Timeliness of Antibiotic Prophylaxis for Children with Sickle Cell Disease

Section 1. Basic Measure Information

1.A. Measure Name

Timeliness of Antibiotic Prophylaxis for Children with Sickle Cell Disease

1.B. Measure Number

0135

1.C. Measure Description

Please provide a non-technical description of the measure that conveys what it measures to a broad audience.

This measure assesses the percentage of children with a newborn screen positive for sickle cell disease (SCD) who receive appropriate preventive antibiotics by 3 months of age. Preventive antibiotics reduce the risk of life-threatening infections for children with SCD in this age group. This measure is meant to be implemented with data maintained by State newborn screening (NBS) programs.

Spleen damage is a common and crucial characteristic of SCD. A damaged spleen cannot effectively clear bacteria from the blood, leaving SCD patients, particularly young children, highly susceptible to infection. Children with SCD have rates of infection caused by bacterial pneumonia 30-100 times that of children without SCD, and pneumonia vaccines are of limited effectiveness in this age group because of low antibody response. However, twice-daily doses of an antibiotic sharply reduce the incidence of pneumonia in children with SCD.

Studies have shown that children with SCD who are enrolled in Medicaid frequently are not dispensed medication soon enough or in sufficient quantities to cover ongoing daily use of an antibiotic. Sometimes these children receive no antibiotics at all, even though this simple precaution greatly reduces their risk of contracting a debilitating and often deadly infection. Clinical guidelines and the results of randomized controlled trials indicate that providers should prescribe appropriate antibiotic prophylaxis to children with SCD who are under 5 years of age. There are no existing quality measures for antibiotic prophylaxis in children with SCD.

1.D. Measure Owner

The Quality Measurement, Evaluation, Testing, Review, and Implementation Consortium (Q-METRIC).

1.E. National Quality Forum (NQF) ID (if applicable)

Not applicable.

1.F. Measure Hierarchy

Please note here if the measure is part of a measure hierarchy or is part of a measure group or composite measure. The following definitions are used by AHRQ:

1. **Please identify the name of the collection of measures to which the measure belongs (if applicable). A collection is the highest possible level of the measure hierarchy. A collection may contain one or more sets, subsets, composites, and/or individual measures.**

   This measure is part of the Q-METRIC Sickle Cell Disease Measures collection.

2. **Please identify the name of the measure set to which the measure belongs (if applicable). A set is the second level of the hierarchy. A set may include one or more subsets, composites, and/or individual measures.**

   This measure is part of the Q-METRIC Sickle Cell Disease Administrative Claims set.

3. **Please identify the name of the subset to which the measure belongs (if applicable). A subset is the third level of the hierarchy. A subset may include one or more composites, and/or individual measures.**

   Not applicable.

4. **Please identify the name of the composite measure to which the measure belongs (if applicable). A composite is a measure with a score that is an aggregate of scores from other measures. A composite may include one or more other composites and/or individual measures. Composites may comprise component measures that can or cannot be used on their own.**

   Not applicable.

1.G. Numerator Statement

The number of eligible children who received appropriate antibiotic prophylaxis within 90 days of age. Eligible children are restricted to three hemoglobin variants considered for this measure to be SCD cases (Table 1, see Supporting Documents). Evidence of antibiotic prophylaxis is determined through administrative records for pharmacy prescriptions filled (Table 2, see Supporting Documents).

1.H. Numerator Exclusions

- Children who died within 90 days of birth.
- Children who were placed in the neonatal intensive care unit (NICU) within 90 days of birth.

- Children with a diagnosis in the State newborn screening (NBS) program records indicating one of the SCD variants listed in Table 3 (see Supporting Documents).

1.I. Denominator Statement

The eligible population is drawn from all SCD cases reported in State NBS program records.

1.J. Denominator Exclusions

- Children who died within 90 days of birth.
- Children who were placed in the neonatal intensive care unit (NICU) within 90 days of birth.
- Children with a diagnosis in the State newborn screening (NBS) program records indicating one of the SCD variants listed in Table 3 (see Supporting Documents).

1.K. Data Sources

Check all the data sources for which the measure is specified and tested.

State newborn screening data.

If other, please list all other data sources in the field below.

Not applicable.

Section 2: Detailed Measure Specifications

Provide sufficient detail to describe how a measure would be calculated from the recommended data sources, uploading a separate document (+ Upload attachment) or a link to a URL. Examples of detailed measure specifications can be found in the CHIPRA Initial Core Set Technical Specifications Manual 2011 published by the Centers for Medicare & Medicaid Services. Although submission of formal programming code or algorithms that demonstrate how a measure would be calculated from a query of an appropriate electronic data source are not requested at this time, the availability of these resources may be a factor in determining whether a measure can be recommended for use.

Specifications can be found in the Supporting Documents for Sickle Cell Disease Measure 2: Timeliness of Antibiotic Prophylaxis for Children with Sickle Cell Disease.

Section 3. Importance of the Measure

In the following sections, provide brief descriptions of how the measure meets one or more of the following criteria for measure importance (general importance, importance to Medicaid and/or CHIP, complements or enhances an existing measure). Include references related to specific points made in your narrative (not a free-form listing of citations).

3.A. Evidence for General Importance of the Measure

Provide evidence for all applicable aspects of general importance:

- **Addresses a known or suspected quality gap and/or disparity in quality (e.g., addresses a socioeconomic disparity, a racial/ethnic disparity, a disparity for Children with Special Health Care Needs (CSHCN), a disparity for limited English proficient (LEP) populations).**
- **Potential for quality improvement (i.e., there are effective approaches to reducing the quality gap or disparity in quality).**
- **Prevalence of condition among children under age 21 and/or among pregnant women**
- **Severity of condition and burden of condition on children, family, and society (unrelated to cost)**
- **Fiscal burden of measure focus (e.g., clinical condition) on patients, families, public and private payers, or society more generally, currently and over the life span of the child.**
- **Association of measure topic with children's future health – for example, a measure addressing childhood obesity may have implications for the subsequent development of cardiovascular diseases.**
- **The extent to which the measure is applicable to changes across developmental stages (e.g., infancy, early childhood, middle childhood, adolescence, young adulthood).**

Sickle Cell Disease Prevalence and Incidence

SCD is one of the most common genetic disorders in the United States (Kavanagh, Sprinz, Vinci, et al., 2011). The National Heart, Lung and Blood Institute (NHLBI) estimates that 2,000 infants are born with SCD in the United States each year (NHLBI, 2002). SCD affects 70,000-100,000 children and adults in the United States, predominantly those of African and Hispanic descent (Hassell, 2010).

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 SCD (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 (Alvim, Viana, Pires, et al., 2005; Lemanek, Ranalli, Lukens, 2009). Although medical management of SCD has continued to

improve over time, 196 U.S. children died from SCD-related causes between 1999 and 2002 (Yanni, Grosse, Yang, 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 percent of these children required inpatient hospitalization, and 39 percent used emergency care during a year. Of these children, 63 percent averaged one well-child visit per year, and 10 percent had at least one outpatient visit with a specialist (Raphael, Dietrich, Whitmire, 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 (Agency for Healthcare Research and Quality [AHRQ], 2012). Kauf and colleagues estimate the lifetime cost of health care per patient with SCD to be approximately $460,000 (Kauf, Coates, Huazhi, et al., 2009).

Outcomes of Timely and Appropriate Antibiotic Prophylaxis for Children with Sickle Cell Disease

Prompt initiation and consistent use of antibiotics in young children with SCD increases survival rates through the prevention of overwhelming bacterial infections (NHLBI, 2002). Because sickle cells obstruct blood flow to the spleen, splenic function is compromised, leading to susceptibility to bacterial infections (NHLBI, 2002). Meningitis, pneumonia, and sepsis are major causes of death in children with SCD, and pneumococcal sepsis is known to progress from the onset of fever to death in fewer than 12 hours (Gaston, Verter, Woods, et al., 1986). Given that the highest rate of infection occurs in children with SCD under the age of 3 years (Hirst and Owusu-Ofori, 2010), NHLBI guidelines recommend that infants identified through NBS as having SCD should be started on daily prophylactic penicillin as early as possible (NHLBI, 2002). (For children unable to tolerate penicillin, erythromycin may be prescribed.) The Prophylactic Penicillin Study (PROPS), a randomized, double-blind, multicenter trial initiated in 1983 by the NHLBI, demonstrated an 84 percent reduction in the risk of sepsis in children with SCD who took penicillin daily. The trial was ended 8 months early, as 13 of 110 patients in the placebo group developed pneumococcal sepsis compared with 2 of 105 in the treatment arm (Gaston, et al., 1986). Results from PROPS II in 1995 showed no significant increased risk of infection when daily penicillin prophylaxis was ended for children over the age of 5 years (Hirst and Owusu-Ofori, 2010).

This measure indicates timely antibiotic prophylaxis to prevent life threatening infections in children between 3 months and 5 years of age. The measure does not change across developmental stages.

Performance Gap – Prophylactic Antibiotics

In a 10-year retrospective cohort study of 407 infants enrolled in the Tennessee Medicaid program, 60 percent of infants with SCD did not have prophylactic antibiotic prescriptions filled within the recommended period (i.e., the first 12 weeks of life) (Warren, Arbogast, Dudley, et al., 2010). The study was based on pharmacy claims data, and therefore it is unknown whether parents did not fill prescriptions or if providers did not prescribe the recommended prophylactic

antibiotics. Regardless, the majority of infants with SCD in this study did not receive the recommended antibiotics.

3.B. Evidence for Importance of the Measure to Medicaid and/or CHIP

Comment on any specific features of this measure important to Medicaid and/or CHIP that are in addition to the evidence of importance described above, including the following:

- **The extent to which the measure is understood to be sensitive to changes in Medicaid or CHIP (e.g., policy changes, quality improvement strategies).**
- **Relevance to the Early and Periodic Screening, Diagnostic and Treatment benefit in Medicaid (EPSDT).**
- **Any other specific relevance to Medicaid/CHIP (please specify).**

This measure is relevant to Medicaid because the majority of children with SCD are also enrolled in Medicaid. In 2009, 67 percent of children with SCD discharged from the hospital were enrolled in Medicaid, while 25 percent had private insurance (AHRQ, 2012). Furthermore, several studies have pointed to disparities in prophylactic medication use among patients with public versus private insurance. In a study of children with SCD on Medicaid in Washington State and Tennessee, 10.3 percent of patients with public insurance received no antibiotics during a 365-day period, while only 21.5 percent received more than 270 days of medication. Median duration of prescriptions was 10 days (Sox, Cooper, Koepsell, et al., 2003). In a 10-year retrospective cohort study of 407 infants enrolled in the Tennessee Medicaid program, 60 percent of infants with SCD did not have prophylactic antibiotic prescriptions filled within the recommended period (i.e., the first 12 weeks of life) (Warren, et al., 2010). A study assessing compliance with penicillin prophylaxis for SCD found that compliance among patients with public insurance was only 37 percent (mean age was 9 years) (Teach, Lillis, and Grossi, 1998). This measure would encourage timely initiation of antibiotic prophylaxis for all infants with SCD, a step that the literature suggests is of urgent concern to those covered through Medicaid.

3.C. Relationship to Other Measures (if any)

Describe, if known, how this measure complements or improves on an existing measure in this topic area for the child or adult population, or if it is intended to fill a specific gap in an existing measure category or topic. For example, the proposed measure may enhance an existing measure in the initial core set, it may lower the age range for an existing adult-focused measure, or it may fill a gap in measurement (e.g., for asthma care quality, inpatient care measures).

There currently are no quality measures for the diagnosis, assessment, or treatment of pediatric SCD.

References

Agency for Healthcare Research and Quality. Welcome to HCUPnet: Healthcare Cost and Utilization Project (HCUP); 2012. Available at https://www.hcup-us.ahrq.gov/. Accessed February 1, 2017.

Alvim RC, Viana MB, Pires MA, et al. Inefficacy of piracetam in the prevention of painful crises in children and adolescents with sickle cell disease. Acta Haematol 2005; 113(4):228-33.

Gaston MH, Verter JI, Woods G, et al. Prophylaxis with oral penicillin in children with sickle cell anemia. A randomized trial. N Engl J Med 1986; 314(25):1593-9.

Hassell KL. Population estimates of sickle cell disease in the U.S. Am J Prev Med 2010; 38(4 Suppl):S512-21.

Hirst C, Owusu-Ofori S. Prophylactic antibiotics for preventing pneumococcal infection in children with sickle cell disease. Cochrane Database Syst Rev 2010; 1-21.

Kauf TL, Coates TD, Huazhi L, et al. The cost of health care for children and adults with sickle cell disease. Am J Hematol 2009; 84(6):323-7.

Kavanagh PL, Sprinz PG, Vinci SR, et al. Management of children with sickle cell disease: a comprehensive review of the literature. Pediatrics 2011; 128(6):e1552-74.

Lemanek KL, Ranalli M, Lukens C. A randomized controlled trial of massage therapy in children with sickle cell disease. J Pediatr Psychol 2009; 34(10):1091-6.

National Heart Lung and Blood Institute. The Management of Sickle Cell Disease. Bethesda, MD: NHLBI; 2002. Available at https://www.nhlbi.nih.gov/files/docs/guidelines/sc_mngt.pdf. Accessed February 1, 2017.

Raphael JL, Dietrich CL, Whitmire D, et al. Healthcare utilization and expenditures for low income children with sickle cell disease. Pediatr Blood Cancer 2009; 52(2):263-7.

Sox CM, Cooper WO, Koepsell TD, et al. Provision of pneumococcal prophylaxis for publicly insured children with sickle cell disease. JAMA 2003; 290:1075-61.

Steinberg MH. Management of sickle cell disease. N Engl J Med 1999; 340(13):1021-30.

Teach SJ, Lillis KA, Grossi M. Compliance with penicillin prophylaxis in patients with sickle cell disease. Arch Pediatr Adolesc Med 1998; 152:274-8.

Warren MD, Arbogast PG, Dudley JA, et al. Adherence to prophylactic antibiotic guidelines among Medicaid infants with sickle cell disease. Arch Pediatr Adolesc Med 2010; 164(3):298-9.

Yanni E, Grosse SD, Yang Q, et al. Trends in pediatric sickle cell disease-related mortality in the United States, 1983-2002. J Pediatr 2009; 154(4):541-5.

Section 4. Measure Categories

CHIPRA legislation requires that measures in the initial and improved core set, taken together, cover all settings, services, and topics of health care relevant to children. Moreover, the legislation requires the core set to address the needs of children across all ages, including services to promote healthy birth. Regardless of the eventual use of the measure, we are interested in knowing all settings, services, measure topics, and populations that this measure addresses. These categories are not exclusive of one another, so please indicate "Yes" to all that apply.

Does the measure address this category?

a. **Care Setting – ambulatory:** Yes .
b. **Care Setting – inpatient:** No.
c. **Care Setting – other – please specify:** No.
d. **Service – preventive health, including services to promote healthy birth:** Yes.
e. **Service – care for acute conditions:** No.
f. **Service – care for children with special health care needs/chronic conditions:** Yes.
g. **Service – other (please specify):** No.
h. **Measure Topic – duration of enrollment:** No.
i. **Measure Topic – clinical quality:** Yes.
j. **Measure Topic – patient safety:** No.
k. **Measure Topic – family experience with care:** No.
l. **Measure Topic – care in the most integrated setting:** No.
m. **Measure Topic other (please specify):** No.
n. **Population – pregnant women:** No.
o. **Population – neonates (28 days after birth) (specify age range):** Yes; birth to 28 days.
p. **Population – infants (29 days to 1 year) (specify age range):** Yes; ages 29-90 days.
q. **Population – pre-school age children (1 year through 5 years) (specify age range):** No.
r. **Population – school-aged children (6 years through 10 years) (specify age range):** No.
s. **Population – adolescents (11 years through 20 years) (specify age range):** No.
t. **Population – other (specify age range):**
u. **Other category (please specify):** Not applicable.

Section 5. Evidence or Other Justification for the Focus of the Measure

The evidence base for the focus of the measures will be made explicit and transparent as part of the public release of CHIPRA deliberations; thus, it is critical for submitters to specify the scientific evidence or other basis for the focus of the measure in the following sections.

5.A. Research Evidence

Research evidence should include a brief description of the evidence base for valid relationship(s) among the structure, process, and/or outcome of health care that is the focus of the measure. For example, evidence exists for the relationship between immunizing a child or adolescent (process of care) and improved outcomes for the child and the public. If sufficient evidence existed for the use of immunization registries in practice or at the State level and the provision of immunizations to children and adolescents, such evidence would support the focus of a measure on immunization registries (a structural measure).

Describe the nature of the evidence, including study design, and provide relevant citations for statements made. Evidence may include rigorous systematic reviews of research literature and high-quality research studies.

This measure focuses on a clinical process (timely antibiotic prophylaxis) that, if followed, results in a desirable clinical outcome (reduced rate of infection among children less than 3 months of age with SCD). The measure highlights where providers or health systems are falling short in providing this essential element of care.

The body of evidence addresses the effect of appropriate antibiotic prophylaxis in children under 5 years of age with SCD in comparison to standard care. Overall, clinical guidelines and the results of randomized controlled trials indicate that providers should prescribe appropriate antibiotic prophylaxis to children with SCD who are under 5 years of age. Table 4 (see Supporting Documents) summarizes several key sources of evidence for this measure, using the U.S. Preventive Services Task Force (USPSTF) rankings (criteria denoted in table).

5.B. Clinical or Other Rationale Supporting the Focus of the Measure (optional)

Provide documentation of the clinical or other rationale for the focus of this measure, including citations as appropriate and available.

In patients with SCD, the mortality rate is highest in the first 5 years of life, with the greatest period of risk occurring between 6 months and 1 year. Because compromised splenic function in children with SCD permits bacterial infections to become overwhelming, meningitis, pneumonia, and sepsis can escalate quickly into potentially deadly illnesses. Daily antibiotic prophylaxis, initiated as early as possible in infants with SCD and continued daily until the child is 5 years old, reduces the patient's susceptibility to serious infection (Gaston, Verter, Woods, et al., 1986; Hirst, Owusu-Ofori, 2010; National Heart, Lung, and Blood Institute [NHLBI], 2002).

References

Gaston MH, Verter JI, Woods G, et al. Prophylaxis with oral penicillin in children with sickle cell anemia. A randomized trial. *N Engl J Med* 1986; 314(25):1593-1599.

Hirst C, Owusu-Ofori S. Prophylactic antibiotics for preventing pneumococcal infection in children with sickle cell disease. *Cochrane Database of Systematic Reviews.* 2010; 1-21.

National Heart, Lung and Blood Institute. The Management of Sickle Cell Disease. In: National Institutes of Health, ed. Bethesda, MD, 2002.

Section 6. Scientific Soundness of the Measure

Explain the methods used to determine the scientific soundness of the measure itself. Include results of all tests of validity and reliability, including description(s) of the study sample(s) and methods used to arrive at the results. Note how characteristics of other data systems, data sources, or eligible populations may affect reliability and validity.

6.A. Reliability

Reliability of the measure is the extent to which the measure results are reproducible when conditions remain the same. The method for establishing the reliability of a measure will depend on the type of measure, data source, and other factors.

Explain your rationale for selecting the methods you have chosen, show how you used the methods chosen, and provide information on the results (e.g., the Kappa statistic). Provide appropriate citations to justify methods.

Data/Sample

This measure is based on data from State NBS programs, which are active in every State. Q-METRIC tested this measure using NBS results from public health agencies in three States: Illinois, Michigan, and Wisconsin. NBS program data capture the vast majority of births in the United States; in Michigan, 97 percent of all children have NBS performed, while in Illinois the figure is nearly 99 percent; for the remaining births, families have opted out of NBS on religious grounds.

This measure was tested as specified, which requires an assessment among the entire population of a State's birth cohort that had an initial NBS indicating SCD. This measure includes no sampling; consequently, no sampling error is introduced that would necessitate the calculation of the measure reliability.

Results for the measures when tested in Illinois and Michigan are shown in Table 5 (see Supporting Documents).

6.B. Validity

Validity of the measure is the extent to which the measure meaningfully represents the concept being evaluated. The method for establishing the validity of a measure will depend on the type of measure, data source, and other factors.

Explain your rationale for selecting the methods you have chosen, show how you used the methods chosen, and provide information on the results (e.g., R2 for concurrent validity).

The validity of this measure was determined through face validity established by a national panel of experts and advocates for families of children with SCD. Face validity is the degree to which the measure construct characterizes the concept being assessed, which was established by two Q-

METRIC SCD expert panels. The panel established a very high degree of face validity for this measure through a detailed review of concepts and metrics considered to be essential to effective SCD management and treatment. 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 expert panels included 14 experts, providing a comprehensive perspective on SCD management and the measurement of quality metrics for States and health plans. From this group, concepts and draft measures were rated for their relative importance. This measure was among the most highly rated, with the expert panel's average score of 8.6 (out of a maximum of 9). Two rating methods were used to minimize any potential bias due to outlier ratings; this measure received identically high ratings (8.6) using both methods. In addition, the expert panelists noted that this measure not only was important, but could be accessed through State health departments and would likely have a high degree of availability from pharmacy prescription claims records.

Validity testing for this measure comparing data from the Michigan NBS program with claims data pulled from the Community Health Automated Medicaid Processing System (CHAMPS) showed moderate to high levels of agreement for 2010 and 2011, respectively (see Table 6 in the Supporting Documents). Among SCD cases enrolled in Medicaid during the first 90 days of life, there was a 58 percent agreement between the SCD case roster and Michigan claims data in 2010 (36 percent+22 percent). For 2011, the agreement increased substantially to 80 percent (47 percent+33 percent).

Section 7. Identification of Disparities

CHIPRA requires that quality measures be able to identify disparities by race, ethnicity, socioeconomic status, and special health care needs. Thus, we strongly encourage nominators to have tested measures in diverse populations. Such testing provides evidence for assessing measure's performance for disparities identification. In the sections below, describe the results of efforts to demonstrate the capacity of this measure to produce results that can be stratified by the characteristics noted and retain the scientific soundness (reliability and validity) within and across the relevant subgroups.

7.A. Race/Ethnicity

The measure was tested using the entire birth cohort of newborns with a positive initial SCD screen in three States over a 5-year period. This measure includes no sampling and, therefore, is the entire population of children with SCD. Demographic information describing these populations of children with SCD is presented below.

The measure was tested in three States among the entire population of children with SCD, which is largely concentrated among African American infants. Table 7 (see Supporting Documents) summarizes the distribution across race and ethnicity groups for each State. Note the degree to which information on the race and ethnicity of infants with SCD varies among States.

7.B. Special Health Care Needs

In those States in which Q-METRIC conducted testing, NBS data for infants did not include indicators of special care needs.

7.C. Socioeconomic Status

Although State NBS data for infants do not directly capture information on socioeconomic status, Medicaid eligibility, shown in Table 8 (see Supporting Documents), provides one proxy indicator.

7.D. Rurality/Urbanicity

Table 9 (see Supporting Documents) summarizes the urban/rural distribution for the three testing States and shows that the great majority of newborns with a positive screen for SCD reside in urban settings. This information was not available from Wisconsin's NBS data.

7.E. Limited English Proficiency (LEP) Populations

This information is not available from NBS data in the three States in which this Q-METRIC measure was tested.

Section 8. Feasibility

Feasibility is the extent to which the data required for the measure are readily available, retrievable without undue burden, and can be implemented for performance measurement. Using the following sections, explain the methods used to determine the feasibility of implementing the measure.

8.A. Data Availability

1. What is the availability of data in existing data systems? How readily are the data available?

This measure is implemented using data maintained by State universal NBS programs. This testing is mandatory in all 50 States, as well as in the District of Columbia, Puerto Rico, the U.S. Virgin Islands, and Guam.

Table 10 (see Supporting Documents) outlines the SCD NBS process used by the three States in which this measure was tested by Q-METRIC (Illinois, Michigan, and Wisconsin). Note that the data required for this measure are tracked by NBS programs for follow up of SCD cases and vary by State. In Illinois, antibiotic administration to newborns with SCD is obtained from the definitive diagnosis form that is completed by the physicians; these data are subsequently collected on annual follow-up forms. In Michigan, follow up is conducted by the Michigan Department of Community Health (MDCH) with the physician or parent/guardian of each case with an initial NBS result indicating SCD. Note that in Wisconsin, these data are not currently available from the State NBS program; at present, the initial date of a physician office visit is recorded but not whether an antibiotic was prescribed.

All States conduct NBS for SCD among all births; the birth cohorts for the three States in which this measure was tested by Q-METRIC are summarized in Table 11 (see Supporting Documents).

2. If data are not available in existing data systems or would be better collected from future data systems, what is the potential for modifying current data systems or creating new data systems to enhance the feasibility of the measure and facilitate implementation?

The proposed measure was determined to be feasible by Q-METRIC in two States; however, it was found to be not feasible in one State (Wisconsin) using existing NBS data systems. Importantly, significant initiatives are currently underway nationally that will greatly increase the use of electronic health record (EHR) systems by primary care providers and specialists who treat children with SCD. As a result, the availability of information regarding the prescription of antibiotics to infants with SCD will likely improve substantially in the next few years. For example, e-prescribing systems using a controlled terminology for generic and brand medications will allow easy access to medication prescription orders and fill status from pharmacies. Thus, prescriptions for antibiotics to infants with an initial SCD screening result will be available electronically in real-time. These results can be reported to NBS programs through health information exchange (HIE) technologies that are rapidly becoming operational throughout the United States. HIEs will enable the reporting of e-prescription information for antibiotics among newborns with SCD to NBS programs. In States where this reporting already exists through other methods, the HIE reporting will enable improvements to the timeliness and completeness of these events being reported from physician practices.

8.B. Lessons from Use of the Measure

1. Describe the extent to which the measure has been used or is in use, including the types of settings in which it has been used, and purposes for which it has been used.

This measure is not currently in use in the three States in which Q-METRIC testing was conducted; further, we do not believe it is in use in any State .

2. If the measure has been used or is in use, what methods, if any, have already been used to collect data for this measure?

Not applicable.

3. What lessons are available from the current or prior use of the measure?

Not applicable.

Section 9. Levels of Aggregation

CHIPRA states that data used in quality measures must be collected and reported in a standard format that permits comparison (at minimum) at State, health plan, and provider levels. Use the following table to provide information about this measure's use for reporting at the levels of aggregation in the table.

For the purpose of this section, please refer to the definitions for provider, practice site, medical group, and network in the Glossary of Terms.

If there is no information about whether the measure could be meaningfully reported at a specific level of aggregation, please write "Not available" in the text field before progressing to the next section.

Level of aggregation (Unit) for reporting on the quality of care for children covered by Medicaid/ CHIP†:

State level* Can compare States

Intended use: **Is measure intended to support meaningful comparisons at this level? (Yes/No)**
Yes.

Data Sources: **Are data sources available to support reporting at this level?**
Yes.

Sample Size: **What is the typical sample size available for each unit at this level? What proportion of units at this level of aggregation can achieve an acceptable minimum sample size?**
All sickle cell cases reported in a respective State.

In Use: **Have measure results been reported at this level previously?**
No.

Reliability & Validity: **Is there published evidence about the reliability and validity of the measure when reported at this level of aggregation?**
No.

Unintended consequences: **What are the potential unintended consequences of reporting at this level of aggregation?**
None identified; this is the level at which results from newborn screening data for sickle cell disease are collected and maintained in the United States.

Other geographic level: Can compare other geographic regions (e.g., MSA, HRR)

Intended use: **Is measure intended to support meaningful comparisons at this level? (Yes/No)**
No.

Data Sources: **Are data sources available to support reporting at this level?**
No.

Sample Size: **What is the typical sample size available for each unit at this level? What proportion of units at this level of aggregation can achieve an acceptable minimum sample size?**

Not available.

In Use: **Have measure results been reported at this level previously?**
No.

Reliability & Validity: **Is there published evidence about the reliability and validity of the measure when reported at this level of aggregation?**
No.

Unintended consequences: **What are the potential unintended consequences of reporting at this level of aggregation?**
Not available.

Medicaid or CHIP Payment model: Can compare payment models (e.g., managed care, primary care case management, FFS, and other models)

Intended use: **Is measure intended to support meaningful comparisons at this level? (Yes/No)**
No.

Data Sources: **Are data sources available to support reporting at this level?**
No.

Sample Size: **What is the typical sample size available for each unit at this level? What proportion of units at this level of aggregation can achieve an acceptable minimum sample size?**
Not available.

In Use: **Have measure results been reported at this level previously?**
No.

Reliability & Validity: **Is there published evidence about the reliability and validity of the measure when reported at this level of aggregation?**
No.

Unintended consequences: **What are the potential unintended consequences of reporting at this level of aggregation?**
Not available.

Health plan*: Can compare quality of care among health plans.

Intended use: **Is measure intended to support meaningful comparisons at this level? (Yes/No)**
No.

Data Sources: **Are data sources available to support reporting at this level?**
No.

Sample Size: **What is the typical sample size available for each unit at this level? What proportion of units at this level of aggregation can achieve an acceptable minimum sample size?**
Not available.

In Use: **Have measure results been reported at this level previously?**
No.

Reliability & Validity: **Is there published evidence about the reliability and validity of the measure when reported at this level of aggregation?**
No.

Unintended consequences: **What are the potential unintended consequences of reporting at this level of aggregation?**
Not available.

Provider Level
Individual practitioner: Can compare individual health care professionals

Intended use: **Is measure intended to support meaningful comparisons at this level? (Yes/No)**
No.

Data Sources: **Are data sources available to support reporting at this level?**
No.

Sample Size: **What is the typical sample size available for each unit at this level? What proportion of units at this level of aggregation can achieve an acceptable minimum sample size?**
Not available.

In Use: **Have measure results been reported at this level previously?**
No.

Reliability & Validity: **Is there published evidence about the reliability and validity of the measure when reported at this level of aggregation?**
No.

Unintended consequences: **What are the potential unintended consequences of reporting at this level of aggregation?**
Not available.

Provider Level
Hospital: Can compare hospitals

Intended use: **Is measure intended to support meaningful comparisons at this level? (Yes/No)**
No.

Data Sources: **Are data sources available to support reporting at this level?**
No.

Sample Size: **What is the typical sample size available for each unit at this level? What proportion of units at this level of aggregation can achieve an acceptable minimum sample size?**
Not available.

In Use: **Have measure results been reported at this level previously?**
No.

Reliability & Validity: **Is there published evidence about the reliability and validity of the measure when reported at this level of aggregation?**
No.

Unintended consequences: **What are the potential unintended consequences of reporting at this level of aggregation?**
Not available.

Provider Level
Practice, group, or facility:** Can compare: (i) practice sites; (ii) medical or other professional groups; or (iii) integrated or other delivery networks

Intended use: **Is measure intended to support meaningful comparisons at this level? (Yes/No)**
No.

Data Sources: **Are data sources available to support reporting at this level?**
No.

Sample Size: **What is the typical sample size available for each unit at this level? What proportion of units at this level of aggregation can achieve an acceptable minimum sample size?**
Not available.

In Use: **Have measure results been reported at this level previously?**
No.

Reliability & Validity: **Is there published evidence about the reliability and validity of the measure when reported at this level of aggregation?**
No.

Unintended consequences: **What are the potential unintended consequences of reporting at this level of aggregation?**
Not available.

Section 10. Understandability

CHIPRA states that the core set should allow purchasers, families, and health care providers to understand the quality of care for children. Please describe the usefulness of this measure toward achieving this goal. Describe efforts to assess the understandability of this measure (e.g., focus group testing with stakeholders).

This measure provides parents with an intuitive measure of antibiotic protection among children with SCD. Low rates are easily understood to be satisfactory. Likewise, the simplicity of the measure makes it a straightforward guide for providers and plans to assess comprehensive protection for the infant SCD population.

This measure has not been assessed for comprehension. Because the measure uses State NBS program data, State employees responsible for collecting information for this measure—the number of eligible children who receive antibiotic prophylaxis within 90 days of age—are familiar with the concepts of this process. In the three States where Q-METRIC tested the measure, employee comprehension was excellent.

Section 11. Health Information Technology

Please respond to the following questions in terms of any health information technology (health IT) that has been or could be incorporated into the measure calculation.

11.A. Health IT Enhancement

Please describe how health IT may enhance the use of this measure.

It is anticipated that major enhancements will come through the use of home-based medication management tools. There are numerous mobile device apps now available that allow recording the administration of doses using a controlled terminology for generic and brand medications. As a consequence, these tools will enable a diary for self-reporting the administration of doses and would likely be the most accurate data to use for this measure. Less accurate, though perhaps more feasible today, is the use of prescription fill data from Surescripts or similar prescription relay messaging services (Grossman, Cross, Boukus, et al., 2012; Joseph, Sow, Furukawa, et al., 2013). This system will provide an actual prescribed medication, date, and prescribers' signature, which will be an accurate marker for the timeliness of prescribed medications. However, these data will not furnish information regarding whether the child ever received the medication as prescribed; a proxy for this may be obtained through multiple successive fills, which may be an indicator of medications being used.

11.B. Health IT Testing

Has the measure been tested as part of an electronic health record (EHR) or other health IT system?

Yes.

If so, in what health IT system was it tested and what were the results of testing?

This measure was tested using data maintained by State NBS programs.

11.C. Health IT Workflow

Please describe how the information needed to calculate the measure may be captured as part of routine clinical or administrative workflow.

The information for this measure will be captured by existing EHR systems. NBS results will be obtained from faxed or electronically submitted reports and documented in the EHR. Therefore, this positive result will be either a discrete element in a report or a component of provider documentation that must be extracted using computational techniques. From this group of positives, the EHR will have data from documented prescriptions that will identify the percentage of patients who were prescribed prophylactic antibiotics. This number will be refined by using fill data to identify the percentage who picked up a prescription for these antibiotics and by home administration data (the most difficult part of this measure) to identify patients who received it. The stop date (confirmatory testing negative) will be obtained from the results reporting system or from computational techniques applied to provider documentation.

11.D. Health IT Standards

Are the data elements in this measure supported explicitly by the Office of the National Coordinator for Health IT Standards and Certification criteria (see healthit.hhs.gov/portal/server.pt/community/healthit_hhs_gov__standards_ifr/1195)?

Yes.

If yes, please describe.

The ONC's Health IT Standards explicitly address the receipt of electronic prescribing into EHRs, which is directly related to the measurement of the timeliness and completeness of antibiotic prescriptions for children with SCD. The ONC standards include the following specific requirements in the Certification criteria (Federal Register, 2010):

b. Electronic Prescribing Standards
The Medicare Prescription Drug, Improvement and Modernization Act of 2003 (MMA) provided for, among other things, the Voluntary Prescription Drug Benefit Program. Under that program, electronically transmitted prescriptions and certain other information for covered Part D drugs prescribed for Part D eligible individuals must be sent in a manner that complies with applicable standards that are adopted by the Secretary. The Secretary proposed the first of these standards in a February 2005 rulemaking (70 FR 6256). Subsequently, on June 23, 2006 (71 FR 36020), HHS published an interim final rule that maintained the National Council for Prescription Drug Programs (NCPDP) SCRIPT 5.0 as the adopted standard, but allowed for the voluntary use of a subsequent backward compatible version of the standard, NCPDP SCRIPT 8.1.

As a result of pilot testing of six "initial standards" that had been identified in 2005, the Secretary issued a notice of proposed rulemaking on November 16, 2007 (72 FR 64900) which proposed adoption of certain standards. The Secretary also used this proposed rule to solicit comments regarding the impact of adopting NCPDP SCRIPT 8.1 and retiring NCPDP SCRIPT 5.0. Based on the comments that were received, the Secretary issued a final rule (73 FR 18918)

on April 7, 2008 that adopted NCPDP SCRIPT Version 8.1 and retired NCPDP SCRIPT Version 5.0. In adopting an initial set of standards to meet the requirement specified at section 3004(b)(1) of the PHSA, we have taken into account these electronic prescribing standards and ensured that our standards are consistent with them.

11.E. Health IT Calculation

Please assess the likelihood that missing or ambiguous information will lead to calculation errors.

Missing or ambiguous information in the following areas will lead to missing cases or calculation errors:

1. Child's date of birth.
2. NBS results for hemoglobin screening (initial and confirmatory), including the specific name of the hemoglobin condition.
3. Date of prescription.
4. Type of medication (antibiotic).

11.F. Health IT Other Functions

If the measure is implemented in an EHR or other health IT system, how might implementation of other health IT functions (e.g., computerized decision support systems in an EHR) enhance performance characteristics on the measure?

Implementation of an order entry system will allow easy access to date and type of antibiotic prescription. Orders will facilitate knowing the medication and dosing regimen prescribed for prophylactic antibiotics; additional technologies, such as receiving RxFill data and patient-reported medication adherence data using new technologies like personal health records would greatly enhance measure accuracy.

References

Grossman JM, Cross DA, Boukus ER, et al. Transmitting and processing electronic prescriptions: experiences of physician practices and pharmacies. J Am Med Inform Assoc 2012; 19(3):353-9.

Health information technology: Initial set of standards, implementation specifications, and certification criteria for electronic health record technology. Fed Reg 75(8):2013-47.

Joseph SB, Sow MJ, Furukawa MF, et al. E-prescribing adoption and use increased substantially following the start of a federal incentive program. Health Aff 2013; 32(7):1221-7.

Section 12. Limitations of the Measure

Describe any limitations of the measure related to the attributes included in this CPCF (i.e., availability of measure specifications, importance of the measure, evidence for the focus of

the measure, scientific soundness of the measure, identification of disparities, feasibility, levels of aggregation, understandability, health information technology).

This measure assesses the percentage of children with a newborn screen positive for SCD who receive appropriate preventive antibiotics by 3 months of age. Preventive antibiotics reduce the risk of life-threatening infections for children with SCD in this age group.

Q-METRIC testing determined that this measure can be addressed using existing data systems. However, some States may not currently track antibiotic prescriptions in conjunction with NBS. In those jurisdictions this measure will rely more upon advances in health IT that will enable wider availability of electronic prescription results through increased use of EHRs and information sharing through HIE technologies.

It should be noted that at present, some children with SCD may receive antibiotics at no cost. It is unclear whether the dispensing pharmacies will track those events using electronic prescribing mechanisms.

Section 13. Summary Statement

Provide a summary rationale for why the measure should be selected for use, taking into account a balance among desirable attributes and limitations of the measure. Highlight specific advantages that this measure has over alternative measures on the same topic that were considered by the measure developer or specific advantages that this measure has over existing measures. If there is any information about this measure that is important for the review process but has not been addressed above, include it here.

This measure, Timeliness of Antibiotic Prophylaxis for Children with Sickle Cell Disease, assesses the percentage of children with a newborn screen positive for SCD who receive appropriate preventive antibiotics by 3 months of age. Preventive antibiotics reduce the risk of life-threatening infections for children with SCD in this age group. This measure provides an intuitive measure of antibiotic protection and encourages timely prescribing for all infants with SCD, a step that the literature suggests is of urgent concern for those covered through Medicaid.

Currently, there are no quality measures for the diagnosis, assessment, or treatment of pediatric SCD. Yet SCD is one of the most common genetic disorders in the United States, and its impact among children is great. In patients with SCD, the mortality rate is highest in early childhood, and infancy is a period of special risk. Young children with SCD experience infections, stroke, pain episodes, and hospitalizations; these events restrict sleep and daily activities, affecting a child's ability to engage academically and socially.

In particular, spleen damage can occur early in infants diagnosed with SCD, affecting their ability to resist infections and leaving them at risk for sudden debilitating illnesses and even death. However, prompt initiation of antibiotic prophylaxis in infants and consistent use of antibiotics in young children dramatically increase survival rates through the prevention of overwhelming bacterial infections. By reducing the occurrence of serious infection among children with SCD, timely and consistent use of antibiotics also enables families and providers to

address other medical challenges related to SCD, thus helping lower the overall burden of illness. This, in turn, may help reduce long-term costs for purchasers.

The Q-METRIC SCD expert panel established a high degree of face validity for this measure through a detailed review of concepts and metrics essential for effective SCD management. Reliability for the measure is supported by the comprehensive nature of NBS data, which by law, is collected in every State and reflects the vast majority of births. (In Michigan, 97 percent of all children have NBS performed; in Illinois, that figure approaches 99 percent.) Validation testing in Michigan for the measure showed agreement levels of 58 percent and 80 percent for 2010 and 2011, respectively, between SCD roster data and Medicaid claims data for antibiotic prescriptions within the first 90 days of life.

Testing results for the measure itself ranged from 61 percent in Illinois in 2009 to 31 percent in 2012. In Michigan, measure rates ranged from 36 percent in 2008 to 50 percent in 2011, underscoring the need to assess the level of prompt prescription of antibiotics for infants following a positive newborn screen for SCD. The measure was not feasible in Wisconsin using existing NBS data systems, where the initial date of a physician office visit is recorded but not whether an antibiotic was prescribed. However, significant initiatives are underway nationally, such as implementation of e-prescribing programs, that will greatly increase the use of EHR systems by primary care providers and specialists who treat children with SCD. As a result, the availability of information regarding the prescription of antibiotics to infants with SCD will likely improve substantially in the next few years.

This measure is relevant to Medicaid, as the majority of children with SCD are also enrolled in Medicaid. Several studies have pointed to disparities in prophylactic medication use for SCD among young patients with public versus private insurance. Frequently, children with SCD who are enrolled in Medicaid are not dispensed medication soon enough or in sufficient quantities to cover ongoing daily use of an antibiotic; sometimes, they receive no antibiotics at all.

Section 14: Identifying Information for the Measure Submitter

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The CHIPRA Pediatric Quality Measures Program (PQMP) Candidate Measure Submission Form (CPCF) was approved by the Office of Management and Budget (OMB) in accordance with the Paperwork Reduction Act.

The OMB Control Number is 0935-0205 and the Expiration Date is December 31, 2015.

Public Disclosure Requirements

Each submission must include a written statement agreeing that, should U.S. Department of Health and Human Services accept the measure for the 2014 and/or 2015 Improved Core Measure Sets, full measure specifications for the accepted measure will be subject to public disclosure (e.g., on the Agency for Healthcare Research and Quality [AHRQ] and/or Centers for Medicare & Medicaid Services [CMS] websites), except that potential measure users will not be permitted to use the measure for commercial use. In addition, AHRQ expects that measures and full measure specifications will be made reasonably available to all interested parties. "Full measure specifications" is defined as all information that any potential measure implementer will need to use and analyze the measure, including use and analysis within an electronic health record or other health information technology. As used herein, "commercial use" refers to any sale, license or distribution of a measure for commercial gain, or incorporation of a measure into any product or service that is sold, licensed or distributed for commercial gain, even if there is no actual charge for inclusion of the measure. This statement must be signed by an individual authorized to act for any holder of copyright on each submitted measure or instrument. The authority of the signatory to provide such authorization should be described in the letter.

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