institute for Clinical and Economic Review

U.S. Centers for Disease Control and Prevention. (2019, April 24). *CDC Advises Against Misapplication of the Guideline for Prescribing Opioids for Chronic Pain* [Press Release]. https://www.cdc.gov/media/releases/2019/s0424-advises-misapplication-guideline-prescribing-opioids.html

Bradt P, Spackman E, Synnott PG, Chapman R, Beinfeld M, Rind DM, Pearson SD. Crizanlizumab, Voxelotor, and L-Glutamine for Sickle Cell Disease: Effectiveness and Value. Institute for Clinical and Economic Review, January 23, 2020. https://icer.org/wp-content/uploads/2020/10/ICER_SCD_EvidenceReport_031220-FOR-PUBLICATION.pdf

CMS Office of Minority Health. Opioid Prescription in Medicare Beneficiaries: Prescription Opioid Policies and Implications for Beneficiaries with Sickle Cell Disease. Baltimore, MD: Centers for Medicare & Medicaid Services; June 2019. https://www.cms.gov/About-CMS/Agency-Information/OMH/Downloads/Opioid-Prescription-in-Medicare-Beneficiaries-Report.pdf

Approach



- Update publicly available opioid UM criteria to note that SCD members are exempt from the requirements
- Add a question asking whether member has SCD in ePA question set
- Specify in UM approval/denial letters sent to members and providers that SCD members are exempt from the opioid UM criteria
- Allow member IDs associated with a SCD diagnosis code to bypass the opioid UM POS rejections
- Allow member IDs with a claim for a SCD treatment drug to bypass the opioid UM POS rejections

P&T: Pharmacy & Therapeutics; SCD: Sickle cell disease; ePA: Electronic prior authorization; UM: Utilization management; POS: Point-of-sale

Findings

Modest change in the number of SCD members using opioids after opioid utilization requirement exception implemented on April 1, 2020

Commercial SCD Members Only

	2019	2020	2021
Pharmacy Benefit Membership	143	147	142
Distinct Opioid Users	58	54	61
% of Members Using an Opioid	41%	37%	43%
% of Members Using a Long-Acting Opioid	7%	7%	8%

Commercial Line of Business

	2019	2020	2021
Pharmacy Benefit Membership	1.04M	1.03M	1.08M
Distinct Opioid Users	118,220	110,471	115,835
% of Members Using an Opioid	11%	11%	11%
% of Members Using a Long-Acting Opioid	2%	2%	2%

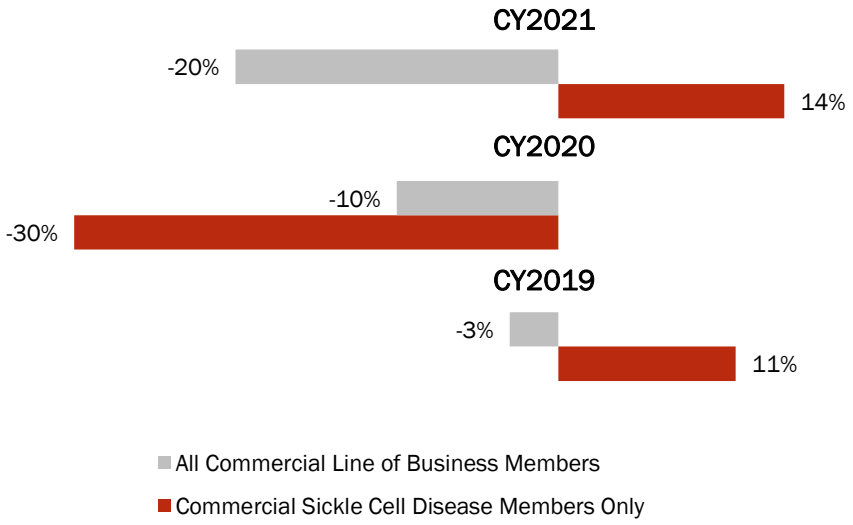
[1]Premera internal analysis; pharmacy benefit only; Average over CY

Findings

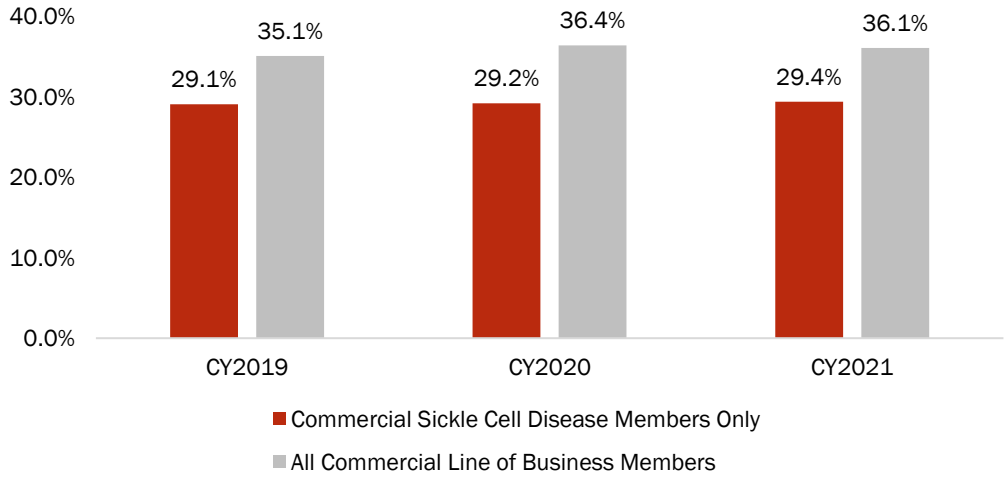
Changes for sickle cell disease members after opioid utilization requirement exception implemented on April 1, 2020

- *Modest increase in >90 MME/day member count YOY trend*
- *Modest increase in chronic opioid use with concurrent use of other high-risk drugs*

>90 MME/Day Member Count YOY Trend[1]



Chronic Opioid Use with Concurrent Use of Other High Risk Drugs[2]



[1]Premera internal analysis; pharmacy benefit only; Year-over-year (YOY) is defined as the current year compared to the previous year; MME (morphine milligram equivalents)
[2]Premera internal analysis; pharmacy benefit only; at least 60 days of opioid use and a high-risk drug (muscle relaxants, hypnotics, benzodiazepines, and barbiturates)

Sickle Cell Disease Dashboard

Views

- Individual member summary
- Disease summary with trends and updates

Supports treatment outcomes monitoring

- Post-treatment monitoring
- Treatment adherence

- Comorbidities
- Costs of Care
- Age
- Gender
- Line of Business
- Member Location
- Healthcare Visits
- Services
- Treatments

Formulary Review Considerations

- Will the drug be used in an underserved population? How can we identify them?
- Are there population-specific differences in pharmacodynamics/kinetics, etc?
- What barriers to accessing this treatment could they face? How can we help?
- What education do providers need to ensure optimal use of the drug?
- What challenges does the disease pose in a patients' daily life? How will the drug help overcome them?
- What socioeconomic/logistic/geographical factors might hinder successful use?
- What cultural factors (e.g., distrust of health care, health beliefs) might hinder use?

Formulary Resources

Physician experts that treat the disease

Patient organizations representing the population

Targeted literature search focused on the population

ICER reports, if available

Additional Opportunities

1. Evaluate how health disparity considerations are reviewed during P&T Committee meetings
2. Annual health disparities training for:
   - P&T Committee members
   - Medical Policy Committee members
   - Pharmacists, physicians, nurses, and case managers
3. Partner with key provider systems in network to provide Emergency Department SCD racial bias and treatment disparities training
4. Build equity and disparity conversations into standard formulary evaluation tools/considerations

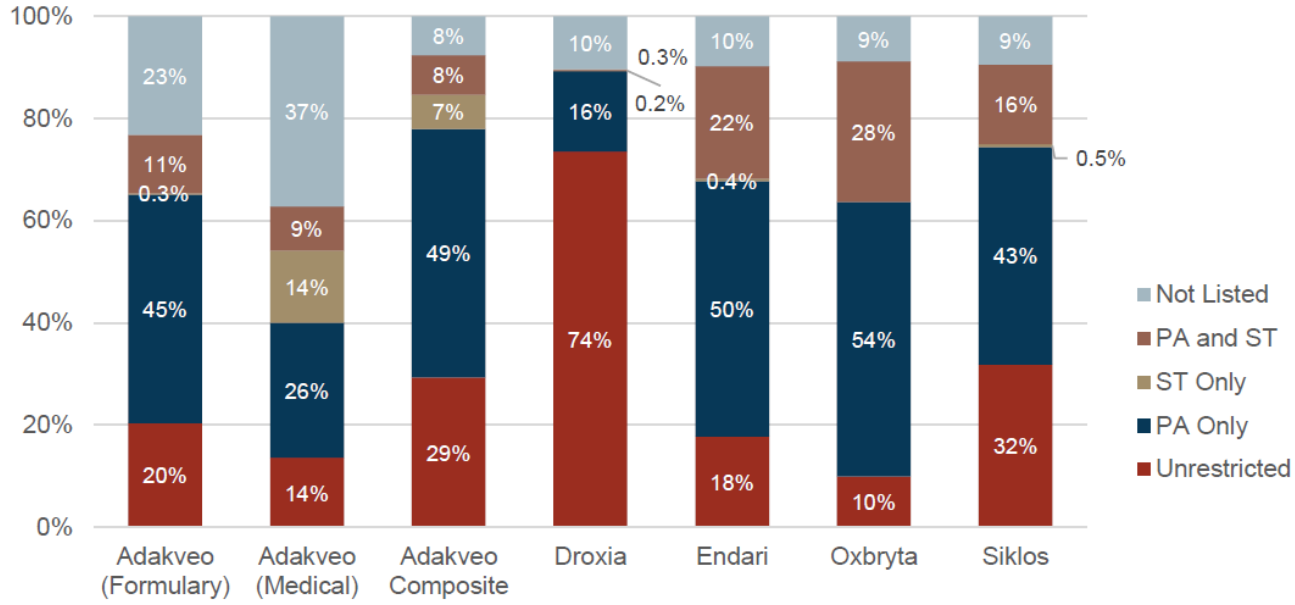
Category	Factor	Evaluation of Relevant Considerations
Other Benefits	Health benefits not captured by QALYs	
	Improved adherence	[Explain]
	Reduces disparities	[Explain]
	Reduces caregiver burden	[Explain]
	Novel mechanism	[How it will benefit patient that failed existing treatments]
	Work impact	☐Improves productivity ☐Decreases absenteeism ☐Reduces loss from work force ☐Other [Explain]
	Other benefits	

P&T: Pharmacy & Therapeutics; SCD: Sickle cell disease; UM: Utilization management

Medicaid Management of SCD

State Medicaid programs and Medicaid MCOs use utilization management strategies both to ensure beneficiaries are given clinically appropriate treatments and as a cost-saving strategy

Figure 3 – Utilization Management Techniques for SCD Therapies, All Medicaid



Medicaid Landscape and Access Review for Prescription Drugs Treating Sickle Cell Disease. https://sickcells.org/wp-content/uploads/2022/08/Sick-Cells_Medicaid-Access-and-Landscape-Review_Final-Report.pdf

Changing Treatment Landscape

- States are beginning to address the role of cell and gene therapies to inform future decision making
- State Medicaid programs and MCOs are preparing for cell and gene therapies by:
  - discussing, creating, or considering coverage policies and/or precertification criteria
  - value-based contracts
  - specific drug carve-outs from MCO contracts

Medicaid Landscape and Access Review for Prescription Drugs Treating Sickle Cell Disease. https://sickcells.org/wp-content/uploads/2022/08/Sick-Cells_Medicaid-Access-and-Landscape-Review_Final-Report.pdf

Medicaid Health Equity Initiatives

State Medicaid programs can take steps to address health equity and social determinants of health (SDoH), including:

- Examining the impact of bias in prescription drug coverage decisions
- Providing SDoH and discrimination training to relevant decision makers (i.e., DURB committee members, pharmacy directors, PBMs)
- Performing outreach to community experts (e.g., build relationships, utilize advisory panels)
- Actively screening patients for SDoH-related needs
- Collecting real-world data and patient reported quality of life information to use in coverage decision making
- Using cost effective analyses that adequately account for nonmedical and indirect costs associated with SCD
- Incorporating processes which facilitate meaningful stakeholder engagement

Medicaid Landscape and Access Review for Prescription Drugs Treating Sickle Cell Disease. https://sickcells.org/wp-content/uploads/2022/08/Sick-Cells_Medicaid-Access-and-Landscape-Review_Final-Report.pdf
Advancing Stakeholder Engagement with Medicaid: Centering the Patient Voice in Coverage Decisions. https://sickcells.org/wp-content/uploads/2022/03/Advancing-Stakeholder-Engagement-with-Medicaid.pdf

2022 COVERAGE FOR SCD SUMMIT

SICK CELLS

2022 COVERAGE FOR SCD SUMMIT

Improving Equity and Affordability of SCD Therapies

Best Practices in Benefit Strategies and Payer Management