

ECHO conference 9-2-2020

Northern Virginia Pediatric Sickle Cell Program

Experience with Hydroxyurea Implementation
starting in infancy, in a Community Practice

Team:

Elizabeth Yang, physician

Robin Dulman, physician

Angle Lewis, NP

Noravy Briere, NP

Bailey Notarangelo, nurse educator & coordinator

Pediatric Blood & Cancer

Prospective longitudinal follow-up of children with sickle cell disease treated with hydroxyurea since infancy

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Question:

If you start Hydroxyurea in infancy,
maximize Hydroxyurea therapy,
maintain good compliance with sickle cell care continuously,
how well can you do?

Methods – HU treatment

1. Parents of babies with Hgb SS or Hgb S beta0 were educated about HU at the baby's first clinic visit and were informed about the plan to start HU at ~9 months of age.
1. HU started at 20-25mg/kg/day, increased monthly up to 35mg/kg/day, or MTD (ANC 1000, plt 80)
1. HU monitoring visits q2 months. Hgb F checked at every q2 month visit.
1. HU dose titrated to achieve goal of Hgb F 30% as much as possible.
1. If non-adherent, patients were brought back to clinic monthly until adherence established.

Methods – Prospective data collection

The screenshot shows the REDCap web interface. The top header displays the project name "CCBD Northern VA Sickle Cell Database(IRB Protocol No.: Pro00002745, PI: Dr. Elizabeth Yang)". The left sidebar contains navigation links: "My Projects", "Project Home or Project Setup", "REDCap Messenger", "Data Collection" (with a minus sign), "Scheduling", "Record Status Dashboard", and "Add / Edit Records". The main content area has a top navigation bar with "Project Home", "Project Setup", "Other Functionality", and "Project Revision History". Below this is a "Quick Tasks" section with three buttons: "Codebook", "Export data", and "Create a report". To the right of these buttons are descriptive text blocks for each task.

REDCap™

Logged in as **elizabeth_yang**
Log out

My Projects
Project Home or Project Setup
REDCap Messenger
Project status: **Production**

Data Collection [minus icon]

Scheduling
Record Status Dashboard
Add / Edit Records

CCBD Northern VA Sickle Cell Database(IRB Protocol No.: Pro00002745, PI: Dr. Elizabeth Yang)

Project Home Project Setup Other Functionality Project Revision History

Quick Tasks

Codebook
The Codebook is a human-readable, read-only version of the project's Data Dictionary and serves as a quick reference for viewing field attributes.

Export data
Export your data from REDCap to open or view in Excel or various stats packages.

Create a report
Build custom reports for quick views of your data, and export reports to Excel/CSV.

2011 –






Longitudinal database

q6 month data capture for each patient

Follow each baby as he/she grows

The grid below displays the form-by-form progress of data entered for the currently selected record. You may click on the colored status icons to access that form/event. If you wish, you may modify the events below by navigating to the [Define My Events](#) page.

Legend for status icons:

-  Incomplete  Incomplete (no data saved) 
-  Unverified
-  Complete

[Choose action for record](#)
Medical Record Number 01735535

No upcoming calendar events

<div><div></div></div> <div>Data Collection Instrument</div>	Time point 1	Time point 2	Time point 3	Time point 4	Time point 5	Time point 6	Time point 7	Time point 8	Time point 9	Time point 10	Time point 11	Time point 12	Time point 13	Time point 14	Time point 15	Time point 16
Demographics	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Sickle Cell Disease Category	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Medical History Sickle Cell Related	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Co Morbid Conditions	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Transfusions	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Surgical History	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Medications	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Hospitalizations	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Emergency Room visits	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Immunizations	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
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Family History	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Social History	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Growth Vital Parameters	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Hematology	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Brain	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Lung	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Heart	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Liver	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Kidney	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Ophthalmology	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Genetic Counseling	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Social Worker Service	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Support Group	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Transition Program	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>
Bone Marrow Stem Cell Transplant	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>	<div><div></div></div>

Sickle Cell Disease Category

 Editing existing Medical Record Number **30384544**

Event Name: **Time point 4**

Medical Record Number

30384544

Six Month Interval Date



2019-09-13



Today

Y-M-D

Type of Sickle Cell Disease

- ☒ SS
- ☐ SC
- ☐ S-Beta +Thalassemia
- ☐ S-Beta 0 Thalassemia
- ☐ SS-Alpha Thalassemia Trait
- ☐ SD
- ☐ SE
- ☐ SO-Arab
- ☐ S-HPFH
- ☐ Other

[reset](#)

Comments



[Expand](#)

Form Status

Complete?



Complete ▼

Save & Exit Form

Save & Stay



-- Cancel --

Medical History Sickle Cell Related

Save & Exit Form

Save & Stay

-- Cancel --

Editing existing Medical Record Number **30384544**

Event Name: **Time point 4**

Medical Record Number

30384544

Six Month Interval Date



2019-09-13



Today

Y-M-D



yyyy-mm-dd

Medical history - SCD related



- ☐ Acute chest syndrome (oxygen requirement)
- ☐ Aplastic Crisis/parvovirus
- ☐ Avascular necrosis (AVN)
- ☐ Cardiac failure/dysfunction
- ☐ Cholelithiasis (s/p cholecystectomy)
- ☐ Dactylitis
- ☐ Delayed puberty
- ☐ Enuresis
- ☐ Erectile dysfunction
- ☐ Growth delay
- ☐ Headaches
- ☐ Hepatic sequestration
- ☐ Hypersplenism/splenic sequestration (on-going)
- ☒ Hypersplenism/splenic sequestration (s/p splenectomy)
- ☐ Iron overload (Ferritin>500)
- ☐ Leg ulcers
- ☐ Neurologic deficit
- ☐ Osteomyelitis
- ☐ Priapism
- ☐ Pulmonary embolism
- ☐ Pulmonary hypertension
- ☐ Retinopathy
- ☐ Sepsis/Bacteremia - Non-pneumococcal bacterial
- ☐ Sepsis/Bacteremia - Pneumococcal
- ☐ Sepsis - non-bacteremial (blood culture -)
- ☐ Silent infarct
- ☐ Stroke
- ☐ TCD abnormal
- ☐ Thrombosis (other than PE)
- ☐ Transient Ischemic Attack
- ☐ Vasculopathy (stenosis), cerebral
- ☐ Vaso-occlusive pain crisis
- ☐ Other

Check All That Apply

Date Diagnosed: Hypersplenism (s/p splenectomy)



2018-05

yyyy-mm

Comments



 Editing existing Medical Record Number **30384544**

Event Name: **Time point 3**

Medical Record Number

30384544

Six Month Interval Date



2019-03-13



Today

Y-M-D

yyyy-mm-dd

Hydroxyurea?



☒ Yes

☐ No

[reset](#)

Hydroxyurea Dose (mg/kg/day)



26.5

mg/kg/day

Hydroxyurea Start Date



2018-07

yyyy-mm

Currently on Hydroxyurea?



☒ Yes

☐ No

[reset](#)

Folic Acid?



☐ Yes

☒ No

[reset](#)

Penicillin?



☒ Yes

☐ No

[reset](#)

Penicillin Start Date



2017-11

yyyy-mm

Currently on Penicillin?



☒ Yes

☐ No

[reset](#)

Iron Chelator



☐ Exjade (deferasirox)

☐ Desferal (deferrioxamine)

☐ Jadenu (deferasirox)

☐ Ferriprox (deferiprone)

☐ Other

☒ None

Hospitalizations



 Editing existing Medical Record Number **30384544**


Event Name: **Time point 3**

Medical Record Number

30384544

Six Month Interval Date

  Today Y-M-D


 yyyy-mm-dd

Hospitalizations summary

Number of Hospitalizations in Interval





Dates of Hospitalizations in Interval


Expand

YYYY-MM

Diagnoses During Hospitalizations in Interval (Check all that apply)

-  
- ☒ acute chest syndrome (oxygen requirement)
 - ☐ aplastic crisis
 - ☐ asthma exacerbation
 - ☐ bacteremia
 - ☐ cholecystitis/cholelithiasis
 - ☐ dactylitis
 - ☐ dehydration
 - ☐ fever
 - ☐ hepatic sequestration
 - ☐ influenza
 - ☐ other infection
 - ☐ parvovirus infection
 - ☐ pneumonia
 - ☐ pulmonary embolus
 - ☒ scheduled surgery/procedure
 - ☐ splenic sequestration
 - ☐ stroke
 - ☐ urinary tract infection/pyelonephritis
 - ☐ viral illness without specific diagnosis
 - ☐ vaso-occlusive pain crisis
 - ☐ other

[Check All That Apply](#)

If Surgery, Specify


Expand

[List All](#)

☐ Blood culture

Editing existing Medical Record Number 30384544

Event Name: Time point 3

Medical Record Number 30384544

Six Month Interval Date



2019-03-13



Today

Y-M-D



yyyy-mm-dd

Transfusion in Interval?

☐ Yes☐ No

reset

Splenomegaly?

☐ Yes☐ No

reset

Hematologic Indices

Date of lab (Most Recent)



2019-03-02



yyyy-mm-dd

Hgb Number (g/dL)



9.2



g/dL

HCT (%)



25.4



%

Retic %



6.2



Absolute Reticulocyte Number



181.7



MCV (fL)



86.4



fL

WBC Total (thousands/uL)



24.74



thousands/uL

Neutrophil Count (thousands/uL)



52.1



thousands/uL

PLT (thousands/uL)



467



thousands/uL

Bilirubin Total (mg/dL)



mg/dL

LDH (U/L)



U/L

Hemoglobin Profile

Date of lab (Most Recent)



2019-02-28



yyyy-mm-dd

Hgb A %



Hgb A2 %



Hgb F %



28.6



Hgb S %



Methods – Analysis

Inclusion criteria:

1. Hgb SS/Sbeta0 born since January 2011
2. Continuously on HU since infancy (starting 9-12 months of age)
3. Continuously followed in the No Va peds sickle cell program
4. At least 2 years of age at the time of analysis (June 2018) to allow for at least 1 yr HU treatment

Exclusion criteria:

1. Children who moved to our clinic after age 1 – no verifiable historical data
2. Children who did not start HU by age 1

Parameters analyzed:

Hgb, Hgb F

Hospitalizations, ED visits, transfusions

Results – continuous HU therapy starting in infancy

24 children, 95 person-years

TABLE 1 Hemoglobin (g/dL) and fetal hemoglobin (%) of patients on HU by age 1 at 6-month intervals

				Years followed																											
				0.5 year		1 year		1.5 years		2 years		2.5 years		3 years		3.5 years		4 years		4.5 years		5 years		5.5 years		6 years		6.5 years		7 years	
Patient	Dx	M/F	Age at HU prescription	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF	Hgb	HgbF		
1	SS	M	9 months	9	26.2	10.5	29	10.6	30.3	10.3	29.3																				
2	SS	F	1 year	10.5	27.9	9.6	25.5	10.2	35.9	10.2	34.3																				
3	SS	M	9 months	7.4	36.2	8.2	26.1	9.5	36.6	9.3	33.5																				
4	SS	M	9 months	9.4	34.5	10	23.6	10.9	40	11.1	33.3	9.9	34.4																		
5	SS	M	1 year	10.8	44.6	10.5	42.1	11.1	43.5	11.3	37.1	11	41.2																		
6	SS	M	9 months	8.7	32.2	9.5	24.7	9.9	33.4	10.1	28	10.2	30.6																		
7	SS	F	9 months	10.6	34	10.7	23.5	11.5	26.6	10.6	25.8	10.9	24.4																		
8	SS	M	9 months	9.2	38.4	9.4	29.9	9.8	34.8	9.7	35.3	9.5	28.7	8.6	29.8																
9	SS	F	10 months	10.3	32.5	11.3	28.3	11.3	35	11.8	34.5	12.1	37.2	11.8	33.5																
10	SS	M	9 months	9.8	24.8	10.4	25.6	11.5	26.9	9.9	28	11.3	34.1	11.2	29.4																
11	SS	M	9 months	9.2		11.3	32.8	11.1	38.9	11.2	28.5	11.3	32.8	9.4	28.2																
12	SS	F	11 months	10.8	38.3	10.8	25.8	10.6	31.5	11	29.1	11.9	30.3	12.1	28.6	11.3	27.4														
13	SS	F	11 months	9.7	37.4	10.6	29.8	11.6	32.6	11	43.1	11.6	41.7	11.8	37	11.4	38.9														
14	SS	M	10 months	8.7	33.4	9.8	31	10.9	38.6	11.2	32.3	10.5	37.9	10.6	33.4	11.3	32.8	10.8	28.9												
15	SS	M	7 months	9.6	39.5	10.1	26.2	10.6	27.6	10.2	29.2	8.9	24	8.4	18.6	8.9	21.1	9.2	29.2												
16	SS	F	9 months	8	33.4	9.7	28.8	10.7	38.2	10.6	37.4	10.6	35.7	10.2	34.4	11.3	35.8	11.2	40.3	10.6	40.7										
17	SS	M	9 months	9.3	34	10.4	35.7	12.1	41.9	11.9	39.3	11.6	32.4	11.3	33.1	10.6	31.8	11.2	30	8.6	31.2										
18	SS	M	1 year	9.3		10.2	19.9	10.5	21.3	9.5		9.6	31.9	10.5	37.8	9.6	32.9	9.5	30.3	9.4	32.4	10	29.8								
19	SS	M	1 year	9.1	37	9	34.5	10.3	33.9	9.2	34.5	8.8	35.8	8.9	33.9	8.9	28.3	8.9	27.5	9.1	27.1	8.2	29.8	8.7	27.7						
20	SS	F	6 months	8.4	23.4	7.9	29.4	8.5	25.6	8.9	29.5	9.6	22	9.5	29.1	9.9	28.4	10.2	28.3	9.9	28.8	9.1	27.1	9.2	33.9						
21	SS	F	1 year	9.8	38.8	12.2	28.4	11.3	30.9	12	31.5	12.3	31	11.5	27.6	11.4	25.8	11.7	33.1	12.7	31.4	11.5	34.6	12.4	34.6	12.3	31.3				
22	SS	F	1 year	10.2	31.4	10.3	30.5	10	31.2	9.9	31.4	9.2	21	8.5	26	9.3	24.2	10.1	24.8	9.6	22.4	9.2	31.6	8.9	22.6	9	23.8	9.4	25.6	8.1	29.6
23	SS	F	1 year	10.5		9.9	31	11.6	41.8	11.7	43.4	11.7	43	11.3	42.3	11.6	37	10.9	26.8	11.4	29.5	11.2	27.2	11.5	34.1	11.3	32.6	10.8	33.5	11.7	35
24	SS	F	1 year	9.1		10.3	29.4	10.7	36.6	10.6	39.5	10.7	35.3	10.9	32.5	9.7	32.2	9.6	34	9.4	31.1	9	22.8	8.9	21.5	9.6	27.4	9.9	24.2	8.7	25.8

Abbreviations: F, female; Hgb, hemoglobin; HgbF, fetal hemoglobin; HU, hydroxyurea; M, male.

Results – continuous HU therapy starting in infancy duration of followup

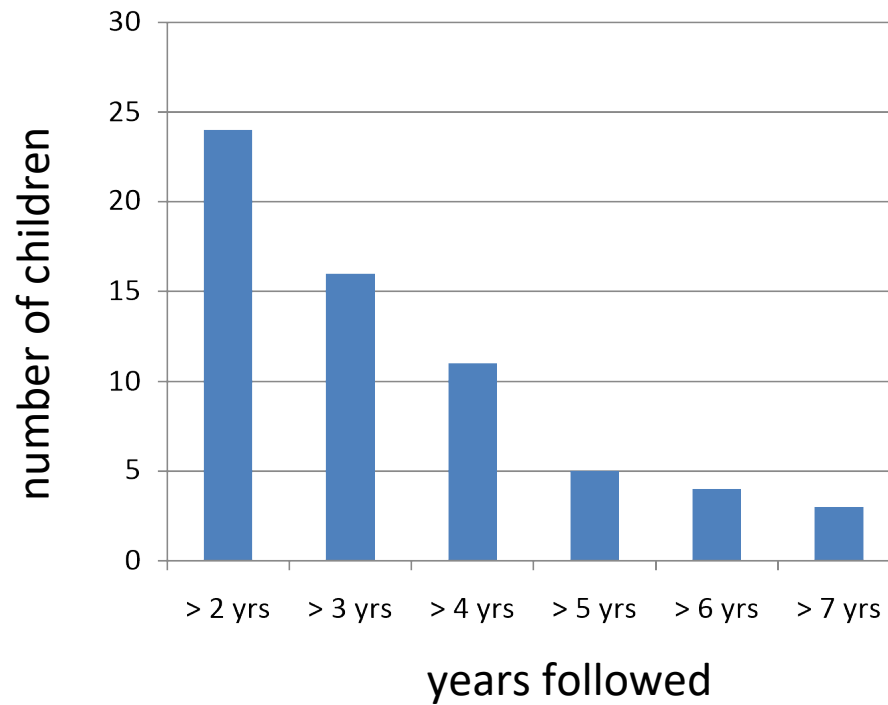
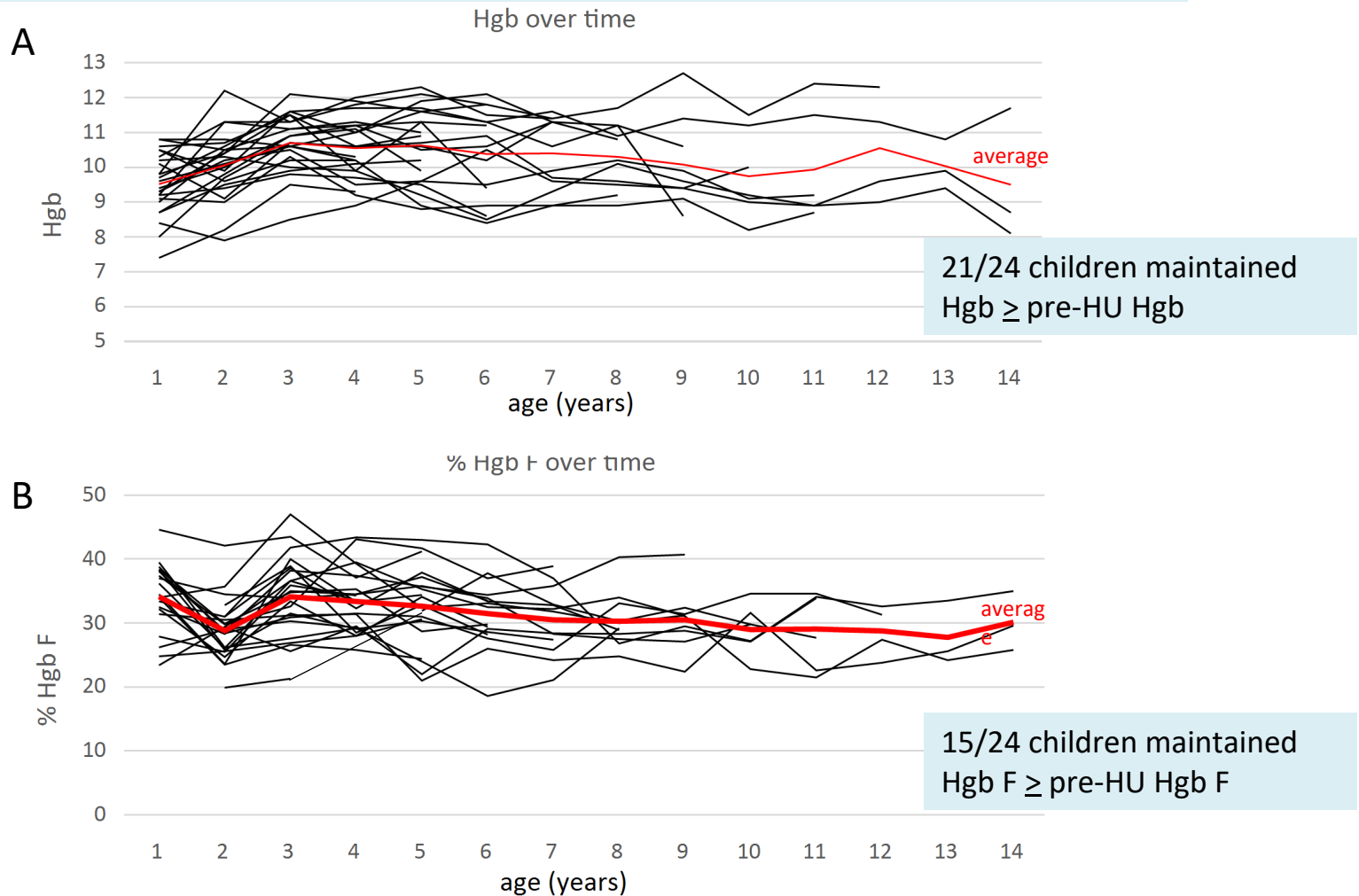


Figure 1 Longitudinal Hgb and Hgb F in patients started on HU by age 1



C

Age at follow-up (years)	0.5	1	1.5	2	2.5	3	3.5	4	4.5	5	5.5	6	6.5	7
# pts w/ continuous f/u	24	24	24	24	19	16	11	11	8	7	6	4	3	3
average Hgb (g/dL)	9.5	10.1	10.7	10.6	10.6	10.4	10.4	10.3	10.1	9.7	9.9	10.6	10	9.5
\pm stdev	0.9	0.9	0.8	0.9	1.1	1.3	1.0	0.9	1.3	1.2	1.6	1.5	0.7	1.9
average Hgb F (%)	34.1	28.9	34.1	33.4	32.6	31.5	30.5	30.3	30.5	29	29.1	28.8	27.8	30.1
\pm stdev	5.1	4.7	6.6	4.9	6.2	5.3	5.2	4.2	4.9	3.8	6.0	4.0	5.0	4.6
average HU dose (mg/kg/d)	0	22.6	23.9	24.2	24.6	25.6	26.4	26.6	27.4	27.1	27.3	25.8	29.3	29
\pm stdev		5.2	5.7	4.8	4.8	5.6	5.0	5.0	3.2	4.0	4.3	3.0	2.9	4.6

Hgb range 8.1 – 12.7

Hgb F range 18.6% – 43.5%

HU dose range 15 – 35 mg/kg/d

Results - continuous HU therapy starting in infancy

Table 2 Hospitalizations, treat-and-release ED visits, transfusions in 6-month intervals

age range	0-0.5	0.5-1	1-1.5	1.5-2	2-2.5	2.5-3	3-3.5	3.5-4	4-4.5	4.5-5	5-5.5	5.5-6	6-6.5	6.5-7	total
# Hospitalizations (all)	6	10	2	3	4	2	0	0	0	0	0	0	0	0	27
fever, infections	5	9	2	2	0	2									20
pain	1	0	0	0	0	0									1
splenic sequestration	0	1	0	0	4	0									5
other^	0	1	0	1	0	0									2
# ED visits (all)	7	17	11	15	6	4	2	2	2	1	1	0	0	0	68
fever, infection	4	13	10	10	4	4	2	2	2	1	1				53
pain	1	1	0	0	0	0	0	0	0	0	0				2
viral*	1	2	1	4	1	0	0	0	0	0	0				9
other**	1	1	0	1	1	0	0	0	0	0	0				4
# transfusions (all)	1	1	0	0	3	0	0	0	0	0	0	0	0	0	5
splenic sequestration	1	1			3										5

other^: 1 cyanotic episode, 1 accidental drug ingestion

viral*: upper respiratory infections, bronchiolitis, gastroenteritis

other**: constipation, laceration, synovitis

Total # hosp: 27

Age of hosp: 16/27 during age < 1; 27/27 during age ≤ 3

Hospitalization rate: 0.28/person-year (27hosp/95 person-yrs)

Reason for hosp: fever/infection > splenic sequestration > other
4 pulm infiltrates, no hypoxia, no txf required

Results - continuous HU therapy starting in infancy

Table 2 Hospitalizations, treat-and-release ED visits, transfusions in 6-month intervals

age range	0-0.5	0.5-1	1-1.5	1.5-2	2-2.5	2.5-3	3-3.5	3.5-4	4-4.5	4.5-5	5-5.5	5.5-6	6-6.5	6.5-7	total
# Hospitalizations (all)	6	10	2	3	4	2	0	0	0	0	0	0	0	0	27
fever, infections	5	9	2	2	0	2									20
pain	1	0	0	0	0	0									1
splenic sequestration	0	1	0	0	4	0									5
other^	0	1	0	1	0	0									2
# ED visits (all)	7	17	11	15	6	4	2	2	2	1	1	0	0	0	68
fever, infection	4	13	10	10	4	4	2	2	2	1	1				53
pain	1	1	0	0	0	0	0	0	0	0	0				2
viral*	1	2	1	4	1	0	0	0	0	0	0				9
other**	1	1	0	1	1	0	0	0	0	0	0				4
# transfusions (all)	1	1	0	0	3	0	0	0	0	0	0	0	0	0	5
splenic sequestration	1	1			3										5

other^: 1 cyanotic episode, 1 accidental drug ingestion

viral*: upper respiratory infections, bronchiolitis, gastroenteritis

other**: constipation, laceration, synovitis

Total # ED: 68

Age of ED: 50/68 during age ≤ 2 , 60/68 during age ≤ 3

Pts with ED: among age 0-3: 50% pts visited ED each yr

ED rate: 0.72/person-year (68 EDs/95 person-yrs)

Reason for ED: 91 % fever/infection (62/68), 3% (2/68) pain (pre-HU)

Results - continuous HU therapy starting in infancy

Table 2 Hospitalizations, treat-and-release ED visits, transfusions in 6-month intervals

age range	0-0.5	0.5-1	1-1.5	1.5-2	2-2.5	2.5-3	3-3.5	3.5-4	4-4.5	4.5-5	5-5.5	5.5-6	6-6.5	6.5-7	total
# Hospitalizations (all)	6	10	2	3	4	2	0	0	0	0	0	0	0	0	27
fever, infections	5	9	2	2	0	2									20
pain	1	0	0	0	0	0									1
splenic sequestration	0	1	0	0	4	0									5
other^	0	1	0	1	0	0									2
# ED visits (all)	7	17	11	15	6	4	2	2	2	1	1	0	0	0	68
fever, infection	4	13	10	10	4	4	2	2	2	1	1				53
pain	1	1	0	0	0	0	0	0	0	0	0				2
viral*	1	2	1	4	1	0	0	0	0	0	0				9
other**	1	1	0	1	1	0	0	0	0	0	0				4
# transfusions (all)	1	1	0	0	3	0	0	0	0	0	0	0	0	0	5
splenic sequestration	1	1			3										5

other^: 1 cyanotic episode, 1 accidental drug ingestion

viral*: upper respiratory infections, bronchiolitis, gastroenteritis

other**: constipation, laceration, synovitis

Total # transfusions: 5

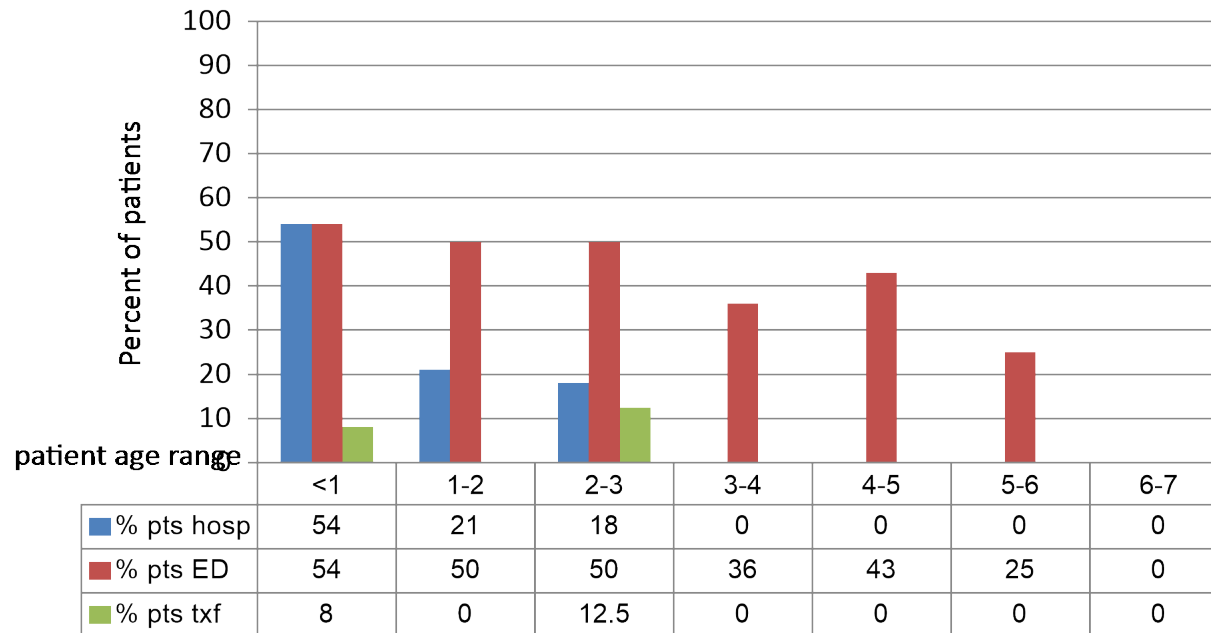
pts transfused: 2

Transfusion rate: 0.05/person-year (5 txf/95 person-yrs)

Reason for transfusion: splenic sequestration

Results - continuous HU therapy starting in infancy

Percent of patients utilizing hospitalizations, ED visits, and transfusions



Conclusions

Starting HU in infancy and maintaining maximal HU therapy continuously resulted in:

- maintainence of Hgb \geq pre-HU hgb in most children, with average Hgb ~ 10

- maintainence of Hgb F \geq pre-HU hgb F in more than half of the children,
with average Hgb F $\sim 30\%$

- hosp rate 0.28/person-yr, the majority for fever/infection in first few yrs of life

- transfusion rate of 0.05/person-yr, for splenic sequestration only

- no CNS events

- no pain crisis needing medical attention after initiation of HU

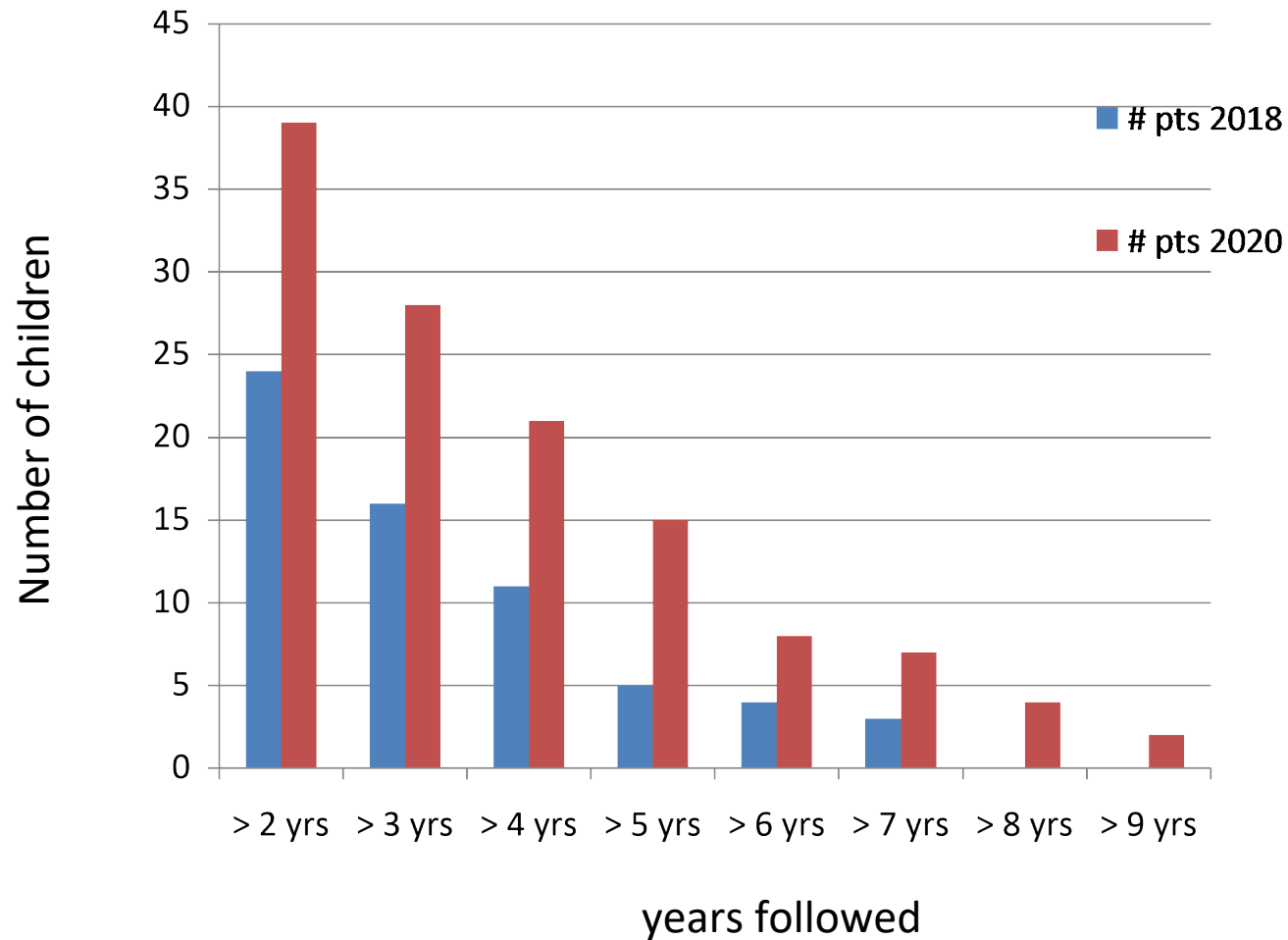
- no acute chest syndrome requiring transfusion (except after surgery)

- no significant change in ED visits, the vast majority of which were for fever

2020 update - continuous HU therapy starting in infancy

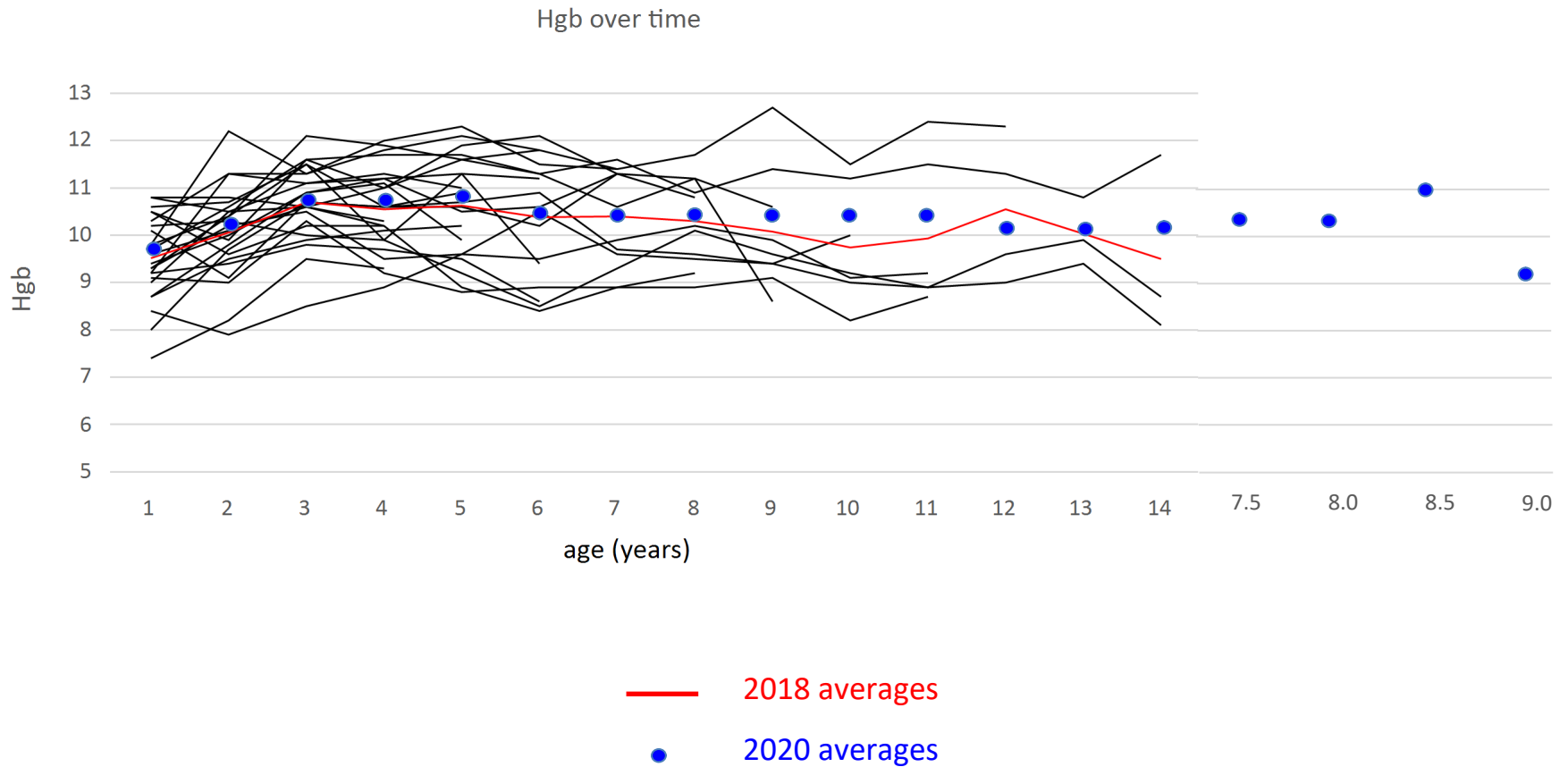
6/2018: 95 person-yrs

8/2020: 124.5 person-yrs



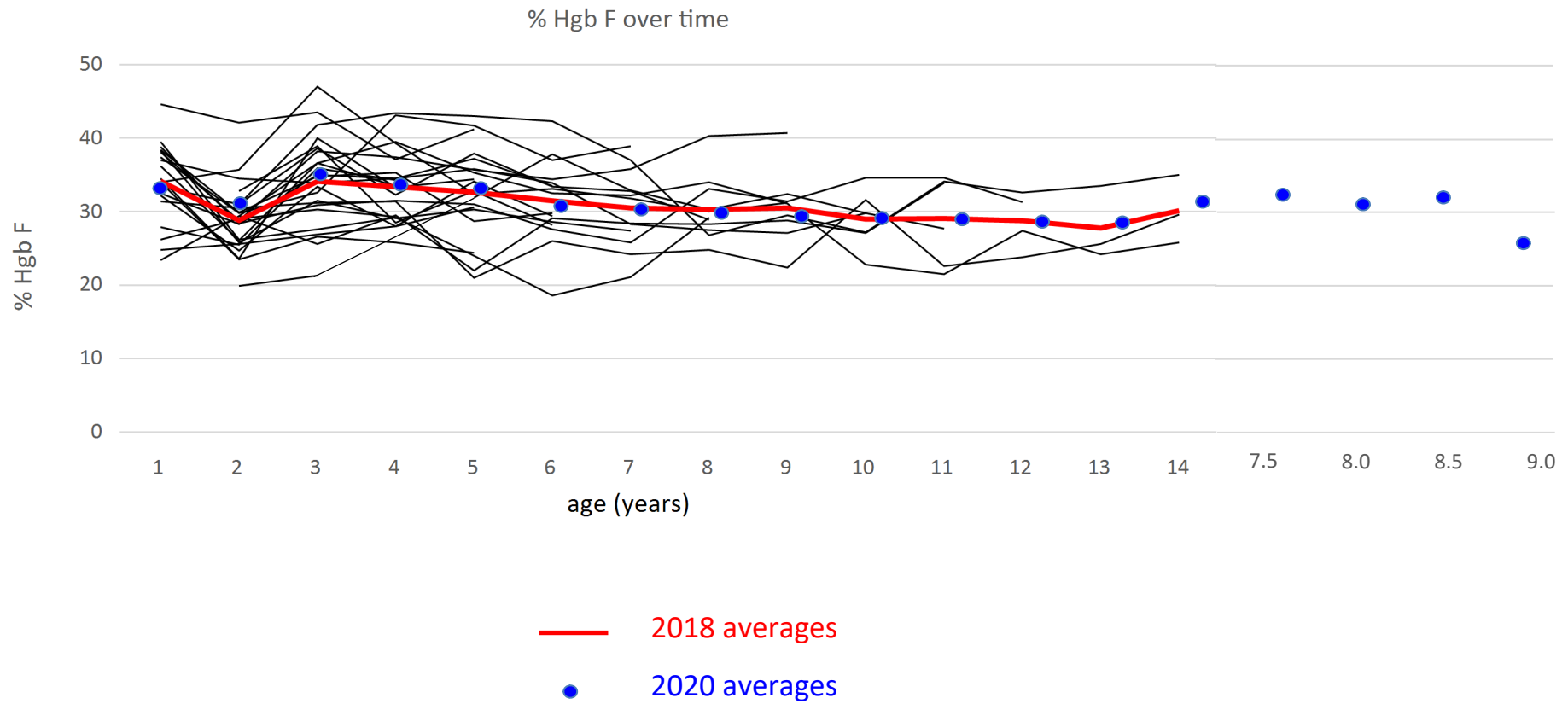
2020 update

Longitudinal Hgb in patients started on HU by age 1



2020 update

Longitudinal Hgb F in patients started on HU by age 1



All children born after January 2011 eligible for HU
(at least 1 yr old and including those who

transfused in)

74 children

72/74 on HU

No conditional or abnormal TCD, no strokes

Minimal pain

Only 1 acute chest (post-op)

Infection/fever is the most common cause of hospitalizations and ED visits in younger children

Splenic sequestration now the most common reason for transfusion among children on HU

Factors contributing to our outcomes

1. Unified HU message and concerted effort from sickle cell team
1. Start HU message from very first clinic visit
1. Push HU dose to MTD (allow ANC cutoff 1000)
1. Check Hgb F at every monitoring visit to aim for Hgb F 30%

Message:

**Excellent outcomes can be achieved
even in the community practice setting**

Limitations

Small sample size

Skew toward younger children

Single-center study

Big question:

What will happen when this cohort become teenagers?