National Alliance of Sickle Cell Centers

Building a Network of Sickle Cell Centers

Julie Kanter, MD

President, NASCC

Director, Adult Sickle Cell Program
Co-Director Lifespan Sickle Cell Research Center
Associate Professor Hematology/Oncology

University of Alabama at Birmingham

DISCLOSURE/Conflict of interest

- **Consultancy:** Imara, Novartis, Forma Graphite, Axcella Rx, Fulcrum Tx, Guidepoint Global, GLG, Coleman
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- **Steering Committee:** Novartis, Astrazeneca
- **Membership on a Scientific Advisory Committee:** Astrazeneca, Sanofi, Beam, Novartis, Acel Rx, Forma, NovoNordisc

Discussion of off-label drug use: N/A

Objectives

- Discuss where we are now with managing sickle cell disease from a systems level
- Evaluate a successful model of treatment of chronic conditions
- Evaluate how we can apply those models of treatment to SCD
- Discuss what defines a sickle cell center?
- Introduce National Alliance of Sickle Cell Centers

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Introduction

Sickle Cell Disease: a rare disease that still limits life expectancy due to chronic complications DESPITE childhood survival and availability of therapies

Many individuals with SCD have complex health care needs that get more complicated as they grow older.

Health care providers with disease-specific knowledge are **critical** for prevention of complications throughout the lifespan.

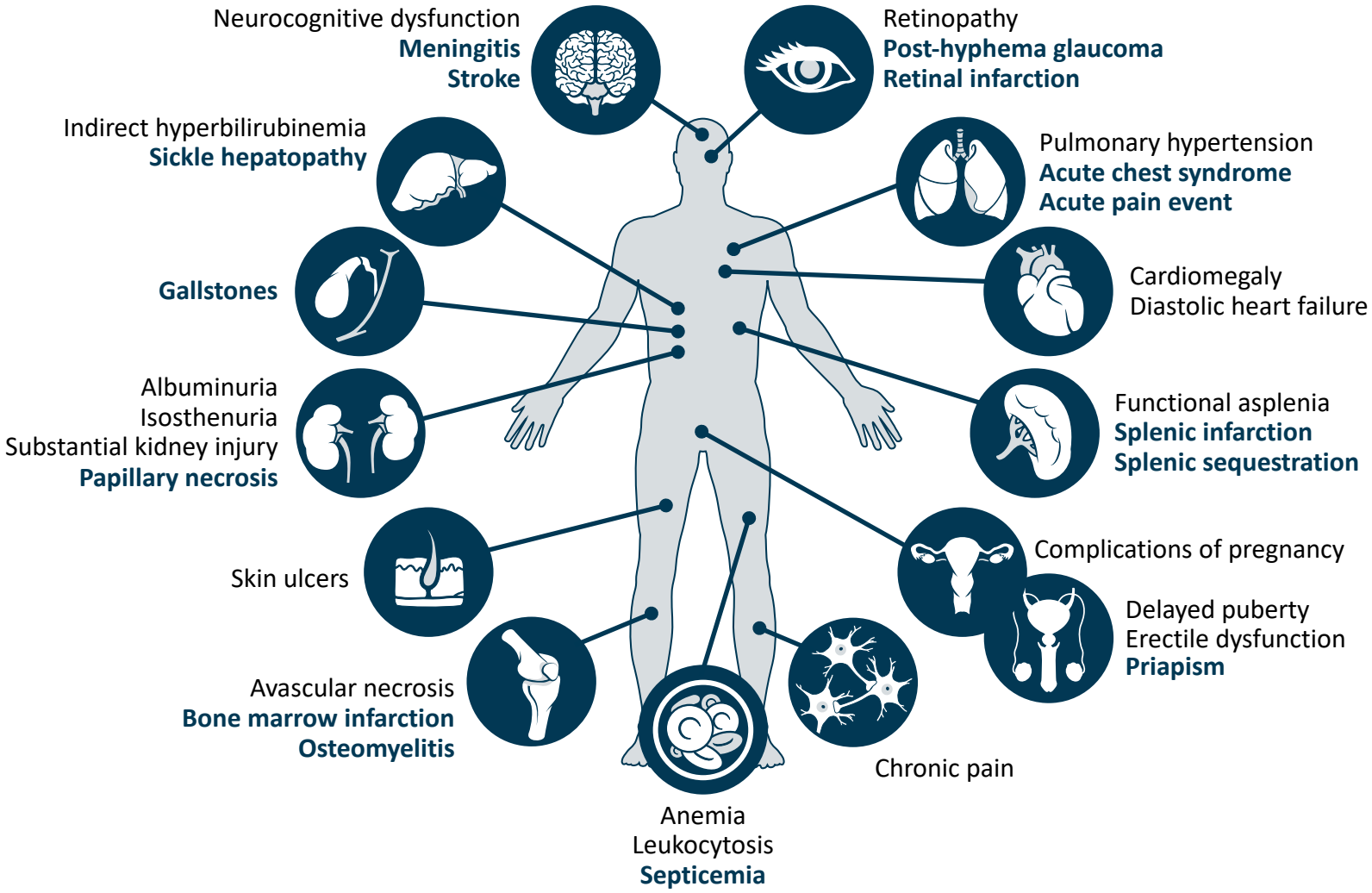
Introduction

- At the individual level, it is about where you live:
  - Healthcare opportunities and insurance are state-based and even more local than that
  - Insurance may be available but ≠ access to necessary care
  - Best care often equilibrates to best advocates or knowledge

- GOAL: Shift programmatic emphasis to how to build a system of care to enhance access and coordination of care throughout the lifespan

- We need to ensure individuals with SCD get appropriate, COMPREHENSIVE care

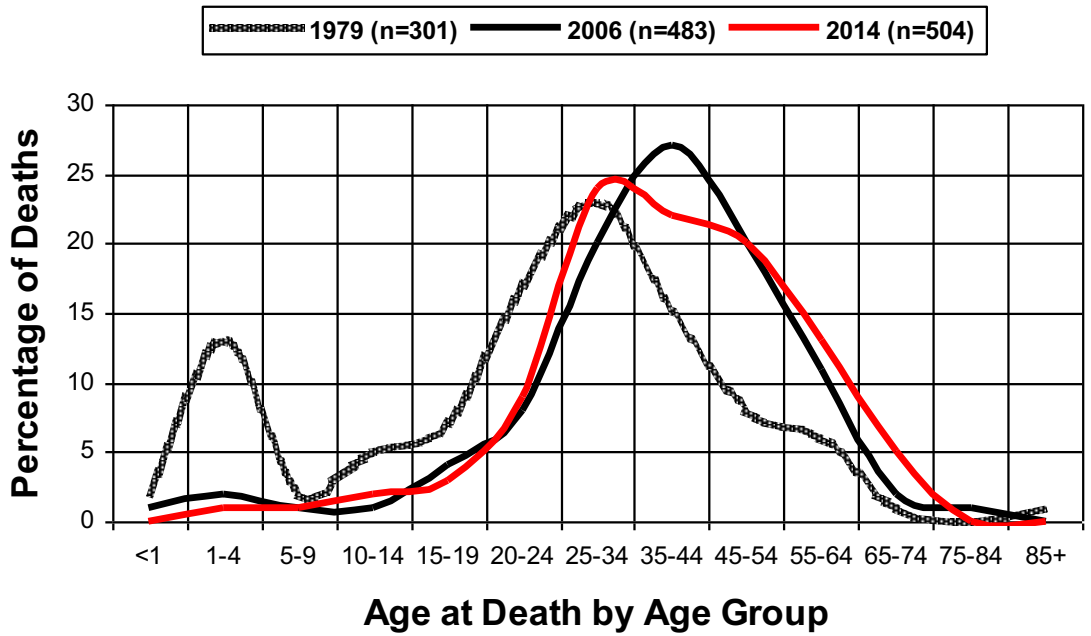
SCD Is a Multi-System Disease



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

SCD and Mortality in the US

- Childhood survival 96-98% for all genotypes
- In 2014, most deaths (66%) occur at ages 25-54 yrs
- More recent surveillance data from Georgia and California show mean age at death 43 yrs for women, 41 yrs for men



Quinn, *Blood* 115:3447, 2010;
Paulukonis, *Public Health Reports* 131:367, 2016

SCD is a complicated, chronic and complex disease

- A complex chronic disease: a condition involving multiple morbidities that requires the attention of multiple health care providers or facilities and possibly community
- Individuals with complex health needs require both medical and social support from a wide variety caregivers.
- A patient with complex chronic disease presents to the health care system with unique needs, disabilities, or functional limitations.

- **So how can we treat complicated, chronic and complex disease from a systems level?**

SCD: what we don’t know

- There are many elements of “standard of care” that are not validated or proven on the population level
- BROAD variation in practice where anecdote and opinion have become translated as data out of necessity
- Even with the data that is proven there is substantial gap exist between knowledge and practice
- Historical professional and political boundaries along with minimal funding have significantly inhibited improvement

HOW DO WE GET BETTER?

- Research of new drugs/therapies are important and should continue but NOT at the expense of ensuring patients get appropriate SCD-specific care
- Ensuring access to a SCD specialist for all persons with SCD should be our primary goal
- We also need to validate practices in standard of care
- AND we need to ensure we are getting appropriately reimbursed for the care we provide-so we can keep giving persons the care we need (and increase our ability to recruit, train and teach new providers)

- Outcomes are the results of systems of care, not just individuals and we have to work together as centers to improve outcomes

How do we treat complicated, chronic and complex disease?

- One example of mechanism for treating chronic (or complicated) conditions is the patient-centered medical home (PCMH).
- **PCMH offers a possible model for providing comprehensive, coordinated PRIMARY care by providing patient-centered coordinated and accessible care that is continuously improved through a systems-based approach to quality and safety (AHRQ, 2011).**
- However, PCHM are designed for adults (first) and for patients with complex health needs which may be different than chronic, lifelong health conditions
  - Heart failure, Diabetes Mellitus, Chronic Kidney Disease
- Not treated by PCMH (traditionally) are chronic diseases that require specialty care
  - Inherited metabolic conditions
  - Cystic fibrosis
  - Cancer
  - Hemophilia

What do we need to treat complicated, chronic and complex disease that requires a specialist?

- What models of care are available to treat lifespan conditions using a similar patient-focused, coordinated care model?
- Review an example of a similar disease:

- Cystic Fibrosis is a model of disease treatment
  - Lifelong, chronic disease that is diagnosed at birth
  - Complications accrue with age
  - Affected individuals require disease-specific, highly coordinated care

How did the cystic fibrosis foundation change outcomes using center-based care

1. They defined criteria of what it meant to be a “center” in the network by defining comprehensive care
2. Centers had to collaborate and agree to use the same longitudinal clinical data collection (a registry)
3. Focused, coordinated efforts to target improvements in care through philanthropy and drug development
4. Ongoing funding for data entry to improve for quality improvement
5. Invested in funding for therapeutic development

We need to model other successful systems-based approach to a lifespan, chronic conditions to improve outcomes in SCD

- Sickle Cell Disease
- NEEDS the same attention to disease-specific, highly coordinated care
- NEEDS cooperative efforts to collect longitudinal, clinical data
- NEEDS attention to quality improvement
- NEED to work together as an alliance of sickle cell centers to achieve these goals
- NEED dedicated funding for this at-risk population who has suffered more health disparities than those with cystic fibrosis, hemophilia, and other genetic diseases. COMBINED

- **THEN….we need to take this show on the road to those who need it most**

Systems level barriers to achieving care improvements in SCD

- Lack of a coordinated network of centers
- The lack of a clinical longitudinal data collection (registry)
- Lack of available centers that can provide expert care to the SCD population of all ages.
- While SCD is more prevalent than hemophilia and cystic fibrosis combined, there are only a handful of comprehensive adult SCD centers in the U.S. compared to 140 hemophilia centers and 280 cystic fibrosis centers
- Until recently, there are no clear definitions of a sickle cell center
- Result: Few FDA approved medications that are used <50% of people who could benefit from them

What do you need to be a an ADULT SCD Center?

- In 2019: conducted a STUDY to develop criteria for a SCD Center in advance of the ASH sickle cell center workshop

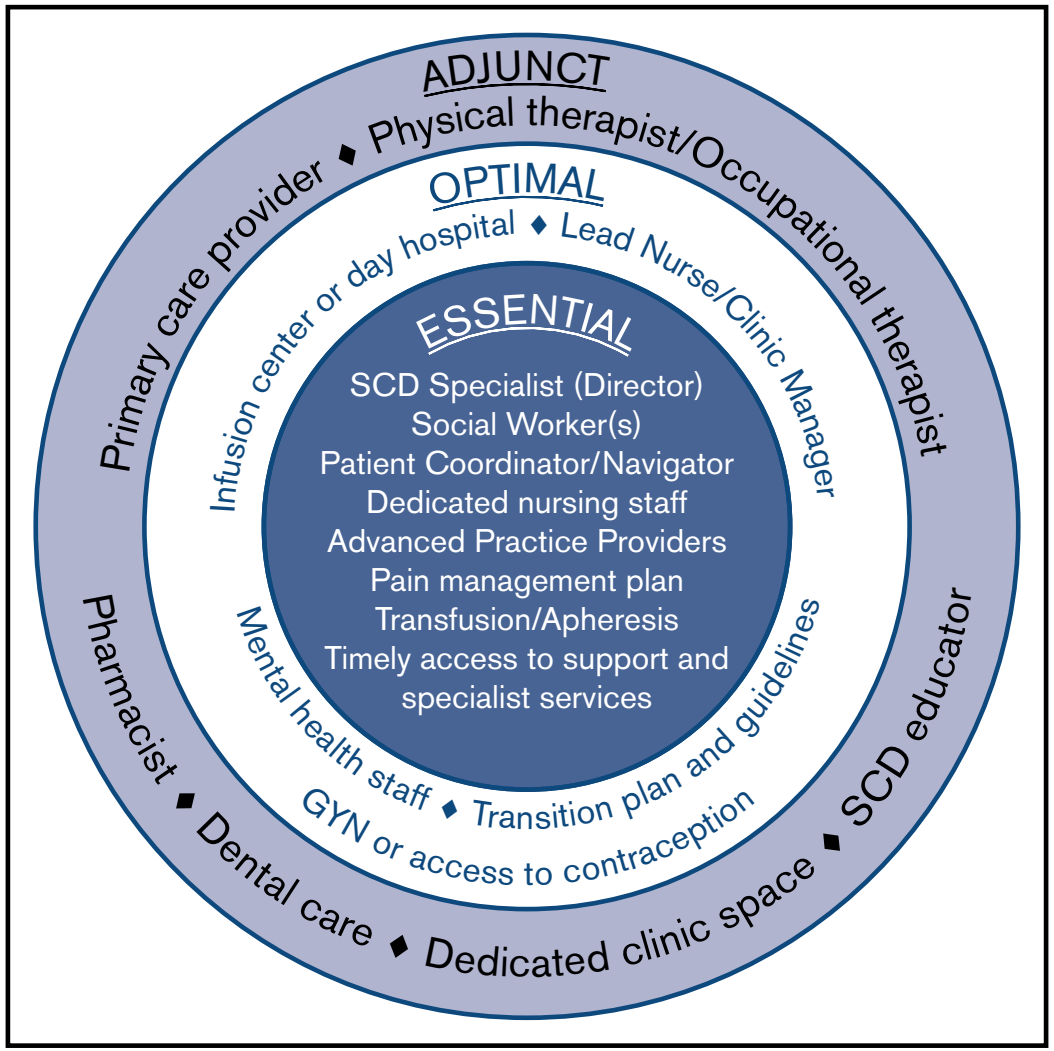
- METHODS:
  - Open-ended, qualitative survey was developed:
  - Sent to established adult SCD treatment centers in the US and the UK (convenience sample of 14 centers)
  - Each center was asked to first define their “center” including: their patient population, staffing, clinic structure, care provision, and outreach efforts.

- Components were defined as:
  - Essential elements of a sickle cell center (components needed to define themselves as a “sickle cell center”)
  - Optimal (but not necessary/essential)
  - Suggested (services that improve care delivery but are not essential to provide guideline-based care)

What is an adult sickle cell center?

- A thoughtful, team-based care approach to individuals living with sickle cell disease
- Consistent, comprehensive and coordinated care for adults with sickle cell disease throughout the “medical environment”
- A center with a programmatic emphasis on SCD for affected individuals
- A center that coordinates SCD care

- Recognizing: Treatment Centers are NOT one size fits all



All adult centers must provide comprehensive SCD medical practice:

- Disease modifying therapy: hydroxyurea, glutamine, etc
- SCD prevention practice
- Iron overload program
- Blood transfusion management
  avoid inappropriate blood transfusion, extended cross-matching of blood,
- Pain management plan/program
- Family planning plan
- Mental health plan

Defining the Elements of a Pediatric Sickle Cell Center

We identified 38 center elements based on:

- 2014 NHLBI SCD guidelines
- Existing literature
- Models from other diseases (CF, hemophilia, diabetes)
- Clinical experience

Elements clustered in 5 categories

- Medical and support staff
- Center processes
- Center physical resources
- Links with resources outside of pediatric SCD program
- Diagnostic studies and treatments

Process

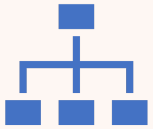
Emailed survey to 20 pediatric hematologists/SCD physicians

All physicians invited are engaged in SCD research, state newborn screening programs, and regional collaborations

Program size range: ~150 to 1600 SCD patients

Process

Modified Delphi process of consensus seeking



Respondents ranked each element as “essential” “optimal” or “suggested”

Elements did not have to be present at respondent’s site to be essential

Consensus = 2/3 agreement for each element

2nd round of surveys sent for elements that did not reach consensus on the first round

RESULTS: essential elements of pediatric centers

Staff	Processes	Diagnosis and Treatment
Sickle Cell Disease physician	Procedure for newborn screen followup	On-site transcranial Doppler ultrasonography
Pediatric Hem/onc team providing inpatient care	Written guidelines for acute & chronic care	Iron overload measurement by MRI
Dedicated outpatient nursing staff*	Protocols for Emergency Dept care	Compounded hydroxyurea
Access to Transfusion Medicine specialist*	Defined program for transition from pediatric to adult care	Erythrocytapheresis for acute complications
Gynecologist/prescriber of contraception		Erythrocytapheresis for chronic complications
Case manager/care coordinator		
Social worker		
Access to reproductive services for parents of children with SCD		

Optimal elements

Staff	Physical space	Treatment	Link with outside program
Advance practice provider	Dedicated inpatient unit	Access to clinical trials	Linkage with CBO
Dedicated inpatient nursing staff	Dedicated outpatient space		
Neuropsychologist*	Infusion center/day hospital		
Education liaison*			
Pain management specialist			

Elements that have not reached consensus

Mental health care provider
Physical therapist
Child life specialist
Written business plan
Standardized order sets in EMR
Individualized pain plans
Partnership with adult SCD program
Partnership with HSCT program
Formal genetic counseling services

Sickle Cell Center Models

- What about Sickle Cell Clinics or Programs who don't quite meet the definition for a "Center"
- **Associate Centers** may have some components of a sickle cell center while developing their full center
  - Centers–in-development
  - Data collection can be ongoing
  - Additions can be made as growth allows

- Sickle Cell Center + **Affiliate Center(s)**
  - Affiliates: spokes of the SCD center that provide care as an affiliation with a SCD center who may rely on the center for some of the components

- Community Heme-Onc Progams: can we connect them to sickle cell centers as affiliate centers?

Bottom Llne

SCD centers must provide compassionate, comprehensive care that is coordinated to ensure continuous and personalized care with attention to both the physical and emotional well-being of the individual.

Providing standardized, **equitable** patient-centered management will ensure higher quality, more cost-effective care for this vulnerable patient population.

INTRODUCING

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In building the alliance

There needs to be collaboration between centers to collect data and perform quality improvement

We need a mechanism to track outcomes and perform quality improvement

We need to develop treatment guidelines and policies that can be tested

We want to ensure we have a board of directors and an advisory board with provider, stakeholder, community and patient representation

Need to ensure we are integrating with the community

AND: A plan for comparative quality assessment and improvement

National Alliance of Sickle Cell Centers

- **Founded by concerned physicians providing care for those living with sickle cell disease**

- **Mission:** To support sickle cell disease (SCD) centers in delivering high-quality comprehensive care by setting standards of care and promoting their adoption, identifying opportunities and resources to strengthen SCD centers, and advocating for access to comprehensive care to improve health outcomes, quality of life, and survival.

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NASCC GOALS:

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- Create an infrastructure for adult and pediatric SCD centers to define, continually enhance, and promote the adoption of standards of primary and specialized care that comprise a comprehensive care center for individuals with SCD.
- Develop tools and share information with SCD centers to use in implementing and operating a comprehensive care model within their organization.
- **Utilize the Globin Research Network for Data and Discovery (GRNDaD), a multi-site registry to optimize quality improvement and quality assurance through data analytics**
- Identify opportunities and resources (federal, state, and private) that SCD centers can utilize to sustain funding and ensure equitable access to comprehensive care.
- Create and sustain an advisory board of stakeholders committed to improving outcomes in SCD
- Work with ASH, SCDAA, CDC, and other similar organizations to accomplish these goals and to collaborate on research, data collection, advocacy, and other efforts of mutual interest.

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systems level possibilities

- Can we demonstrate that NASCC-recognized centers improve outcomes for patients and deserve improved reimbursement
- Can we find or create new payment models to include necessary comprehensive care (mental health care, case coordination, social work, etc)
- How can we enhance the model of sickle cell centers to increase access to care?
- How can we optimally use telemedicine to provide outreach care?
- Can we define, recognize and validate the use of “spokes” or ”affiliates?”

- Which sickle cell center has the lowest acute care use? How can we use the information to help other centers improve outcomes?

In summary:

- We discussed barriers to quality improvement in sickle cell disease from a systems level
- Reviewed a successful model of systems-level management of a chronic, lifespan condition
- Discussed how we can apply that model of treatment to SCD
- Introduced National Alliance of Sickle Cell Centers

- WWW.SICKLECELLCENTERS.ORG

- Hopefully-got you excited about how we can work together to really improve outcomes in sickle cell disease

WWW.SICKLECELLCENTERS.ORG

National Alliance of Sickle Cell Centers

- To JOIN: Make sure you say “yes” I am a center on the website
- Once you apply, NASCC will send you an application to fill out online.

Support

If you have problems:

Website questions: support@sicklecellcenters.org

General Questions: mmadisett@sicklecellcenters.org

*There is a lot of work to be done-we would love help and suggestions. Please stay tuned.

National Alliance of Sickle Cell Centers

Thank you!!!!

- **NASCC co-founders (and inaugural officers): Dr. Sophie Lankzron, Dr. Marsha Treadwell, Dr. Deepa Manwani**
- **NASCC board of directors**
- **NASCC Administrator: Mohan Madisetti**
- **NASCC IT guru: Judson Stevens**
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- **HRSA SCDTP CO-I**
- **GRNDaD EXEC committee**

Presented by: