



National Research Agenda on Health Systems for Children and Youth With Special Health Care Needs

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ABSTRACT

Children and youth with special health care needs (CYSHCN) “have or are at increased risk for chronic physical, developmental, behavioral or emotional conditions and also require health and related services of a type or amount beyond that required by children generally.” CYSHCN rely on health systems, which extend beyond traditional health care entities, to optimize their health and well-being. The current US health system is not fully equipped and functioning to meet the needs of CYSHCN. Recognizing this, the Maternal and Child Health Bureau and the US Health Resources and Services Administration established the Research Network on Health Systems for CYSHCN (CYSHCNNet, <http://www.cyshcnet.org>). With input from >800 US stakeholders, CYSHCNNet developed a national research agenda on health systems for CYSHCN designed to: 1) highlight important health system challenges faced by key stakeholders (ie, patients and families, health care providers,

insurers, administrators, etc.); 2) organize research topics and goals to identify opportunities for improvement, to address challenges and to promote progress toward the ideal health system; and 3) provide a blueprint for health systems research ideas and studies that will guide CYSHCN investigators and other stakeholders going forward. We introduce the 6 research topics currently included in the research agenda—transition to adulthood, caregiving, family health, child health care, principles of care, and financing—to inform and guide investigators as they embark on a trajectory of health systems research on CYSHCN.

KEYWORDS: children and youth with special healthcare needs; health services research; research agenda

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WHAT'S NEW

This paper introduces the papers in the Supplement that describe a new national health systems research agenda for children and youth with special healthcare needs (CYSHCN). It summarizes each paper, and provides readers with examples of how they might be used by different types of stakeholders undertaking or using health systems research for CYSHCN.

CHILDREN AND YOUTH with special health care needs (CYSHCN) are those who “have or are at increased risk for chronic physical, developmental, behavioral or emotional conditions and also require health and related services of a type or amount beyond that required by children generally.”¹ An estimated 14.1 million US children and youth (~20%) have a

special health care need.¹ Examples of special health care needs include use of chronic medications, durable medical equipment and supplies, therapies, as well as health services across the care continuum (eg, home, outpatient, inpatient, emergency, perioperative). CYSHCN as a population face a heterogeneous array of health challenges and chronic conditions, including mental and physical.

According to the World Health Organization, a health system comprises all organizations, institutions, and resources that produce actions whose primary purpose is to improve health and well-being.² CYSHCN rely on systems which extend beyond traditional health care entities (eg, hospitals, clinics) to include the network of supports and programs related to education, housing, food, environment, etc., that—when functioning well—enable CYSHCN and their families to reach their optimal level

of health and wellness. It is critically important to consider the health system for CYSHCN in this broad context, because the health of CYSHCN is dynamic and it is dependent on each of these system components as well as their interactions. Unmet needs in any area can substantially affect the health of CYSHCN.

RATIONALE FOR A NATIONAL HEALTH SYSTEMS RESEARCH AGENDA

The fact that the US health system is not well equipped to serve CYSHCN was recognized over 30 years ago.³ As decades passed, there have been groundbreaking advances in diagnosis, medical and surgical treatment, and therapies for many CYSHCN. These advances have enabled them to live in better health. However, fundamental gaps and deficiencies in health systems for CYSHCN remain, leaving many children and families with unmet health care needs, unnecessary caregiving burdens, and suboptimal health, well-being, and quality of life.⁴ Moreover, the different systems that impact the health of CYSHCN do not interact and integrate sufficiently.

To measure a well-functioning health system for CYSHCN, the Maternal and Child Health Bureau (MCHB) and their partners developed the following 6 Core Outcomes⁵: 1) Family professional partnerships to improve care at all levels; 2) Provision of family-centered, coordinated, comprehensive care through the Medical Home; 3) Adequate insurance/financing for needed services; 4) Screening of all children early and continuously for special health care needs; 5) Organization of services so that families can easily access and use them; and 6) Transitions for youth with special health care needs to help them receive the services they need in all aspects of adult life.⁵

To catalyze systems-level action around the MCHB Core Outcomes and operationalize them in practice, the Association of Maternal and Child Health Programs, in collaboration with the Lucile Packard Foundation for Children's Health and the National Academy for State Health Policy, developed the Standards for Systems of Care for CYSHCN (currently on version 2.0).⁶ The Standards delineate the structure and processes of an ideal health system for CYSHCN in relation to known best practices, federal law and requirements, and relevant measures of quality of care. Together, the MCHB Core Outcomes and National Standards for Systems of Care are the foundation on which high-quality, family-centered health systems can be built and sustained.

In 2017, the US Health Resources and Services Administration MCHB established the Research Network on Health Systems for CYSHCN (CYSHCNet, <http://www.cyshcnet.org>).⁷ CYSHCNet is an interdisciplinary, multi-site, collaborative research network that leads, promotes, and coordinates national research activities to strengthen the evidence base related to key components of a comprehensive, high quality system of care for CYSHCN. As a central piece in the CYSHCNet portfolio of work,

CYSHCNet developed a national research agenda on health systems for CYSHCN. The research agenda, informed by the Core Outcomes and Standards (v2.0), highlights and prioritizes important health system challenges faced by key stakeholders (eg, patients and families, health care providers, insurers, policymakers), organizes research topics to identify opportunities for health system improvement, and provides a blueprint for research studies that will guide CYSHCN researchers, funders, and consumers.

DEVELOPMENT OF THE NATIONAL RESEARCH AGENDA

To create the research agenda, Collier et al⁸ solicited responses from a wide range of US stakeholders, including families of CYSHCN, health care providers, researchers, and policymakers, to identify what research with or about CYSHCN is the most important to conduct. Using an adapted RAND/UCLA Appropriateness Method (a modified Delphi approach), an expert panel rated research topics across 3 domains: need and urgency, research impact, and family centeredness. The 15 topics that achieved the highest rankings across domains were grouped into the following priority areas: social determinants of health (SDH) (including disparities and rurality); caregiving (family resilience and care at home); clinical model refinement (effective model elements, labor divisions, telemedicine, and system integration); value (stakeholder-centered value outcomes, return on investment, and alternative payment models); and youth-adult transitions (planning, insurance, and community supports).

To help validate the expert panel's interpretation, these topics were evaluated through focus groups by a broad group of CYSHCN stakeholders, including CYSHCN and their families from diverse racial/ethnic and socioeconomic backgrounds as well as health care providers across the care continuum. Stakeholders refined and unified the research agenda topics into the following 6 areas: transition to adulthood, caregiving at home, family health, impact of care on child health, principles of care, and health care financing.

The seven manuscripts in this supplement cover the six topics in the research agenda; one topic (impact of care on child health) is discussed in 2 manuscripts (addressing SDH and telehealth) to accommodate its breadth (Table). They are organized loosely in a progression from care experienced by patients and families (transitions of care, caregiving at home, and family health) through health care provision (SDH; telehealth) to organization of health care (care principles and financing). Each paper 1) describes what is known about each topic, 2) highlights important knowledge gaps, and 3) provides examples of research to address these gaps and advance the health system for CYSHCN. To complement the set of papers, we introduce the research topics below and then discuss the implications of their use, limitations, and future directions.

Table. Topics of the National Research Agenda on Health Systems for Children and Youth With Special Health Care Needs

Topic	Definition	Example Research Question
Transition to adulthood	Movement from adolescence to adulthood in all areas, including home, health care, education, and community	What health system model best accomplishes transition to adulthood?
Caregiving	Tasks, resources, and services needed to meet the day-to-day needs of CYSHCN at home	How can home health care providers enable families to deliver efficient and effective caregiving at home for CYSHCN?
Family health	Mental, physical, and spiritual health of parents, siblings, and extended family of CYSHCN and their impact on the family	What is the most effective way to optimize family adaptability, resilience, and health (emotional and physical) for CYSHCN?
Social determinants of health	Conditions in the environments where the children and their families live, learn, work, and play and how these conditions influence access to and quality of health care as well as health outcomes	What is the best way to screen for and address risk factors associated with social determinants of health in a health system for CYSHCN?
Telehealth	Phone- and video-based health care encounters; patient-provider communications with secure messaging, email, and file exchanges; and use of remote monitoring in a patient's home	Which health services are safe and effective to conduct using telehealth for CYSHCN? Which services are not safe to conduct?
Principles of care	Identification of CYSHCN in an outpatient clinic practice, offering medical home services, integration with community entities and resources, facilitation of high-quality care transitions (eg, hospital to home), quality improvement, and use of health information technology for care planning and coordination	How can implementation of standards on care principles for CYSHCN improve their health and well-being?
Financing	Direct and indirect costs and payment for health care delivered across the care continuum, including that incurred by patients and families	Which health system payment models for CYSHCN enable high-quality care and the best health outcomes?

CYSHCN indicates children and youth with special health care needs.

RESEARCH AGENDA TOPIC PAPERS

TRANSITION TO ADULTHOOD

In the first paper, Okumura et al⁹ discuss health care transitions (HCT) from pediatric to adult health care systems. The goal of HCT is to improve the ability of youth to manage their health and health care to maintain their optimal health and well-being. Gaps in knowledge on HCT include distinguishing which HCT service models are most effective across health care systems, understanding how to feasibly implement those models, and how health care financing can best incentivize HCT and enable accountability for HCT outcomes. Recommended research on CYSHCN transition to adulthood includes 1) developing process and outcome measures that distinguish successful transitions, 2) partnerships with youth and families to develop and implement effective transition health services models, and 3) evaluate fiscal and policy models that incentivize the processes of transition planning and implementation.

CAREGIVING AT HOME

Hoover et al¹⁰ highlight the need for health system research on CYSHCN caregiving. Caregiving encompasses the set of tasks, resources, and services that meet the day-to-day needs of CYSHCN at home. Extensive knowledge gaps in caregiving for CYSHCN exist. For example, little is known about how to 1) integrate the amount of time and effort required of home caregivers to implement plans of care that are developed by CYSHCN health care professionals; 2) measure and optimize the efficiency and effectiveness of home caregiving; and 3)

acknowledge and integrate home caregivers as full partners in the CYSHCN health care workforce. The authors recommend research on caregiving for CYSHCN, including the development of an actionable framework of full integration between professional health care providers/services and family caregivers that will accurately reflect home caregiving roles and labor. They also propose evaluation of interventions that are best positioned to support and enhance the tasks performed by family caregivers.

FAMILY HEALTH

Kuhlthau et al¹¹ discuss research on family health, which includes the mental, physical, and spiritual health of parents, siblings, and extended family of CYSHCN and their impact on the family. Family health is often overlooked in clinical research, yet a well-functioning family unit can improve outcomes for CYSHCN and decrease stress and caregiver strain. Kuhlthau et al¹¹ identify gaps in research that include understanding 1) what helps families thrive, 2) how family health varies by diversity of family attributes, and 3) health and well-being of siblings and extended family members. Recommendations for family health research include measurement of family health, distinguishing which interventions best improve family mental health and resilience, and understanding how the health of CYSHCN is influenced by family health and well-being.

SOCIAL DETERMINANTS OF HEALTH (SDH)

Van Cleave et al¹² explore research on SDH for CYSHCN, including the conditions in the environments where the children and their families live, learn, work,

and play and how these conditions influence access to and quality of health care as well as health outcomes. Gaps in SDH knowledge include 1) how to best screen CYSHCN for SDH risk factors, including adverse childhood experiences; 2) how to educate and empower care coordinators to address SDH needs for CYSHCN; and 3) how to best align health system priorities with SDH needs of CYSHCN and their families. Suggested research on SDH for CYSHCN includes evaluation of SDH screening strategies, understanding family perceptions of success when evaluating outcomes for interventions on SDH, longitudinally assessing SDH risk factors in CYSHCN over time, and correlating SDH risk factors and outcomes with the children's underlying chronic conditions and other clinical attributes.

TELEHEALTH

Van Cleave et al¹³ highlight health systems research on telehealth for CYSHCN. Telehealth includes phone- and video-based health care encounters; patient-provider communications with secure messaging, email, and file exchanges; and use of remote monitoring in a patient's home. Although telehealth has emerged as an important way to access health services and treatments for CYSHCN during the coronavirus pandemic, critical knowledge gaps in telehealth use remain, including 1) barriers and facilitators of implementation and use among CYSHCN and families; 2) which health services can be offered safely via telehealth; and 3) how to mitigate disparities created by telehealth (eg, digital divide of impaired health care access in CYSHCN and families with limited digital resources). Suggested studies on telehealth for CYSHCN include the following: interviews and surveys with families and providers to compare perceptions of trust and value with telehealth across different health services (eg, well child care, urgent care, therapies, etc.); targeted assessments of telehealth among vulnerable CYSHCN who have barriers to internet and computer access and use; and evaluations of remote monitoring for chronic condition management, including measurement of feasibility and efficacy.

PRINCIPLES OF CARE

Kuo et al¹⁴ highlight the research agenda on principles of care for CYSHCN. Examples of these principles include identification of CYSHCN in an outpatient clinic practice, offering medical home services, integration with community entities and resources, facilitation of high-quality care transitions (eg, hospital to home), and use of health information technology for care planning and coordination. Examples of existing research gaps in principles of care for CYSHCN include lack of standard definitions and measurement practices for the principles themselves as well as for the training and roles of health care team members, including family partners. Kuo et al¹⁴ propose several studies to fill these gaps, including strategies to implement and operationalize the principles across health care settings—with a focus on family partnerships—and

also strategies to assess the effectiveness of the principles on the health outcomes of CYSHCN.

FINANCING

In the final paper, Kuo et al¹⁵ highlight health care financing for CYSHCN, which includes direct and indirect costs and payment for health care delivered across the care continuum, including that incurred by patients and families. The paper highlights inequities in financing of core health care services (eg, outpatient and inpatient care) and community-based services (eg, home nursing) for CYSHCN, which often are insufficiently funded by insurers and/or are often subsidized by patients and families themselves. The paper also draws attention to discordance among families, clinicians, and insurers on which health services offer the greatest value to CYSHCN and therefore merit the largest investments. Gaps in knowledge on financing for CYSHCN include which financing models and insurer coverage plans benefit CYSHCN the most, and underwrite and enable high-quality care coordination. Research proposed by Kuo et al¹⁵ includes comparative effectiveness studies of payment models on access, utilization, and health care outcomes for CYSHCN; evaluation of the return on the financial investment for care coordination; and variation in perceptions of health care value and investments in health services across CYSHCN stakeholders.

DISCUSSION

The overarching goal of the National Research Agenda on health systems for CYSHCN conveyed in this supplement is to facilitate research that will optimize systems of care for CYSHCN and their families. The six research topics currently included in the agenda—transition to adulthood, caregiving, family health, impact of care on child health, principles of care, and financing—are intended to inform and guide investigators as they embark on a trajectory of CYSHCN research to achieve this goal. The topics and projects outlined in the research agenda are responsive to MCHB's Core Outcomes for CYSHCN and also to Association of Maternal and Child Health Program's Standards for Systems of Care for CYSHCN.⁶

CONSIDERATIONS WHEN USING THE NATIONAL RESEARCH AGENDA FOR CYSHCN

Depending on the research agenda topic, a variety of research cohorts, designs, settings, and analyses will be necessary to produce actionable findings. Throughout the questions and project examples discussed in the papers, there is variation in the type of research needed to advance knowledge (eg, observational, intervention-based, and implementation). The illustrative examples of research provided in the research agenda papers complement the current state of research in each topic area. For example, because little information is known about optimal health care financing for CYSHCN, observational studies have been proposed. In contrast, because substantially more information is known about CYSHCN transition to

adulthood, implementation research on targeted interventions has been emphasized.

It is important to recognize that the research agenda does not focus on specific chronic conditions experienced by CYSHCN. There has been considerable, important research on health care and outcomes of CYSHCN with common, chronic conditions, including asthma and attention deficit hyperactivity disorder, among others. Maintaining focus on a health *systems* research agenda, the intent of the research agenda is to promote research that is positioned to yield findings that will generalize across populations of CYSHCN with a variety of chronic conditions, including those associated with less or more medical complexity.

It is critical to consider the clinical practice and policy implications of all health systems research on CYSHCN early in the research development process. Involvement of key stakeholders, including but not limited to patients and families, frontline clinical providers, health system administrators, insurers, and legislators, is paramount to ensure that the research is ideally positioned to produce actionable and valuable results that will be used to advance the health system for CYSHCN. Multi-faceted dissemination strategies may be necessary to sufficiently bring attention to the findings by the stakeholders who are most equipped to act on them. Peer-reviewed publication in a reputable medical journal alone may not be adequate.

The research proposed across topics is purposefully broad. Although the topics are not organized by health service (eg, inpatient, outpatient, home, or perioperative), they may be applied to one or more services. Also, due to the natural winnowing process that occurred during the process of research agenda development, topic exclusion from the current research agenda does not necessarily imply that a nonincluded topic is not meaningful or important to investigate. For example, research on the CYSHCN workforce adequacy and the use of social media in health care are not currently highlighted, but nevertheless merit investigation. Moreover, although health equity is not distinguished as specific topic, it applies to each of the six research topics and should be investigated within all of them.

Creativity and focus may be necessary for users of the research agenda to develop a research study (or portfolio of studies) from the information provided on a research agenda topic. For example, *investigators* interested in research on family caregiving may benefit from narrowing the research question on a particular health system aspect for CYSHCN (eg, family caregiving for CYSHCN who are recovering from major musculoskeletal surgery at home) or integrating an important, additional construct (eg, SDH) into the research design. In this manner, the research agenda can facilitate collaborations across researchers from different fields with intersecting interests. *Research funders*, in contrast, might use the key questions in a specific area more broadly, to help shape investment and funding opportunities. In some cases, a portfolio of projects within a single area may be needed to create a comprehensive and generalizable body of knowledge. For example, if a funder is interested in developing a request for

applications to inform and test innovations in health care financing for CYSHCN, they might consider including not only the “highest priority” questions described in the financing paper in this supplement, but also the high- and medium-priority questions developed and described in the initial agenda setting study.⁸ Finally, *family partners* who work on research teams should view the key questions of the research agenda through the lens of their own experiences, to generate specific examples of situations that are meaningful to them as well as their child’s clinical team. For example, a family living in a rural community might use their specific experiences with telehealth to create and evaluate an intervention, in partnership with an urban, academic research team, to help families in rural communities gain access to the types of services they need most, in ways that help their children the most.

Ideas and resources related to each paper in this supplement have also been included in learning modules that go into more depth on each paper’s topic area. These modules will be released in early 2022 on the CYSHCN website (www.cyshcnet.org), and will connect readers to resources to explore the topic areas in more depth. This interactive platform will also allow researchers and other users of the agenda to suggest additions and revisions to the agenda over time. Key findings from a selection of the papers are also summarized in a webinar series, conducted in late 2021 and early 2022, available on the CYSHCN website and also the website of the Lucile Packard Foundation for Children’s Health (www.lpfch.org).

LIMITATIONS

The process of generating and prioritizing research domains and topics for the research agenda produced an abundance of ideas that could not all be mentioned in the supplement. As noted above, while the research agenda topics were prioritized the most by stakeholders, the topics are not exhaustive, nor inclusive of all important health systems research needed for CYSHCN. The importance of the research agenda topics is likely to change over time along with the evolution of the health system. A research agenda is useful only to the extent that it aligns with policy and practice imperatives, which will also change with time. As a result, the research agenda will be dynamic and responsive to the pertinent needs of CYSHCN and families over time.

CONCLUSION

The 6 research topics covered in the supplement encompass the core of the National Research Agenda on Health Systems for CYSHCN. Collectively, they will guide investigators and other agenda users as they embark on a transformative research trajectory towards a better system of care for CYSHCN.

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