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## **Models of Comprehensive, Multidisciplinary Care for Individuals in the United States with Genetic Disorders**

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## **ABSTRACT**

Approaches to providing comprehensive, coordinated care for individuals with complex diseases include the medical home approach, the chronic care model in primary care, and the disease-specific, multidisciplinary, specialty clinic. There is uneven availability and utilization of multidisciplinary specialty clinics for different genetic diseases. For 2 disorders, (ie, hemophilia and cystic fibrosis), effective national networks of specialty clinics exist and reach a large fraction of the target populations. For other disorders, notably sickle cell disease, fewer such centers are available, centers are less likely to be networked, and centers are used less widely. Models of comanagement are essential for promoting ongoing communication and coordination between primary care and subspecialty services, particular during the transition from pediatric care to adult care. Evaluation of the effectiveness of different models in improving outcomes for individuals with genetic diseases is essential. *Pediatrics* 2009;123:407–412

## **KEY WORDS**

Genetic services, care coordination, healthcare disparities, healthcare utilization

## **ABBREVIATIONS**

CDC—Centers for Disease Control and Prevention

CF—Cystic fibrosis

CSHCN—Children with Special Health Care Needs

HRSA—Health Resources and Services Administration

HTC—Hemophilia diagnostic and treatment centers

NIH—National Institutes of Health

SCD—Sickle cell disease

The modern health care delivery system evolved in response to the need to deliver acute, episodic care for infectious diseases and was not designed to provide ongoing care for complex, chronic illnesses.<sup>1,2</sup> Recent attempts to better provide comprehensive, coordinated care for individuals with complex diseases have introduced the medical home concept,<sup>3</sup> the chronic care model in primary care,<sup>4</sup> and the disease-specific, multidisciplinary, specialty clinic.<sup>5</sup> Models of comanagement are essential to promote ongoing communication and coordination between primary care and subspecialty services. It would be inefficient for subspecialists to provide primary care, and ineffective for primary care providers to attempt to stay abreast of the latest therapies for rare diseases.

Both access to specialty care and effective comanagement by primary and subspecialty providers are limited for genetic and other chronic diseases. For example, a study of children enrolled in Medicaid programs in four states during 1989–1992 found that only 36% of those with sickle cell disease (SCD) and 44% of those with hemophilia had at least one visit in a given year with a subspecialist.<sup>6</sup> A similar analysis of the National Health Interview Survey found that <30% of children with special health care needs saw a specialist or subspecialist in a year, regardless of whether they had public or private insurance.<sup>7</sup>

This paper reviews different models of multidisciplinary care provided to individuals diagnosed with three specific genetic disorders: hemophilia, cystic fibrosis (CF), and SCD. The first two disorders have extensive networks of comprehensive specialty care centers whereas no comparable nationwide network of specialized comprehensive treatment centers exists for SCD.

## **MODELS OF COMPREHENSIVE CARE**

The existence of fragmented health care for children with special health care needs led to the development of the medical home concept among pediatricians beginning in the 1970s.<sup>3</sup> In 1992, the American Academy of Pediatrics adopted the recommendation that all children should have a medical home, defined as care that is “accessible, continuous, comprehensive, family centered, coordinated, compassionate, and culturally effective”. The American Academy of Pediatrics, with support from the Health Resources and Services Administration (HRSA) and other organizations, notably the American Academy of Family Physicians and the American College of Physicians, have continued to

develop and popularize the medical home concept, which is no longer restricted to pediatrics.<sup>3,8</sup> As the usual place for both sick and well care, the medical home should be family-centered, culturally effective, accessible, actively engaged in the coordination and provision of primary and subspecialty health care services within the health care system and across other community-based agencies and services (e.g., other clinicians, educational programs, and community-based counseling and support services). A recent survey found that, when adults have health insurance coverage and a medical home (defined as a health care setting that provides patients with timely, well-organized care, and enhanced access to providers), racial and ethnic disparities in health care access and quality are reduced or even eliminated.<sup>9</sup>

The chronic care model, developed with support from the Robert Wood Johnson Foundation, has emerged as an evidence-based mechanism to improve the management of chronic illnesses, such as type 2 diabetes, within the primary care setting.<sup>2,4,10</sup> It was developed for adults with chronic diseases. Pediatric care providers have expanded the chronic care model to include child health, integrating the medical home concept into that model. Both the chronic care model and medical home concept utilize a multidisciplinary practice team that provides coordination of care based on planned visits and follow-up telephone and electronic contacts that focus on monitoring disease markers, delivering preventive services, and coaching patients and family members in disease self-management. The model has been adapted by the Institute for Healthcare Improvement and the National Initiative for Children’s Healthcare Quality, with support from HRSA, as a model for provision of health services in the medical home. Both the medical home concept and the chronic care model together emphasize the need for accessible, ongoing, comprehensive primary care, coordinated and co-managed with specialty care.

One of the central elements of the medical home concept and chronic care model is coordination of care. Coordination implies that the various aspects of comprehensive health care and related services are integrated to promote their delivery as a unified whole.<sup>11</sup> Individuals with complex chronic conditions can receive care through primary care practices in their community or through a clinic providing subspecialty services; in either case, effective coordination among providers is essential. Who provides the coordination of care for individuals with complex diseases requir-

ing multiple subspecialties is an open question.<sup>12</sup> On the one hand, the primary care professional, with expertise in the provision of preventive care, as well as management of common chronic conditions, might be better able to integrate the comprehensive needs of the patient and family. On the other, the primary care professional is less likely to see individuals with rare conditions on a regular basis and might lack training and expertise in the management of these disorders.

Within medical genetics, there is widespread support for specialized clinics that treat individuals with specific diseases or groups of diseases (e.g., metabolic diseases). The goal of such clinics is to provide comprehensive, patient-centered care that brings together multiple medical specialties, as well as disciplines such as nursing, nutrition, and social work.<sup>5,13</sup> Furthermore, genetic counseling, reproductive counseling, and prenatal diagnosis are believed to benefit both family members and affected individuals. In addition to disease-specific centers, a multidisciplinary clinic could, in principle, treat individuals with a variety of complex disorders.<sup>14</sup>

Models of comprehensive care for genetic diseases often include specialized, disease-specific centers or clinics that provide multidisciplinary, family-centered care. Individuals with disorders that affect multiple organ systems have varied needs that require teams from multiple medical specialties and other disciplines, including nursing, nutrition, and social work. Coordination of care from different providers; case management services to monitor preventive practices; integration or coordination with early intervention and special education services; and social work or legal assistance with issues relating to health insurance, disability benefits, and social services can also be important components of comprehensive care.

Specialty treatment centers can take different forms. Certain centers provide dedicated facilities with core staff available on a daily basis. Others offer periodic clinics that bring together a team of different staff members on a regular, intermittent schedule (i.e., weekly or monthly). Comprehensive, multidisciplinary care should not be equated with one particular modality of service delivery. Regardless of the model for accessing and providing subspecialty services, it is important to remember that specialty centers or clinics focus on the provision of regular (e.g., quarterly or annual), disease-focused surveillance visits, specialized care, and coordination of care and psychosocial needs specific to the

single-gene condition. These centers generally do not take the place of a primary care provider serving as a medical home for routine preventive services such as immunizations and screenings, and any provision of acute care services will be focused on expected complications of the disorder. In addition, they can provide assistance via telephone for medical emergencies specific to the condition.

Experts have attributed the low availability or use of comprehensive specialty care for genetic and other chronic conditions to a variety of factors. These include restrictions by health plans on full reimbursement for care by providers outside the plan's network; lack of specific billing codes for care coordination and services provided by nonmedical staff; capitated reimbursements; and, perhaps most critically, limitations on the number of services that can be billed on a single visit or day.<sup>15,16</sup> In particular, although a child might be seen by multiple subspecialists during a clinic visit, insurers typically refuse to pay for >1 physician's time. It is unclear whether referral requirements constitute a barrier to access.<sup>7</sup> Nonfinancial barriers include the distance to specialty clinics that individuals must travel.<sup>17</sup>

## **DISORDER-SPECIFIC TREATMENT CENTERS**

### **Hemophilia**

Multidisciplinary outpatient centers for patients with hemophilia that provide medical and psychosocial support to patients and their families and develop individual management plans to minimize complications were first established in Britain in the mid-1950s.<sup>18</sup> In 1970, the availability of a concentrated form of clotting factor (cryoprecipitate) allowed hemophilia centers to promote home infusion or self-therapy, which was reported to markedly reduced hospitalization rates among patients with hemophilia.<sup>19</sup>

During the 1970s, several states and the National Hemophilia Foundation worked with the HRSA to establish a US network of hospital-based hemophilia diagnostic and treatment centers (HTCs) to provide comprehensive services for patients and families within one treatment facility.<sup>18,20</sup> In 1975, the US Congress appropriated funds for the HRSA to establish a network of 22 HTCs, each including a coagulation laboratory, a blood bank, and a multidisciplinary hemophilia treatment team, and states funded an additional 21 HTCs.<sup>18,21</sup> The promotion of home infusion therapy by these HTCs was reported to be associated with fewer hospital and emergency department visits.<sup>5</sup>

In the 1980s most hemophiliacs became infected with human immunodeficiency virus and hepatitis through blood products. In 1985, the CDC began funding HTC through the HRSA for AIDS risk reduction, which in 1995 was expanded through direct funding from the CDC to the HTCs to provide ongoing surveillance for blood product safety and a program to prevent complications in this population. An evaluation using population-based surveillance data showed that during 1993–1995 HTCs were successful in reducing both mortality and hospitalizations by approximately among patients with hemophilia who attended HTCs.<sup>22,23</sup> The Universal Data Collection (UDC) project initiated by the HTCs and the CDC in 1998 to measure and improve the health of people with bleeding disorders<sup>18</sup> has demonstrated that obesity is a major risk factor for limitation of joint range of motion in individuals with hemophilia.<sup>24</sup>

Currently, >130 HTCs receive federal funding through the HRSA and the CDC to provide comprehensive care and preventive services to >15 000 individuals in the United States with hemophilia and to >10 000 patients with other bleeding disorders.<sup>5</sup> HTC core team members include a medical director (often an adult or pediatric hematologist) with extensive training and experience in the care of people with bleeding disorders, a nurse coordinator, a psychosocial professional, and a physical therapist. Extended members can include dentists, orthopedists, and genetic counselors. Field clinics and telemedicine services provided by HTC staff can extend comprehensive services to individuals in remote areas. The NHF Medical and Scientific Advisory Council issues care recommendations for the HTCs in the United States.<sup>25</sup> More recently, federally funded HTCs became eligible to participate in the 340B drug pricing program, which lowers the price of clotting factor for consumers and helps the financial sustainability of the HTCs. In addition to an ongoing HTC hemophilia data reporting system (focused on clinical services) supported by the HRSA, the UDC project which has shown the importance of monitoring complications in male patients  $\geq 2$  years of age<sup>24</sup> has been expanded to include special populations such as babies (0–2 years of age) and females (all ages) with bleeding disorders.<sup>26</sup>

Hemophilia management today increasingly focuses on minimizing hemorrhages into joints and (less commonly) into the gastrointestinal tract and the brain, preventing degenerative joint arthropathy, and improving health-related quality of life.<sup>18</sup> Researchers are evaluating the optimal pro-

ocols for on-demand or prophylactic clotting factor replacement therapy in terms of resource costs, morbidity and health-related quality of life.<sup>27</sup> With fewer complications, hemophilia patients require fewer HTC visits, and falling demand for services has led to the attrition of specialized physicians.<sup>16</sup>

### **Cystic Fibrosis**

Recommendations in the United States and Europe state that children and adults with CF should receive regular care from specialty CF centers.<sup>28,29</sup> In the United States, a network of more than 115 comprehensive, multidisciplinary CF care centers accredited and funded by the CF Foundation provide care to the large majority of individuals with diagnosed CF, especially children. CF care center teams include physicians, nurses, nutritionists, respiratory therapists, social workers, genetic counselors, and other medical professionals. The CF Foundation, using expert panels of physicians and scientists, publishes consensus guidelines that call for quarterly visits with at least annual testing to monitor disease severity and complications.<sup>28</sup>

The CF Foundation maintains a national registry of patients attending accredited CF care centers based on data submitted by center personnel.<sup>30</sup> A second, industry-sponsored patient registry<sup>31</sup> also includes a small number of patients who do not attend accredited CF centers. Registry data can be used to compare health outcomes among CF patients across centers and to correlate outcomes with the type of care provided. Analyses have reported substantial differences across centers in survival and other endpoints that appear to be associated with differences in the quality of care provided.<sup>32</sup> Specifically, centers in the top quartile for patients' median pulmonary function monitored their patients with more frequent visits, airway cultures, and pulmonary function tests.<sup>33</sup> Furthermore, CF centers with better pulmonary function hospitalized their patients more and used more oral and IV antibiotics.<sup>33</sup> A recent study that examined the care provided during infancy at centers whose 6–12-year-old children had superior lung function compared with children with CF in the rest of the country also found that these centers had more frequent monitoring visits, performed more airway cultures, and treated more aggressively with antibiotics during infancy than did comparison centers.<sup>34</sup>

## Sickle Cell Disease

It is generally agreed that optimal management of individuals with SCD requires access to comprehensive, multidisciplinary care.<sup>35-38</sup> The comprehensive care team should include a physician familiar with the multiple complications and presentations of SCD; one or more “physician extenders”, such as a physician assistant or nurse practitioner; a health educator; and a medical social worker, and should have access to laboratory services, radiology, and a 24-hour blood bank.<sup>36</sup> A knowledgeable primary care provider can coordinate care within the medical home,<sup>35</sup> but this requires expertise, professional support, and time. Others recommend that the primary care medical home collaborate with SCD centers or subspecialists.<sup>39</sup>

Although SCD is more common than CF or hemophilia in the United States, only a minority of affected individuals in this country are seen at specialized SCD centers.<sup>40</sup> While newborn screening and comprehensive care have made it possible for the majority of patients to survive into adulthood, many adult patients lack access to adult specialists and continue to have inadequate or unmet needs. US managed-care plans often require individuals to be seen by primary care providers, regardless of expertise, and are said to impose barriers to timely access to specialists for acute SCD care.<sup>15,16</sup>

Adequate care for individuals with SCD requires that they receive both specialized services and comprehensive primary care. The fact that only a minority of affected individuals receive specialist care<sup>6</sup> points to the need to not only improve access to specialty services, but also to improve coordination of comprehensive care provided in community primary care settings, including private practices, clinics, and community health centers (CHCs). The HRSA Bureau of Primary Health Care has adopted the chronic care model as a framework for improving chronic care management in CHCs for a number of chronic conditions. This primary care quality improvement model has the potential to improve coordinated care for individuals with SCD.

Since 1972, a network currently comprising 10 SCD comprehensive clinical and research centers has received funding from the NIH. A primary focus of the NIH funding is to support the infrastructure for enrolling patients with SCD in clinical research studies. It is unclear to what extent these centers provide family-centered care comparable with provided by HTC or CF centers. Legislation passed in 2004, the Sickle Cell Disease Treatment Act, authorized the

HRSA to fund up to 40 community-based networks of care to link primary and subspecialty care to provide coordinated and comprehensive care for individuals with SCD.<sup>39,40</sup> The law received appropriations in 2006 (\$2 000 000), and in late 2006 the HRSA funded four local SCD networks and one national coordinating center to enhance SCD care through coordination of service delivery, genetic counseling and testing, bundling of technical services, and training of health professionals. The primary goal of these networks is to ensure that all individuals served have access to a medical home, with coordination and continuity of education, treatment, and care.

Among the major challenges in SCD care are management of acute pain crises, most commonly associated with vasoocclusive events and acute chest syndrome.<sup>38</sup> Effective pain management in SCD incorporates pharmacologic, behavioral, and physical pain management strategies.<sup>41</sup> New technologies and treatments, such as hydroxyurea, chronic transfusions with oral chelation therapy to prevent iron overload, and bone marrow transplantation, offer promise for the prevention of painful crises, although evidence of long-term outcomes is still needed, as well as data on which groups are most likely to benefit.<sup>42</sup> New practices typically require specialized clinical experience and technical expertise; therefore, they are rarely used except in the context of comprehensive care centers.<sup>43</sup> An alternative to separate outpatient pain management facilities is to provide multidisciplinary teams within emergency departments or hospitals.<sup>44,45</sup>

Although experts believe that children with SCD experience lower morbidity and mortality if seen by providers who offer comprehensive and coordinated care,<sup>46</sup> the lack of active SCD surveillance of long-term health outcomes makes it difficult to assess quality of care or outcomes.<sup>40</sup> Black or African-American patients with SCD who live in remote or rural areas are likely to access SCD centers for acute care but not comprehensive care.<sup>17,47</sup> In Alabama, the Children and Youth Sickle Network<sup>SM</sup>, extended services to rural areas beginning in 1995, resulting in sharp reductions in the average age at first visit to a sickle cell clinic and the mortality rate from sepsis.<sup>48</sup> This network is part of the newly funded HRSA SCD networks. It is expected that the HRSA funded networks will establish a surveillance system to monitor long-term health outcomes within the four networks. Additionally, the establishment of the networks will afford the possibility of evaluating treatment protocols.

## CONCLUSIONS

Two different approaches to the provision of care for multi-system genetic diseases have been implemented in the United States. One approach makes disease-specific center-based comprehensive, multidisciplinary care responsible for providing all or most disease-specific care and coordination of such care. This approach requires the availability and accessibility of specialized treatment centers throughout the United States for individuals of all ages, which is true for both CF and hemophilia. Both CF centers and HTC have established patient registries to monitor outcomes and evaluate the effectiveness of treatment protocols, which have been invaluable for demonstrating improvements with high-quality comprehensive care provided by specialized centers. The other approach relies on primary care providers as the dominant source of care, with subspecialists available for referral. This approach characterizes care for individuals with SCD. A small number of comprehensive SCD centers with a research orientation exist, and an even smaller network of SCD and community health centers funded by the HRSA for the delivery of comprehensive care. Most individuals with SCD, particularly adults, do not utilize subspecialty or multidisciplinary care. In the absence of either registries or population-based surveillance, little is known about the clinical outcomes of individuals with SCD or how outcomes differ depending on the type or model of care that is provided.

Comprehensive care can be difficult to pay for through individual health insurance, as discussed above. HTCs and CF centers have institutional sources of funding that are not available for SCD care. The HRSA Children with Special Health Care Needs (CSHCN) program provides funding to states to provide, reimburse, or coordinate medical care to children with specified chronic health conditions, with upper age cutoffs set by states at 18 to 21 years. In addition, 17 states provide their own funds to help pay for medical care for adults with certain genetic conditions, including CF (12 states), hemophilia (8 states), and SCD (2 states).

Whether comprehensive care provided by multidisciplinary teams, in either disease-specific centers or through coordination of care by primary healthcare providers, improves measures of quality of care and health outcomes is an important issue that requires careful study. Although individuals with hemophilia who use HTCs are reported to have better outcomes,<sup>22,23</sup> few studies have compared out-

comes by groups defined by access to center-based care. One study found no difference in SCD mortality in Tennessee in relation to geographic access to SCD center care.<sup>49</sup> One cannot take for granted that either specialty clinic staffs or primary care providers deliver evidence-based, patient-oriented care. It is important to measure the extent to which evidence-based interventions are prescribed in a standardized manner. Data from CF centers demonstrate that if approaches used at the highest performing centers were adopted universally, dramatic improvements in health outcomes could be achieved.<sup>31</sup> In addition, it should not be assumed that specialty care is sufficient to optimize health and well-being for individuals with complex chronic conditions. Primary preventive care and acute care are both essential components of comprehensive health care.

To ensure that comprehensive care is provided to individuals over the lifespan, it is essential that the transition from pediatric to adult care be planned with the involvement of adult care providers. Traditionally, care has typically been provided by pediatric subspecialists, because the greatest opportunities for prevention and lifelong prevention of secondary disabilities are among early-diagnosed children with these disorders. However, successful prevention efforts have resulted in most individuals with CF, hemophilia, and SCD now surviving to adulthood. As individuals transition to adulthood, it is essential to bridge pediatric or adolescent care and adult care. HTCs and CF centers routinely provide care to both children and adults, easing the transition of care. Providers of adult primary and specialty care for individuals with SCD need to be as informed and involved in coordination of care as are pediatricians or primary subspecialists.

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