CENTENE PHARMACY SERVICES

CENTENE Corporation

Updates on Sickle Cell Disease

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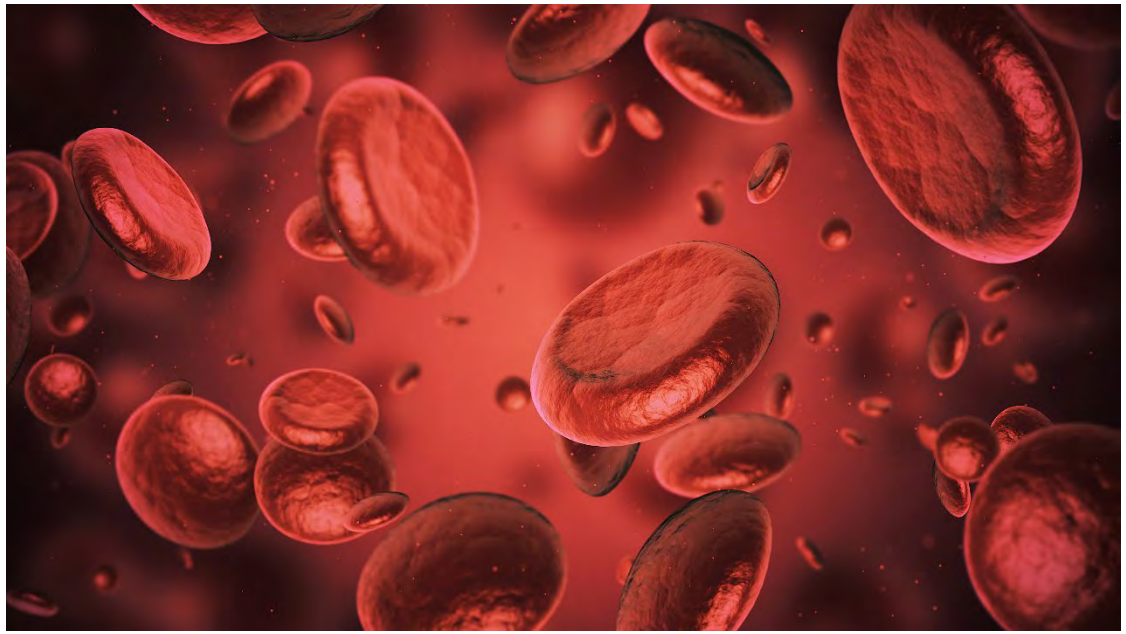
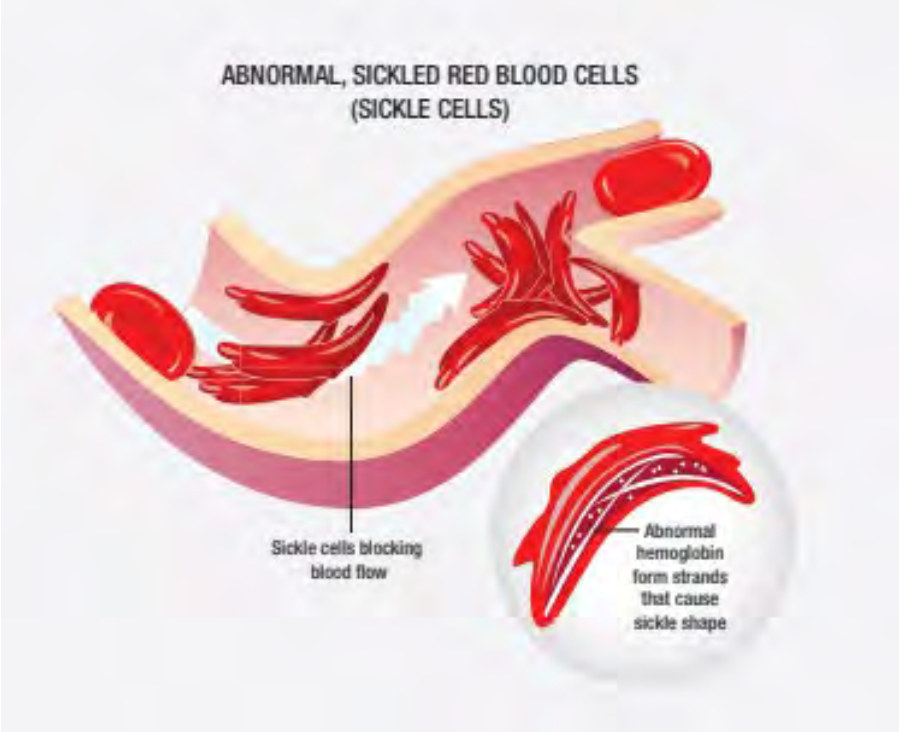
Learning Objectives

- Describe the background of Sickle Cell Disease.
- Discuss barriers to care and health disparities pertaining to Sickle Cell Disease.
- Compare treatment options for Sickle Cell Disease.

What is Sickle Cell Disease?

Hereditary blood disorder that affects about 100,000 Americans



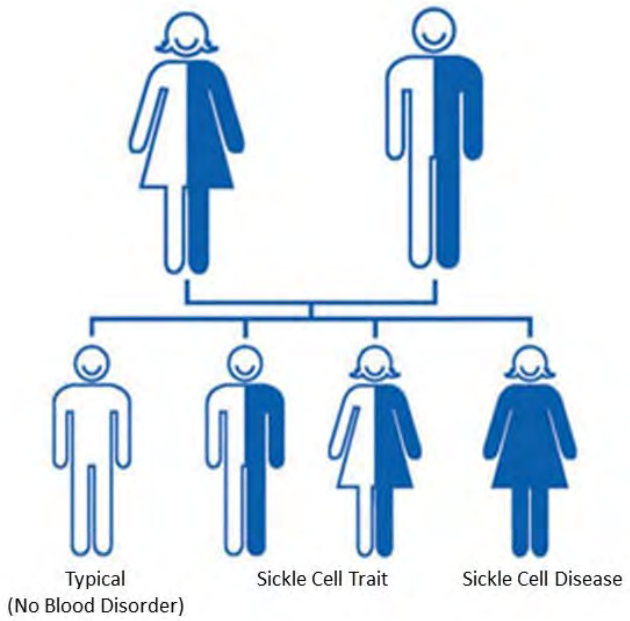


Characterized by misshapen red blood cells

www.cdc.gov/ncbddd/sicklecell/data.html

www.nhlbi.nih.gov/resources/sickle-cell-disease

Heredity



Patients with Sickle Cell Trait typically have no signs or symptoms of disease

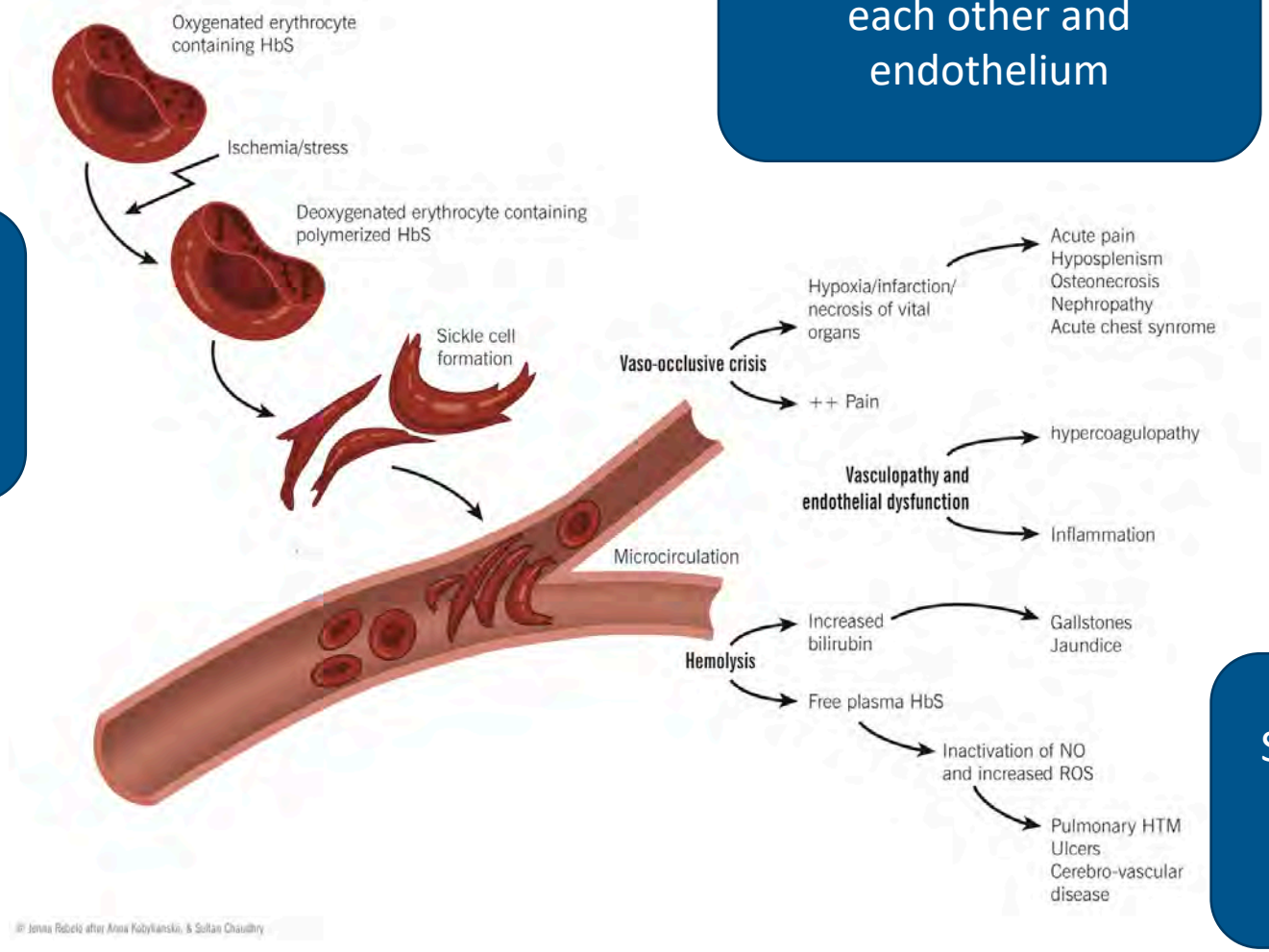
Patients must inherit abnormal hemoglobin genes from both parents

Patients may get different types of abnormal genes from each parent

Pathophysiology

Sickled cells adhere to each other and endothelium

Ischemia and/or stress leads to polymerizing HbS causing cells to “sickle”



Sickled cells breakdown at a faster rate than normal erythrocytes

Complications

Acute Chest Syndrome

Anemia

Blood Clots

End-Organ Damage

Infection

Kidney disfunction

Leg Ulcers

Liver Problems

Pain

Priapism

Pulmonary Hypertension

Sleep-Disordered Breathing

Splenic Sequestration

Stroke

Vision Loss

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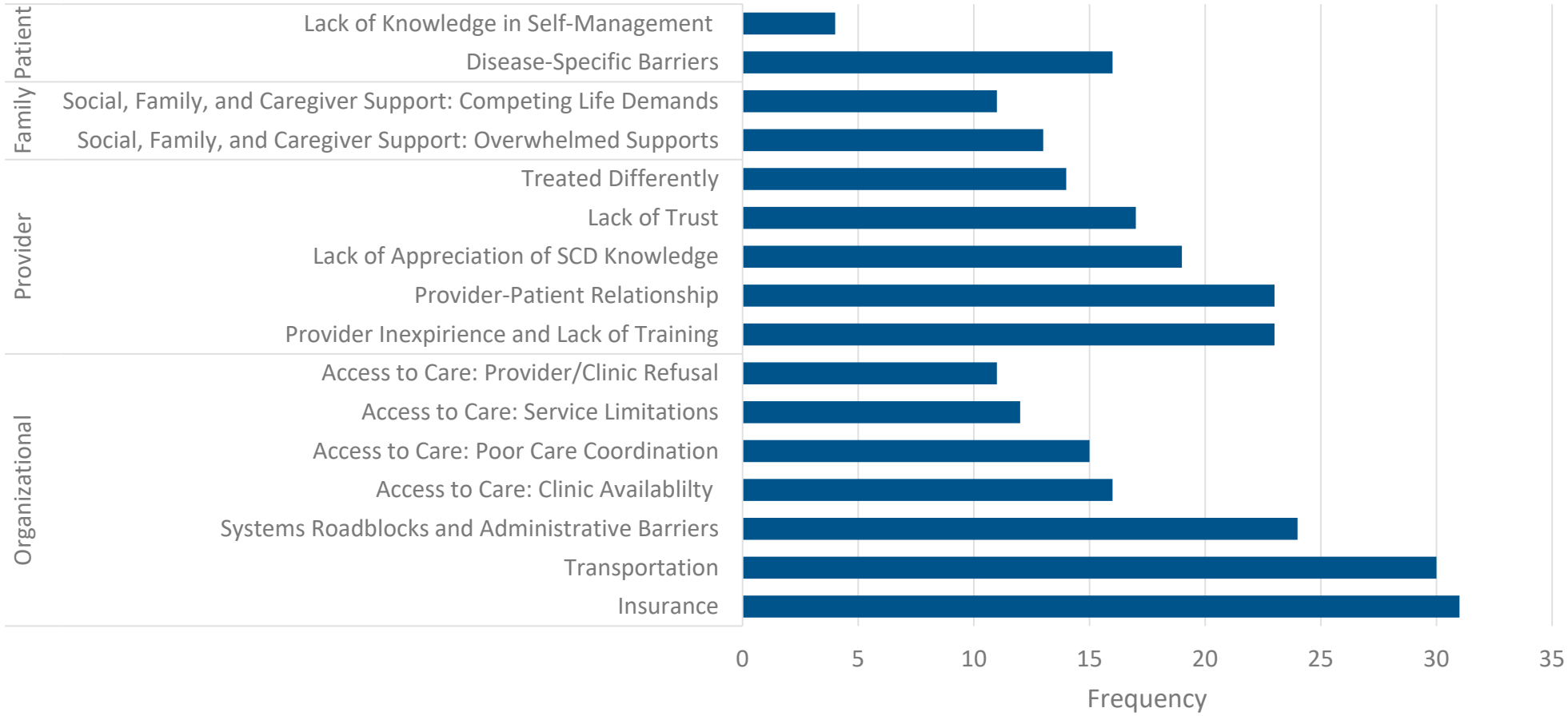
- Compare pharmacological treatments for Sickle Cell Disease.

Barriers to Care

A 2022 study surveyed several hundred patients at an SCD center for their perspectives on barriers to care



journals.plos.org/plosone/article?id=10.1371/journal.pone.0265342

Barriers to Care

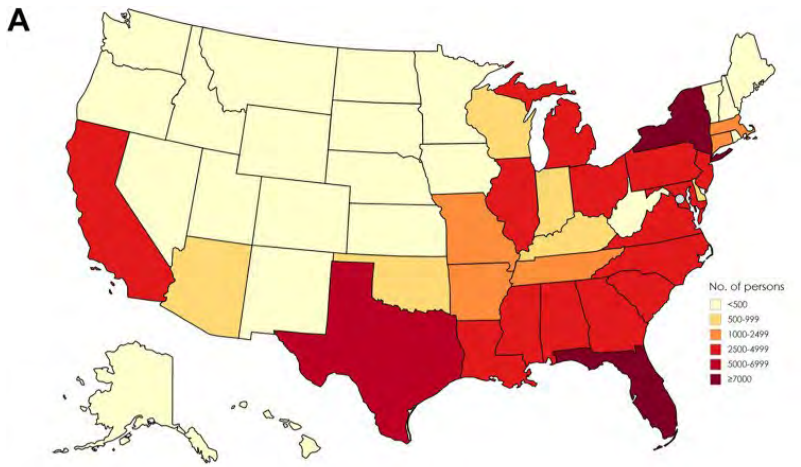
If you live in a city where they don't have a clinic that specializes in sickle cell and you're just trying to find a hematologist, I found that they are hesitant to take on sickle cell patients. They'll typically say no you need to see this specific hematologist, go to this specific institution, this specific clinic. And I've even had one clinic go so far as to tell me the reason that they do that is because they don't want to manage the medication.

They (healthcare providers) should be trained better and let the patient have a say-so because the patient knows what he needs…This is what I need. I know it. I've been through this 100 times. I know.

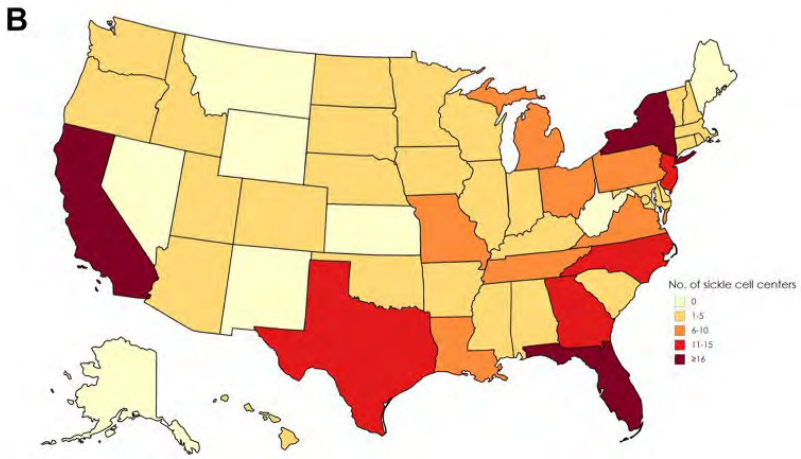
So much is going on and I just remember, you know, my appointment's coming up, I need to schedule it. It may seem easy to go ahead and schedule an appointment, but it's not always that easy.

Health Disparities

Number of persons with SCD



Number of SDC centers



Readmission rates for SCD patients were reported to be 33% at 30 days

Two-to-three-decade shorter lifespan then the general population

Young adults transitioning from pediatric care to adult care see a **2.3-3-fold increase** in mortality

Reducing Health Care Disparities in Sickle Cell Disease: A Review
www.ncbi.nlm.nih.gov/pmc/articles/PMC6832089/

Addressing Health Disparities

Recognize
instances where structural or interpersonal racism exists

Educate
yourself, colleagues, and the institutions you work with

Advocate
for your patients by representing their interests

Listen
to your patients

In 2021, CDC Director Dr Rochelle Walensky gave a speech for the American Society of Hematology

She suggested four specific actions for providers & health systems to consider to tackle health disparities and racism

Additional actions can be found : www.cdc.gov/ncbddd/sicklecell/addressing-health-disparities.html

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Pharmacotherapy for Sickle Cell

Hydroxyurea

Crizanlizumab

L-glutamine

Voxelotor

Hydroxyurea

Generally preferred as disease-modifying **initial therapy** to prevent complications & decrease mortality.

Most beneficial in patients with Hb SS or Hb $S\beta^0$ thalassemia

Benefits not generally seen for several months on target doses

FDA-Approved	1998
Age	≥2 years of age[1]
Route	Oral (Capsules/Tablets)
Benefits	Reduces number of pain events Reduces hospitalizations Improved survival
Side Effects	Myelosuppression GI symptoms
Other Considerations	Avoid in pregnancy

[1]FDA-approved in ≥2 years of age, but used clinically in infants >6 mos

L-glutamine (Endari®)

Second-line option to reduce pain episodes

Can be given in conjunction with hydroxyurea

OTC glutamine formulations have not been adequately studied, pharmaceutical-grade formulation is preferred

FDA-Approved	2017
Age	≥5 years of age[1]
Mechanism of Action	Reduces oxidative stress of sickled cells
Route	Oral (powder added to food/beverage)
Benefits	Reduced acute pain episodes
Side Effects	Constipation, nausea, headache
Other Considerations	No long-term studies

[1]FDA-approved in ≥5 years of age, but may be considered off-label for younger children

www.nejm.org/doi/full/10.1056/NEJMoa1715971

Voxelotor (Oxbyta®)

Second-line option for complication reduction

Can be given in conjunction with hydroxyurea

Demonstrated improved hemoglobin and *tended* toward decreasing pain episodes (but was not statistically significant)

FDA-Approved	2019, 2021
Age	≥4 years of age
Mechanism of Action	Inhibits hemoglobin S polymerization
Route	Oral (tablet)
Benefits	Increased hemoglobin Improved global functioning Improved fatigue
Side Effects	Headache, nausea, diarrhea, fever
Other Considerations	CYP3A4 substrate

www.nejm.org/doi/full/10.1056/nejmoa1903212

Crizanlizumab (Adakveo®)

Second-line option for reducing pain episodes

Can be given in conjunction with hydroxyurea

Requires IV access and infusion site

FDA-Approved	2019
Age	≥6 years of age
Mechanism of Action	Inhibits adhesion of sickled red blood cells
Route	IV
Benefits	Reduced pain episodes
Side Effects	Nausea, arthralgia Infusion reactions (rare)
Other Considerations	May cause platelet clumping in blood samples

https://www.nejm.org/doi/full/10.1056/NEJMoa1611770

Other treatments

Blood Transfusions

- Commonly used to treat acute complications
- May be used perioperatively
- Commonly used for symptomatic anemia

Stem Cell Transplant

- Only curative treatment modality

Prophylactic Penicillin

- Used routinely in infants and young children (up to age 5) to prevent *Streptococcus pneumoniae*

Immunizations

Annual Flu vaccine

DO NOT recommend live vaccine

COVID vaccine

Three-dose bivalent series

Pneumococcal

One-dose of PCV15 or PCV20

MenACWY

Two-dose series at least 8 weeks apart and revaccinate every 5 years

Men B

Two- or three-dose primary series, one dose booster 1 year after primary series, and revaccinate every 2-3 years

Haemophilus influenzae type b vaccination

One dose if not previously given

Non-Pharmacologic Recommendations

Get support

Find good medical care

Look for clinical studies

Get regular check-ups

Learn healthy habits

Prevent infections

Summary

Sickle Cell Disease is an inherited blood disorder that can lead to various complications.

Mainstay of treatment is hydroxyurea; second-line treatments to prevent complications now exist as well.

Systemic barriers and health disparities contribute to the disease burden of SCD

Vaccines and non-pharmacologic treatment are vital for long-term outcomes.

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Thank you!