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Practice Guidelines and Standards of Care for Children with Special Health Care Needs:

Report and Compendium

Prepared for:

Division of Maternal and Child Health
North Carolina Department of Environment,
Health, and Natural Resources

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Table of Contents

I. Overview of Practice Guidelines

Today, as a growing proportion of health care is delivered in managed care settings and there is an increased focus on medical cost containment, standards are needed to promote and assure quality of care for patients. Quality assurance is a particularly important issue for children with special health care needs (CSHCN). These children often have uncommon and complex conditions that may require costly, long-term early interventions and treatments. Meeting the needs of CSHCN presents a particular challenge in a managed care delivery system, where cost containment is a high priority and most health professionals do not have experience treating these uncommon pediatric conditions.

To address the concern for promoting quality of care for this population, there is a growing interest among practitioners, patients, public health officials, and managed care plans in the development and use of practice guidelines. Practice guidelines, as defined in a 1990 report by the Institute of Medicine, are "systematically developed statements to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances." (Institute of Medicine 1990) These guidelines vary in the way they are developed. For example, some rely solely on the scientific evidence indicating effective treatments or protocols. Most involve both evidence-based criteria and "best practice" consensus about clinical practice among health providers and researchers. Different organizations refer to these guidelines as "clinical practice guidelines," "practice parameters," or "practice standards."

Such procedure-specific or disease-specific guidelines are valuable tools for health care providers in the treatment and referral processes. While practice guidelines were originally designed to assist provider and patient decision making, they are also useful as organization-wide standards for managed care plans—with the goal of promoting and evaluating quality of care among participating providers.

II. Technical Assistance Request

In January 1996, officials from the Children and Youth Section (CYS) of the Division of Maternal and Child Health in the North Carolina Department of Environment, Health and Natural Resources requested technical assistance (TA) from Health Systems Research, Inc. (HSR) in the development of tools to assure health care quality and monitor outcomes for CSHCN. North Carolina is one of twenty-eight States currently being served under a three-year contract awarded to HSR by the Maternal and Child Health Bureau in July 1994 ("Technical Assistance to States Developing Comprehensive Systems of Care for Children and Their Families"). This contract expands the scope of an existing contract between HSR and the MCHB begun in October 1992. Combined, both contracts support individually-tailored technical assistance projects in all 50 States, the District of Columbia, the Virgin Islands and Puerto Rico.

North Carolina's TA request reflected an interest among the State's MCH officials in preparing for the not-so-distant future when they anticipate the entire State Medicaid population will be served in managed care settings. The TA request occured at a time when North Carolina has implemented mandatory managed care in only one area of the State (Mecklenburg County) and the Supplemental Security Income (SSI) Medicaid population in that area, which comprises the majority of CSHCN, has been exempted from the mandate for one year. However, the State's CYS hopes to use the information gained from this TA effort to design a set of standards and quality measurements for CSHCN that can be applied first in Mecklenburg County and later across the State as Medicaid managed care is more broadly implemented.

To begin addressing North Carolina's TA request, Ian Hill, HSR Project Director; Vivian Gabor, HSR Senior Associate; and project consultant Jim Perrin, M.D. met in May 1996 with

Tom Vitaglione, Chief of CYS, members of Mr. Vitaglione's staff, and members of the CYS Medical Advisory Committee.[1]

During the meeting with the CYS Medical Advisory Committee, participants agreed that a wide range of medical protocols exist today for CSHCN and that more information was needed on the effectiveness of many technologies and therapies in use. Committee members also noted that, without evidence of the effectiveness of care for specific conditions, health care plans sometimes question whether a treatment or therapy is medically necessary, particularly for a child with a chronic illness or disability who requires expensive and/or long-term treatments. They emphasized the need for more information on clinical practice guidelines for this population, as a means of shaping North Carolina's State policies and standards for serving CSHCN under managed care.

At the conclusion of these meetings, North Carolina officials requested that HSR conduct research on available clinical guidelines for CSHCN and produce a compendium of existing guidelines for the ten most common and costly conditions that affect CSHCN in North Carolina. These ten conditions were identified as:

- Asthma;
- Attention-deficit hyperactivity disorder;
- Cerebral palsy;
- Congenital deafness;
- Cystic fibrosis;
- Diabetes;
- Sickle cell disease;
- Spina bifida;

[1] For more details on this meeting, refer to HSR report: *Technical Assistance to States Developing Comprehensive Systems of Care for Children and Their Families: North Carolina—Initial Site Visit Report*.

- Epilepsy; and
- Mild mental retardation.

To collect the guidelines for this compendium, HSR contacted nearly 100 Federal agencies, research centers, foundations, health professional associations, voluntary health agencies, and patient advocacy groups throughout the country. The availability of practice guidelines and standards of care varied according to the condition in question. For many, standards were in development or were only recently developed.

As a result of this effort, HSR was able to obtain one or more practice guidelines for eight of the ten conditions identified by North Carolina officials. For two of the conditions, no practice guidelines or standards of care for the treatment of children were available. Specifically, for mild mental retardation, the general consensus of those contacted was that none have as yet been published and none are in development. For the second, epilepsy, the Epilepsy Foundation of America reported that it is currently in the early stages of developing guidelines for the treatment of this condition but gave no indication as to when these guidelines would be published. Available from the National Institutes of Health Consensus Program Information Center is a publication on surgery for epilepsy. This document is not, however, included in this compendium as it does not relate specifically to treating children with epilepsy.

III. Summary of Guidelines for CSHCN

The remainder of this overview presents a review of the practice guidelines and/or standards of care identified for eight chronic conditions of children. For each guideline, the process by which it was developed is described. When more than one guideline is included for a specific condition, a brief description of the differences among them is provided. In addition, information on those guidelines that are under development, but not yet available, is presented. Finally, a description of the organizations contacted to obtain the guidelines is included. Copies of all guidelines and standards collected through this effort can be found in Tabs one through eight in Appendix A.

A. Asthma

The search identified several practice parameters for treating persons with asthma. Two guidelines are included in the compendium and are described below. A third, titled *Practice Parameters for the Diagnosis and Treatment of Asthma,* is not included in this compendium as it does not relate specifically to the treatment of children. This publication can, however, be obtained from the American Academy of Allergy and Immunology in Milwaukee, Wisconsin.

In 1994, the Provisional Committee on Quality Improvement of the American Academy of Pediatrics (AAP) wrote *Practice Parameter: The Office Management of Acute Exacerbations of Asthma in Children* and its companion document *Technical Report—Practice Parameter: The Treatment of Acute Exacerbations of Asthma in Children.* This practice parameter, which was reviewed and approved by the American Medical Association, is a short, technical booklet that includes a fold-out assessment and treatment algorithm. Its focus is the treatment of acute exacerbations of asthma in the office setting as opposed to the emergency room or hospital. The guideline is intended to provide an analytical framework for evaluation and treatment but is not a protocol for all patients.

This parameter is based on a report by the National Asthma Education Project of the National Heart, Lung, and Blood Institute (NHLBI) and was derived from expert consensus and a review of the relevant literature. Experts from the Sections on Allergy and Pulmonology of the AAP, general pediatricians, and research methodologists contributed to its development.

Also included in this compendium is the executive summary of the NHLBI report mentioned above. Although this report, titled *Executive Summary: Guidelines for the Diagnosis and Management of Asthma* (1991), does not focus primarily on pediatric care, it does include protocols for the management of asthma in children. This executive summary highlights the major recommendations of a report by an expert panel that was convened by the Coordinating Committee of the National Asthma Education Program. The panel's recommendations are consensus-based and are designed for use by primary care physicians, nurses, asthma specialists

as well as others involved in asthma care such as respiratory care therapists, health educators, social workers, psychologists, and the asthma patient.

B. Attention-Deficit Hyperactivity Disorder

Several organizations focusing on attention-deficit hyperactivity disorder (ADHD), such as Children and Adults with ADHD and the National Attention-Deficit Disorder Association, have or are developing standards of care written for parents and families of children with ADHD. The American Academy of Pediatrics (AAP) also is currently developing evidence-based practice guidelines for pediatricians who care for children with ADHD. The AAP guidelines are expected to be published in 1998.

Included in this compendium is one set of published clinical guidelines for providers who care for children with ADHD. These guidelines, published in a report by the American Academy of Child and Adolescent Psychiatry (AACAP), are titled *Practice Parameters for the Assessment and Treatment of Attention-Deficit Hyperactivity Disorder.* These guidelines delineate the generally accepted level of practice for the assessment and treatment of ADHD in school-aged children, preschool children, and adolescents. They were created over a one-year period by the Work Group on Quality Issues of the AACAP and are presented in a two-page outline format. The parameters are consensus-based, reviewed at least every two years, and revised if advances are made regarding assessment and treatment of ADHD.

C. Cerebral Palsy

Several national and local organizations were contacted for this project, including the United Cerebral Palsy Associations, St. Louis Children's Neurology Service, the American Association of University Affiliated Programs, The ARC, and the Rose Kennedy Center at the Albert Einstein School of Medicine. Representatives of many of these organizations explained that because cerebral palsy (CP) is a complex condition, it is difficult to develop practice guidelines for treating children with the disease. For this reason, some of these organizations are working on developing guidelines for treating one aspect of the condition such as a spastic ankle.

Despite the overwhelming sense that developing guidelines for CP is extremely difficult, HSR identified three guidelines that are now in development and one that has been published. First, the American Academy for Cerebral Palsy and Developmental Medicine is in the preliminary stages of discussing the creation of standards. Second, the JFK Center for Developmental Disabilities at the University of Colorado has written and is about to publish a set of practice guidelines. Finally, the American Occupational Therapy Association is developing standards of care for CP that are anticipated to be published by the Association in 1997.

Standards of Care & Outcome Measures for Children with Cerebral Palsy is the only guideline for the treatment of children with CP that was obtained for this compendium. This publication was written by the Committee on Children with Disabilities of the Bureau for Children with Medical Handicaps, Ohio Department of Health (ODH) in collaboration with the Ohio Chapter of the American Academy of Pediatrics. This six-page document discusses the care of children and young adults from birth to 21 years. These standards reflect a biopsychosocial, function-based model and include outcome and process measures of the quality of care provided to children with CP. Developed in 1989 and revised in 1995, these standards were agreed upon by a standing committee of expert physicians and health professionals in the field of neurology, pediatrics, physical therapy, and research methodology from across the State. The Bureau distributes these standards to its network of providers and other agencies throughout Ohio.

D. Congenital Deafness

While several guidelines for working with deaf patients exist, one developed by the American Speech-Language-Hearing Association (ASHA), titled *Preferred Practice Patterns for the Professions of Speech-Language Pathology and Audiology,*was selected for this compendium. This guideline is not considered the official standard of ASHA, but rather "reflects the normally anticipated professional response to a particular set of circumstances." It provides a comprehensive set of practice standards for screening, assessment, and treatment and includes pediatric care but is not specific to children. Developed over a three-year period, it is the result of a consensus-based process.

E. Cystic Fibrosis

A clinical guideline for the treatment of children with cystic fibrosis in children, titled *Standards of Care and Outcome Measures for Children with Cystic Fibrosis,* was recommended to HSR by the Pediatric Pulmonary Division, Rainbow Babies and Children's Hospital of the University Hospitals of Cleveland. These standards were developed in the 1980s by the Pulmonary Standards Committee of the Ohio Bureau for Children with Medical Handicaps, Ohio Department of Health (ODH). Revised in 1994, this four-page set of standards offers a biopsychosocial, consensus-based approach to treating children with cystic fibrosis and was agreed upon by a committee of pediatricians and pulmonologists. Similar to the standards of care for children with cerebral palsy developed by ODH, these standards are distributed to a network of providers and agencies throughout the State that treat children with cystic fibrosis.

F. Diabetes

Two practice guidelines for the treatment of individuals with diabetes were identified for this report. The first, recommended to HSR by several of organizations including the Juvenile Diabetes Foundation, is titled *Clinical Practice Recommendations 1995* and is the official standards of the American Diabetes Association. Due to the length of the document and the fact that this guideline does not relate specifically to the treatment of children with diabetes, only the table of contents is included in this compendium.

The second guideline, titled *Caring for Children with Diabetes: A Professional's Manual,* is published by the Joslin Diabetes Center in Boston, Massachusetts. This manual is user-friendly and is intended for use by health care professionals treating young children with Type I Diabetes. The guidelines were written in 1996 and are based on clinical experience. Reprint permission could not, however, be obtained for this publication; therefore only the table of contents is included in this compendium.

G. Sickle Cell Disease

Three federally-funded publications were recommended to HSR by the Center for Sickle Cell Disease and the Sickle Cell Disease Association of America. These guidelines differ in their focus for treatment and target audience. The first, *Sickle Cell Disease: Screening, Diagnosis, Management, and Counseling in Newborns and Infants* (and the accompanying *Quick Reference Guide for Clinicians*), pertains specifically to the care of newborns and infants and is geared toward clinicians. Developed in 1993 by the Agency for Health Care Policy and Research (AHCPR) of the Public Health Service, U.S. Department of Health and Human Services, this guideline provides a detailed analysis and discussion of available research, critical evaluation of the assumptions and knowledge in the field, health care decisions, and references. It makes specific recommendations in several areas, including populations to be screened, laboratory methods and responsibilities, medical follow-up for infants with sickle cell disease, and education and decision making counseling.

This guideline for the treatment of sickle cell disease in children is one of several clinical guidelines developed by AHCPR and is meant to serve as a model for the development of other guidelines. AHCPR employed a rigorous process to develop this guideline, including convening an independent panel of internists, pediatricians, nurses, a family physician, a social worker, a person with sickle cell disease, and the parent of a child with sickle cell disease. The panel used a science-based methodology as well as expert clinical judgment to develop specific statements on patient assessment and management. It conducted extensive literature searches and used critical reviews and syntheses to evaluate empirical evidence and significant outcomes. The panel also undertook peer and field reviews to evaluate the validity, reliability, and utility of the guideline in clinical practice.

The second recommended guideline for sickle cell disease, developed by the National Heart, Lung, and Blood Institute (NHLBI) and titled *Management and Therapy of Sickle Cell Disease*, includes health maintenance approaches for patients of all ages and is intended for use by lay and professional communities. It is not included in this compendium as it does not relate specifically to the treatment of children with sickle cell disease.

As opposed to the AHCPR guideline, which is solely based on results of controlled clinical trials, this guideline represents a collective summary of experiences with therapeutic regimens. It is intended to serve as a guide for the health care worker involved in the management of patients with sickle cell disease. A review panel composed of pediatricians, nurses, hematologists, and internists edited the guideline, which was first written in 1990, to reflect advances in the field and modifications in practices and to describe the published findings of clinical research. These include findings from the Preoperative Transfusion Study, the Prophylactic Penicillin Trial II, the Multicenter Hydroxyurea, and epidemiological data derived from the Cooperative Study of Sickle Cell Disease.

The third guideline identified for this report was funded in part through a grant from the Maternal and Child Health Bureau of the U.S. Department of Health and Human Services. *Problem Oriented Management of Sickle Syndromes,* by James R. Eckman, M.D. of Emory University and Allan F. Platt Jr., PA-C of the Georgia Sickle Cell Center (GSCC), is based on the clinical experience at GSCC. This guideline presents recommendations for the treatment of children with sickle cell disease but is also not included in this compendium due to the length of the document. Copies can be obtained from the Maternal and Child Health Clearinghouse in Vienna, Virginia.

H. Spina Bifida

Two guidelines for spina bifida were located by HSR. The first was published in 1995 and provides recommendations for treating individuals of all ages suffering from spina bifida. Written by the Spina Bifida Association of America (SBAA), *Guidelines for Spina Bifida Health Care Services Throughout Life* are the first national spina bifida guidelines available. They are intended as a baseline for clinical practice and a foundation upon which to build clinical standards of care. The guidelines are user-friendly and meant for use by providers of care as well as those with spina bifida and their families.

Developed over a three-year period, these guidelines are based on the knowledge and experience of experts in spina bifida. They were originally developed by a nurse coordinator

group, which was established at an SBAA conference in 1987, and were endorsed by the SBAA Board of Directors in 1989. The document is reviewed every two years and revised if updating is necessary.

The second guideline identified for children with spina bifida was developed by the Ohio Department of Health (ODH) and is titled *Standards of Care & Outcome Measures for Children with Myelodysplasia (Spina Bifida)*. This guideline was developed in the 1970s and revised in 1995 by the ODH's Myelodysplasia Standards Committee, which is composed of physical therapists, pediatricians, and neurologists. Similar to the other standards developed by ODH, these standards are intended to assist health care workers in assuring that children with spina bifida "achieve maximum habilitation throughout life." This eight-page document focuses on quality management, physiological, functional, psychosocial, and family satisfaction outcomes.

IV. Conclusion

HSR's exhaustive search of the medical literature and national and State organizations identified only a small number of clinical guidelines, practice guidelines, and standards of care for the most common conditions affecting CSHCN in North Carolina. Yet, the guidelines and standards of care included in this report represent the best available information at the national level and include standards of care that have been developed with the consensus and input of public health and private medical experts.

While few in number, the guidelines and standards of care located for this compendium represent the available information in an area of growing interest and research in the health care field. Many professional medical associations, voluntary health organizations, and patient or disease-specific advocacy groups throughout the country view the development of clinical guidelines for the care of children to be a high priority for both medical and public health practice. As noted earlier, many of the organizations contacted through this effort are in the process of developing new guidelines for one or more of the conditions that are the focus of this

document. Individual State organizations are also working to develop consensus in their public and private health communities around standards of care for CSHCN. This trend will likely continue as the need for practice guidelines increases and the emphasis on quality assurance in the health care industry grows.

References

Agency for Health Care Policy and Research. *Using Clinical Practice Guidelines to Evaluate Quality of Care*. Rockville, MD: Agency for Health Care Policy and Research, 1995.

American Academy of Allergy and Immunology. "Practice Parameters for the Diagnosis and Treatment of Asthma." *Journal of Allergy and Clinical Immunology*, November 1995.

Eckman JR, Platt AF. *Problem Oriented Management of Sickle Syndromes*. Atlanta, GA: Grady Memorial Hospital, 1991.

Field MJ, Lohr KN, eds. *Guidelines for Clinical Practice: From Development to Use*. Washington, DC: National Academy Press, 1992.

Gabor V, Hill I. *Technical Assistance to States Developing Comprehensive Systems of Care for Children and Their Families: North Carolina—Initial Site Visit Report*. Washington, DC, August 19, 1996.

Harris-Wehling J, Ireys HT, Heagarty M, eds. *Strategies for Assuring the Provision of Quality Services Through Managed Care Delivery Systems to Children with Special Health Care Needs: Workshop Highlights*. Washington, DC: Institute of Medicine, 1995.

Institute of Medicine, Committee on Clinical Practice Guidelines. *Clinical Practice Guidelines: Directions for a New Program*. Washington, DC: National Academy Press, 1990.

Jaggar SF. Letter to the Honorable William M. Thomas, Chairman, Subcommittee on Health. GAO/HEHS-96-95. May 30, 1995.

McCormick KA, Moore SR, Siegel RA, eds. *Clinical Practice Guideline Development: Methodology Perspectives*. Rockville, MD: Agency for Health Care Policy and Research, 1994.

National Institutes of Health, National Heart, Lung, and Blood Institute. *Management and Therapy of Sickle Cell Disease*. National Institutes of Health, No. 95-2117, December 1995.

Appendix A: Sources of Practice Guidelines on Standards of Care for CSHCN

Sources of Practice Guidelines on Standards of Care for CSHCN

Asthma

American Academy of Pediatrics, Provisional Committee on Quality Improvement. *Practice Parameter: The Office Management of Acute Exacerbations of Asthma in Children*. Pediatrics, 93:119-126, 1994.

Tel: (800) 433-9016

National Institutes of Health. *Executive Summary: Guidelines for the Diagnosis and Management of Asthma*. Bethesda, MD: National Institutes of Health, June 1991.

Publication No. 91-3042A
Tel: (301) 251-1222

Attention-Deficit Hyperactivity Disorder

American Academy of Child and Adolescent Psychiatry. *Practice Parameters for the Assessment and Treatment of Attention-Deficit Hyperactivity Disorder*. Journal of the American Academy of Child and Adolescent Psychiatry, 30, May 1991.

Tel: (202) 966-7300

Cerebral Palsy

Ohio Department of Health and Ohio Chapter, American Academy of Pediatrics. *Standards of Care and Outcome Measures for Children with Cerebral Palsy*. October 1995.

Tel: (614) 466-1700

Congenital Deafness

American Speech-Language-Hearing Association. *Preferred Practice Patterns for the Professions of Speech-Language Pathology and Audiology*. ASHA, 35(3), Supplement No. 11, March 1993.

Tel: (301) 897-5700

Cystic Fibrosis

Ohio Department of Health. *Standards of Care and Outcome Measures for Children with Chronic Pulmonary Disease*.

Tel: (614) 466-1700

Diabetes

American Diabetes Association. *Clinical Practice Recommendations 1995*. Diabetes Care, 18, Supplement 1, January 1995.

Tel: (703) 549-1500

Lawlor MT, LMB Laffel, B Anderson, AM Bertorelli. *Caring for Young Children with Diabetes: Professional Manual*. Boston, MA: Joslin Diabetes Center, 1996.

Publication No. JDC315
Tel: (617) 732-2400

Sickle Cell Disease

Agency for Health Care Policy and Research. *Sickle Cell Disease: Screening, Diagnosis, Management, and Counseling in Newborns and Infants*. Rockville, MD: Agency for Health Care Policy and Research, April 1993.

Publication No. 93-0562
Tel: (800) 358-9295

Ohio Department of Health. *Standards of Care and Outcome Measures for Children with Myelodysplasia (Spina Bifida)*.

Tel: (614) 466-1700

Spina Bifida Association of America. *Guidelines for Spina Bifida Health Care Services throughout Life*. Ed. K Rauen. Washington, DC: Spina Bifida Association of America, 1995.

Tel: (800) 621-3141