General

Title
Sickle cell disease (SCD): percentage of children younger than 18 years of age identified as having sickle cell anemia who received anticipatory guidance regarding the risks and benefits of treatment with hydroxyurea as part of outpatient care during the measurement year.

Source(s)

Measure Domain

Primary Measure Domain
Clinical Quality Measures: Process

Secondary Measure Domain
Does not apply to this measure

Brief Abstract

Description
This measure is used to assess the percentage of children younger than 18 years of age identified as having sickle cell anemia who received anticipatory guidance regarding the risks and benefits of treatment with hydroxyurea as part of outpatient care during the measurement year. A higher proportion indicates better performance, as reflected by appropriate treatment.

Rationale
Approximately 2,000 infants are born with sickle cell disease (SCD) in the United States (U.S.) each year, a condition that occurs predominantly in people of African and Hispanic descent. SCD is a chronic blood disorder, characterized by the presence of hemoglobin S (Hb S). From infancy onward, the presence of this hemoglobin variant can lead to an array of serious medical conditions. Hydroxyurea is a drug that increases the percentage of fetal hemoglobin (Hb F) in the blood, a variant that is protective against the clinical severity of sickle cell anemia (SCA), which is caused by Hb S. Hydroxyurea is indicated for pediatric patients with SCA who have recurrent vaso-occlusive events, including frequent pain episodes and acute chest syndrome. While abundant evidence has been gathered over the years supporting its efficacy in children and its low
toxicity, the drug is currently only approved by the U.S. Food and Drug Administration for use in adults. Although off-label use in children with SCA is supported by the National Institutes of Health (NIH) and the National Heart, Lung and Blood Institute (NHLBI), use of hydroxyurea therapy in children with SCA remains low. Possible reasons for this include inadequate knowledge among health care providers, financial challenges for patients, and lack of capacity in medical delivery system to support the volume of patient encounters necessary for sustained use of the drug. For patients receiving anticipatory guidance, the two most important long-term concerns to discuss with families are potential treatment-related infertility (no major relationship yet seen) and cancer (anecdotes exist but there are no assumptions of causality). As patient years of treatment accumulate, concerns about serious long-term consequences are abating at many academic programs. While barriers remain, evidence suggests that hydroxyurea treatment is of substantial benefit to many young children with SCA and that treatment should begin before the onset of substantial organ damage.

Evidence for Rationale


Primary Health Components

Sickle cell disease (SCD); sickle cell anemia; hydroxyurea; anticipatory guidance; infants; children; adolescents

Denominator Description

The eligible population for the denominator is the number of children younger than 18 years of age with sickle cell anemia (SCA) who received outpatient care during the measurement year (January 1 to December 31) (see the related "Denominator Inclusions/Exclusions" field).

Numerator Description

The eligible population for the numerator is the number of children younger than 18 years of age with sickle cell anemia (SCA) who received anticipatory guidance regarding the risks and benefits of treatment with hydroxyurea as part of outpatient care during the measurement year (January 1 to December 31) (see the related "Numerator Inclusions/Exclusions" field).

Evidence Supporting the Measure

Type of Evidence Supporting the Criterion of Quality for the Measure

A clinical practice guideline or other peer-reviewed synthesis of the clinical research evidence

A formal consensus procedure, involving experts in relevant clinical, methodological, public health and organizational sciences

A systematic review of the clinical research literature (e.g., Cochrane Review)

One or more research studies published in a National Library of Medicine (NLM) indexed, peer-reviewed journal

Additional Information Supporting Need for the Measure

Sickle Cell Disease Prevalence and Incidence

Sickle cell disease (SCD) is one of the most common genetic disorders in the United States (U.S.) (Kavanagh et al., 2011). The National Heart, Lung and Blood Institute (NHLBI) (2002) estimates that 2,000 infants are born with SCD in the U.S. each year. SCD affects 70,000 to 100,000 children and adults in the U.S., predominantly those of African and Hispanic descent (Hassell, 2010). The condition is chronic, lifelong, and associated with a decreased lifespan.
**Sickle Cell Disease Pathology and Severity**

Vaso-occlusion (the sudden blockage of a blood vessel caused by the sickle shape of abnormal blood cells) is responsible for most complications of SCD, including pain episodes, sepsis, stroke, acute chest syndrome, priapism, leg ulcers, osteonecrosis and renal insufficiency (Steinberg, 1999). In addition, SCD can have hemolytic and infectious complications that result in morbidity and mortality in children with the condition (Kavanagh et al., 2011).

**Sickle Cell Disease Burden in Daily Life**

The effect of SCD on children and families is significant; severe pain episodes and hospitalizations restrict daily activities and reflect negatively on school attendance and performance, as well as on sleep and social activities (Lemanek, Ranalli, & Lukens, 2009; Alvim et al., 2005). Although medical management of SCD continues to improve over time, 196 children in the United States died from SCD-related causes between 1999 and 2002 (Yanni et al., 2009).

**Sickle Cell Disease Cost**

In a study of health care utilization among low income children with SCD between 2004 and 2007, 27% of these children required inpatient hospitalization and 39% used emergency care during a year. Of these children, 63% averaged one well-child visit per year and 10% had at least one outpatient visit with a specialist (Raphael et al., 2009). Patients with SCD use many parts of the health care system, incurring significant costs. In 2009, mean hospital charges for children with SCD and a hospital stay were $23,000 for children with private insurance and $18,200 for children enrolled in Medicaid (HCUPnet, Healthcare Cost and Utilization Project, 2012). Kauf et al. (2009) estimate the lifetime cost of health care per patient with SCD to be approximately $460,000.

See the original measure documentation for additional evidence supporting the measure.

**Evidence for Additional Information Supporting Need for the Measure**

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Extent of Measure Testing

Reliability

Data and Methods. The testing data consisted of an audit of medical records from the three largest centers serving sickle cell disease (SCD) patients in Michigan during 2012: Children's Hospital of Michigan (CHM, Detroit), Hurley Medical Center (Hurley, Flint), and the University of Michigan Health System (UMHS, Ann Arbor). Combined, these sites treat the majority of children with SCD in Michigan. Medical records for all children with SCD meeting the measure specification criteria during the measurement year were abstracted at each site. Abstracting was conducted in two phases; during Phase 1, 435 records were abstracted among the three sites. In Phase 2, an additional 237 cases were abstracted at one site. In total, 672 unique records were reviewed for children with SCD to test this measure.

Reliability of medical record data was determined through re-abstraction of patient record data to calculate the inter-rater reliability (IRR) between abstractors. Broadly, IRR is the extent to which the abstracted information is collected in a consistent manner (Keyton et al., 2004). Low IRR may be a sign of poorly executed abstraction procedures, such as ambiguous wording in the data collection tool, inadequate abstractor training, or abstractor fatigue. For this measure, the medical record data collected by two nurse abstractors were compared.

Measuring IRR at the beginning of the abstraction is imperative to identify any misinterpretations early on. It is also important to assess IRR throughout the abstraction process to ensure that the collected data maintain high reliability standards. Therefore, the IRR was evaluated during Phase 1 at each site to address any reliability issues before beginning data abstraction at the next site.

IRR was determined by calculating both percent agreement and Kappa statistics. While abstraction was still being conducted at each site, IRR assessments were conducted for 5% of the total set of unique patient records that were abstracted during Phase 1 of data collection. Two abstractors reviewed the same medical records; findings from these abstractions were then compared, and a list of discrepancies was created.

Three separate IRR meetings were conducted, all of which included a review of multiple SCD measures that were being evaluated. Because of eligibility criteria, not all patients were eligible for all measures. Therefore, records for IRR were not chosen completely at random; rather, records were selected to maximize the number of measures assessed for IRR at each site.

Results. For this measure, 16 of 435 unique patient records (4%) from Phase 1 of the abstraction process were assessed for IRR across the three testing sites.

Table 5 of the original measure documentation shows the percent agreement and Kappa statistic for the measure numerator for each site and across all sites. The agreement for this measure is 100% and the Kappa is 1.00, indicating that a perfect IRR level was achieved.

Validity

Face Validity. The face validity of this measure was established by a national panel of experts and advocates for families of children with SCD convened by the Quality Measurement, Evaluation, Testing, Review, and Implementation Consortium (Q-METRIC). The Q-METRIC expert panel included nationally recognized experts in SCD, representing hematology, pediatrics, and SCD family advocacy. In addition, measure validity was considered by experts in state Medicaid program operations, health plan quality measurement, health informatics, and health care quality measurement. In total, the Q-METRIC SCD panel included 14 experts, providing a comprehensive perspective on SCD management and the measurement of quality metrics for states and health plans.

The Q-METRIC expert panel concluded that this measure has a high degree of face validity through a detailed review of concepts and metrics considered to be essential to effective SCD management and treatment. Concepts and draft measures were rated by this group for their relative importance. This measure was highly rated, receiving an average score of 8.9 (with 9 as the highest possible score).

Validity of Abstracted Data. This measure was tested using medical record data which is considered the gold standard for clinical information and had a high degree of face validity and reliability. This measure was tested among a total of 310 children younger than 18 years of age with SCD (Table 6 of the original measure documentation). Overall, 23% of children with SCD received anticipatory guidance regarding the risks and benefits of treatment with hydroxyurea as part of outpatient care (range: 17% to 51%).

Evidence for Extent of Measure Testing
State of Use of the Measure

State of Use

Current routine use

Current Use

not defined yet

Application of the Measure in its Current Use

Measurement Setting

Ambulatory/Office-based Care

Hospital Outpatient

Professionals Involved in Delivery of Health Services

not defined yet

Least Aggregated Level of Services Delivery Addressed

Single Health Care Delivery or Public Health Organizations

Statement of Acceptable Minimum Sample Size

Unspecified

Target Population Age

Age less than 18 years

Target Population Gender

Either male or female
National Strategy for Quality Improvement in Health Care

National Quality Strategy Aim
Better Care

National Quality Strategy Priority
Person- and Family-centered Care
Prevention and Treatment of Leading Causes of Mortality

Institute of Medicine (IOM) National Health Care Quality Report Categories

IOM Care Need
Living with Illness

IOM Domain
Effectiveness
Patient-centeredness

Data Collection for the Measure

Case Finding Period
The measurement year

Denominator Sampling Frame
Patients associated with provider

Denominator (Index) Event or Characteristic
Clinical Condition
Diagnostic Evaluation
Encounter
Patient/Individual (Consumer) Characteristic
Therapeutic Intervention

Denominator Time Window
Denominator Inclusions/Exclusions

Inclusions
The eligible population for the denominator is the number of children younger than 18 years of age with sickle cell anemia (SCA) who received outpatient care during the measurement year (January 1 to December 31).

Note:
- Eligible children are restricted to those diagnosed with SCA, determined by hemoglobin variants identified in Table 1 of the original measure documentation, with the appropriate International Classification of Diseases, Ninth Revision (ICD-9) codes documented in the medical record.
- **Intake Period:** January 1 through December 31 of the measurement year.
- **Outpatient Care:** A Health Maintenance Exam (HME) or an Evaluation and Management (E&M) visit with primary care provider or a specialist (refer to Table 2 of the original measure documentation).

Exclusions
- Inpatient stays, emergency department visits, and urgent care visits are excluded from the calculation.
- Children with a diagnosis in the sampled medical record indicating one of the sickle cell disease (SCD) variants listed in Table 3 of the original measure documentation should not be included in the eligible population unless there is also a diagnosis for a sickle cell variant listed in Table 1.

Exclusions/Exceptions

not defined yet

Numerator Inclusions/Exclusions

Inclusions
The eligible population for the numerator is the number of children younger than 18 years of age with sickle cell anemia (SCA) who received anticipatory guidance regarding the risks and benefits of treatment with hydroxyurea as part of outpatient care during the measurement year (January 1 to December 31).

Note:
- *Anticipatory guidance* is any written or face-to-face verbal communication regarding the risks and benefits of treatment with hydroxyurea as part of outpatient care with patient, parent, or family member.
- Evidence of anticipatory guidance is determined through medical record review. Documentation in the medical record must include, at minimum, a note containing the date on which verbal or written anticipatory guidance was provided.

Exclusions
Unspecified

Numerator Search Strategy

Fixed time period or point in time

Data Source
Electronic health/medical record

Type of Health State

Does not apply to this measure
Instruments Used and/or Associated with the Measure
Unspecified

Computation of the Measure

Measure Specifies Disaggregation
Does not apply to this measure

Scoring
Rate/Proportion

Interpretation of Score
Desired value is a higher score

Allowance for Patient or Population Factors
not defined yet

Standard of Comparison
not defined yet

Identifying Information

Original Title
Anticipatory guidance regarding hydroxyurea treatment for children with sickle cell disease.

Measure Collection Name
Sickle Cell Disease Measures

Submitter

Developer

Funding Source(s)
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Financial Disclosures/Other Potential Conflicts of Interest

Unspecified

Adaptation

This measure was not adapted from another source.

Date of Most Current Version in NQMC

2014 Apr

Measure Maintenance

Unspecified

Date of Next Anticipated Revision

Unspecified

Measure Status

This is the current release of the measure.
The measure developer reaffirmed the currency of this measure in January 2016.

Measure Availability


For more information, contact Q-METRIC at 300 North Ingalls Street, Room 6C08, SPC 5456, Ann Arbor, MI 48109-5456; Phone: 734-232-0657; Fax: 734-764-2599.

NQMC Status

This NQMC summary was completed by ECRI Institute on January 23, 2015. This NQMC summary was verified by the measure developer on March 2, 2015.

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Production

Source(s)


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