

Health System Research Priorities for Children and Youth With Special Health Care Needs

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abstract

OBJECTIVES: In this study, we sought to establish priorities for a national research agenda for children and youth with special health care needs (CYSHCN) through a structured, multistakeholder, mixed-methods approach.

METHODS: Using surveys, we solicited responses from >800 members of expert-nominated stakeholder organizations, including CYSHCN families, health care providers, researchers, and policymakers, to identify what research with or about CYSHCN they would like to see in a national research agenda. From 2835 individual free-text responses, 96 research topics were synthesized and combined. 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. Domains were rated on 9-point Likert scales. Panelist ratings were used to sort research topics into 4 relative-priority ranks. Rank 1 (highest priority) research topics had a median of ≥ 7 in all domains.

RESULTS: The RAND/UCLA Appropriateness Method was used to prioritize CYSHCN research topics and depict their varying levels of stakeholder-perceived need and urgency, research impact, and family centeredness. In the 15 topics that achieved rank 1, social determinants of health (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) were emphasized.

CONCLUSIONS: High-priority research topics identified by CYSHCN experts and family leaders underscore CYSHCN research trends and guide important directions. This study is the first step toward an efficient and cohesive research blueprint to achieve highly-effective CYSHCN health systems.



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WHAT'S KNOWN ON THIS SUBJECT: Achieving comprehensive, effective, and coordinated systems for children and youth with special health care needs (CYSHCN) has been elusive. Realizing ideal health systems for CYSHCN requires research to concentrate on the most pressing issues identified by diverse stakeholders.

WHAT THIS STUDY ADDS: As a step toward a national CYSHCN research agenda, diverse experts developed a research topic taxonomy, depicting varying levels of need and urgency, research impact, and family centeredness. Highest-ranked research topics were centered on social determinants of health, family resilience, value, and youth-adult transitions.

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Children and youth with special health care needs (CYSHCN) have, or are at an 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 A system of services for CYSHCN (ie, a “family-centered network of community-based services designed to promote the healthy development and well-being of CYSHCN and their families”²) includes their health care, mental health, education, public health, and social services. This health system is an important improvement target because CYSHCN make up nearly 20% of US children,³ and although by definition they require more health services than otherwise healthy children,¹ they are the most vulnerable to problems with access.⁴ Health system design impacts cost and care quality for children with chronic illness,⁵ and effective systems provide mechanisms to improve CYSHCN outcomes.^{6,7}

Health system innovation has increased over the last 3 decades as CYSHCN definitions, data, and policies have increased in prominence.^{1,2,8-14} The US Maternal and Child Health Bureau (MCHB) 6 Core Outcomes in 2001¹⁵ and Association of Maternal and Child Health Programs (AMCHP) Standards for Systems of Care in 2014¹⁶ have advanced progress in developing comprehensive CYSHCN systems.² The MCHB outcomes (eg, comprehensive care within a medical home and adequate insurance) provide means to measure progress toward achieving the health systems CYSHCN require. The AMCHP Standards for Systems of Care also represent structures (eg, capacity for timely access to services) and processes (eg, transportation assistance provided to families with difficulty) presumed to improve CYSHCN health outcomes.

Despite these efforts, a reliable, effective, comprehensive system of care for CYSHCN has been elusive.² Only 16% of US CYSHCN receive care meeting all 6 MCHB core outcomes; only 43% receive care within a medical home.³ To create health systems that achieve the best possible CYSHCN outcomes, research needs to be concentrated on the most pressing issues identified by diverse stakeholders. However, CYSHCN research has myriad potential topics and stakeholders. If research priorities are not cohesive, researchers could conduct substantial work without driving demonstrable forward progress in population-level CYSHCN health and health care. A guiding framework thoughtfully developed by stakeholders might facilitate more efficient, cohesive, and, ultimately, transformative research.

In 2017, the Health Resources and Services Administration MCHB established a cooperative agreement to create a national research network, the Children and Youth with Special Health Care Needs National Research Network (CYSHCNNet) (<http://www.cyshcnet.org>). CYSHCNNet is an interdisciplinary, multisite, collaborative network to lead, promote, and coordinate national research activities to inform a high-quality system of care for CYSHCN. The network’s initial charge was to create a research agenda reflective of multistakeholder priorities. This agenda could help (1) prioritize the most important health system questions facing stakeholders, (2) define research goals for issues of greatest importance, and (3) provide a blueprint for research that CYSHCN investigators and other stakeholders can follow.

As an initial step in creating this agenda, we undertook the mixed-methods RAND/UCLA Appropriateness Method (RAM),¹⁷ a rigorous modified Delphi process, to integrate literature review and expert

opinion. RAM has previously been used to develop quality measures and design clinical models for CYSHCN.¹⁸⁻²¹ Our objective was to synthesize the opinions of a national multidisciplinary group of CYSHCN experts, including family caregivers, to prioritize research topics facing CYSHCN. With this study, we take a foundational step toward developing a national research agenda for CYSHCN systems of care.

METHODS

RAM Overview

We adapted RAM to set priorities for a CYSHCN research agenda (Fig 1). We used the following terms and processes. First, we developed a set of research topics from responses to an open-ended survey and published literature. Next, stakeholders evaluated every research topic according to 3 different domains, that is, the research topic’s perceived (1) need and urgency, (2) research impact, and (3) family centeredness. During a moderated expert panel, stakeholders gave every research topic 3 ratings (ie, 1 score in each domain). Finally, by using the median ratings of the 3 domains, each research topic was assigned into a rank order reflecting the research topic’s priority from 1 (high) to 4 (low). The Colorado Multiple Institutional Review Board considered this work exempt from human subjects review.

Research Topic Development

Between February 2018 and March 2018, we sought input from professionally, demographically, and geographically diverse stakeholders. Members of stakeholder organizations identified by the CYSHCNNet Executive Committee (Supplemental Information) were

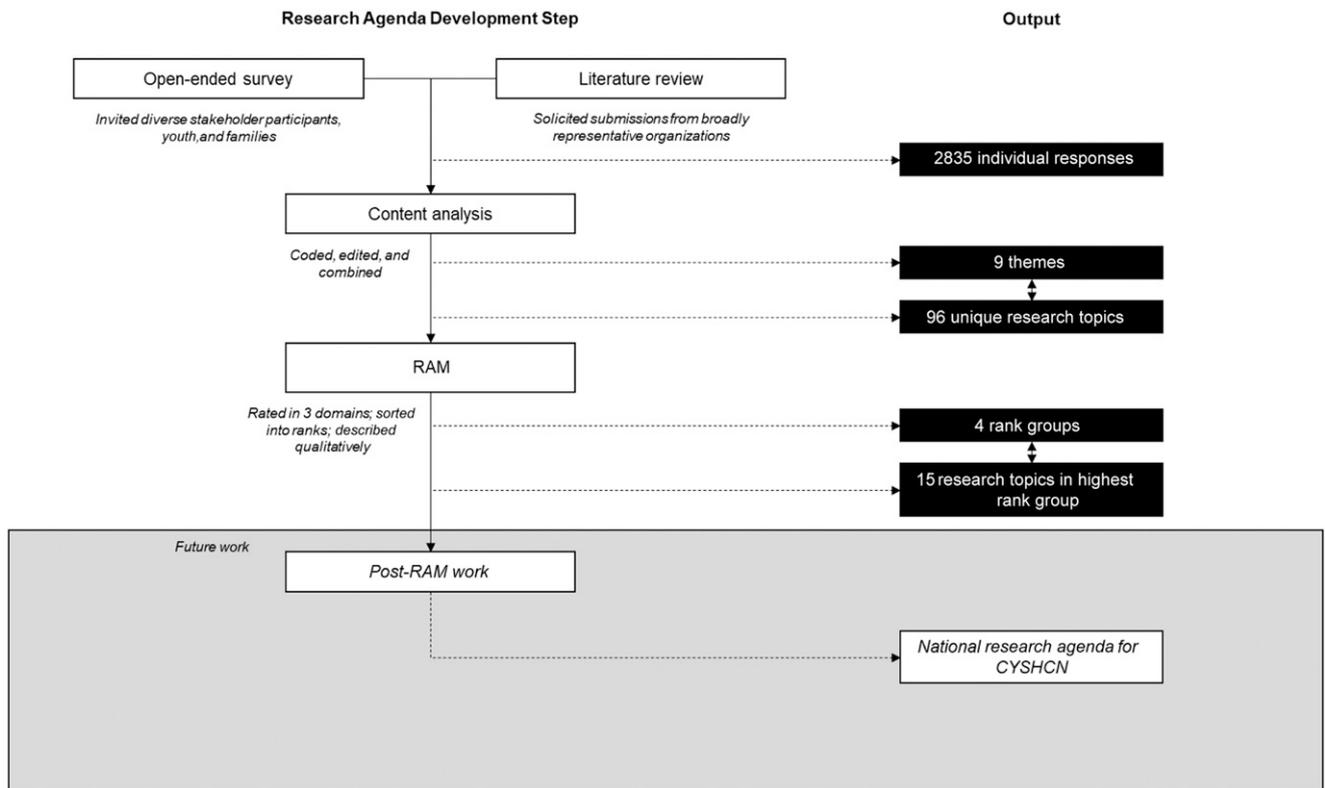


FIGURE 1

Overview of appropriateness method to establish national research agenda priorities for CYSHCN. Research topics were generated from synthesized and aggregated open-ended survey responses, following an ongoing, consensus-seeking approach. Conceptually similar research topics were sorted into themes. Themes emerged from a content analysis of survey responses. Each research topic was rated using 3 rating domains, corresponding to perceived (1) need and urgency, (2) research impact, and (3) family centeredness. Domains were rated by using 9-point Likert scales: 1 to 3 (low) was used when a research topic represented a domain to a low degree; 4 to 6 was used when a research topic represented an uncertain domain; and 7 to 9 (high) was used when a research topic represented a domain to a high degree. To convey relative prioritization, each research topic was assigned a rank order: rank 1: median of ≥ 7 in all 3 domains and > 7 in at least 2 of 3 domains; rank 2: median of ≥ 7 in all 3 domains and > 7 in 1 of 3 domains; rank 3: median < 7 in 1 or 2 of 3 domains, or median = 7 for all domains; and rank 4: median < 7 in all 3 domains or any disagreement.

invited to complete an open-ended electronic questionnaire (Qualtrics, Provo, UT) developed and pilot tested with CYSHCN family leaders. In the questionnaire, respondents identified “one or two things that they would most like to see improved in the approach to health for CYSHCN,” and “what research with or about CYSHCN they would like to see in a national research agenda.” Participants had 2 weeks to reply, after which nonresponders received up to 2 reminders at 1-week intervals. Although an individual could be a member of > 1 stakeholder organization, they were asked to complete the survey only once.

Surveys from 269 respondents yielded 2835 responses, which 3

team members coded, edited, and combined into 86 unique research topics through an iterative, consensus-seeking approach. We used conventional inductive content analysis²² to identify key themes emerging during synthesis and aggregation of the CYSHCN research topics. Study team members reviewed the final list of research topics and themes to ensure representation of all submitted ideas.

Although a systematic literature review was not feasible because of the breadth of the topic of health systems for CYSHCN, stakeholder organizations were asked to submit articles perceived to guide future CYSHCN research directions. The research team updated the final list of

research topics on the basis of a review of the submitted articles.

RAM Panelists

Members of the CYSHCN Executive, Steering, and Advisory Committees (each of which included family members of CYSHCN), the network’s MCHB Division of Research project officers, and members of the MCHB services branch for CYSHCN were invited to participate in the RAM expert panel. The personal and professional characteristics of the 24 RAM panelists are summarized in Table 1. Three RAM participants were coauthors (D.Z.K., K.K., and C.G.H.) to ensure that the interpretation and presentation of the results was

consistent and balanced from clinical, research, and family participant perspectives. Panelists received rating materials and a written summary of the research topic development that included the qualitative analysis of survey responses and the articles submitted by stakeholders.

Appropriateness Ratings

During 2 rounds, in March 2018 and April 2018, panelists rated the degree to which each research topic represented 3 domains: need and urgency, research impact, and family centeredness. The need and urgency domain was defined as the influence the research topic had on the health

and well-being of CYSHCN, the severity of consequences (eg, number of CYSHCN affected by the issue, mortality risk, severity of symptoms, or functional loss), and the presence of critical gaps in knowledge to solve the problem. Research impact was defined as the ability of the knowledge gained from this research topic to translate to meaningful progress in 5 to 10 years and the potential to lead to improvements in health status, quality of care, public health, or policy over the longer-term. Family centeredness was defined as how well the research topic reflected the experiences and priorities of most CYSHCN and their families.

Using a 9-point Likert scale, panelists gave each research topic 3 ratings (1 rating in each of the 3 domains). A rating of 1 to 3 indicated low representation, a rating of 4 to 6 indicated uncertain representation, and a rating of 7 to 9 indicated high representation of a domain.

Consistent with RAM convention, disagreement was defined if one-third or more of panelists rated an item in the low range (ie, 1–3), whereas one-third or more of panelists rated the same item in the high range (ie, 7–9). Panelists could include comments to improve clarity of each research topic and to suggest additional research topics.

CYSHCN Expert Stakeholder Panel

During round 1, panelists completed the ratings electronically over 2 weeks, with 2 weekly reminders sent to nonresponders. During round 2, panelists participated in an in-person 2-day meeting to discuss the round 1 ratings. Panelists received a copy of their ratings and de-identified summary tables with group distributions for each research topic. Meeting moderators (R.J.C. and C.S.) did not rate topics. The facilitated group discussion goal was to ensure panelists had a consistent understanding of terms and concepts. The aim was to identify legitimate areas of disagreement and not to build rating consensus.¹⁷ After adjustments to rating materials (eg, wording changes for clarity) panelists rated the research topics.

After round 2, we summarized rating distributions, areas of agreement and disagreement, and frequencies of each rating for research topics. Anticipating that the ratings would be skewed toward higher numbers, we ranked research topics into groups to convey their relative prioritization. The following criteria were used to define each rank group.

TABLE 1 RAM Participant Characteristics

Characteristics	RAM Research Topic	RAM Expert
	Development Survey (N = 269)	Panel (N = 24)
	n (%)	n (%)
Female sex	189 (70)	15 (63)
Age, y		
18–44	89 (33)	6 (25)
45–54	67 (25)	6 (25)
55+	88 (32)	12 (50)
Not answered	25 (9)	0
States represented		
No. unique states represented by participants	41 ^a	15
Race and/or ethnicity		
White	214 (80)	16 (67)
African American	7 (3)	0
American Indian or Alaskan native	2 (1)	0
Asian American	13 (5)	4 (17)
Native Hawaiian or Pacific Islander	1 (0)	0
Hispanic	6 (2)	2 (8)
Multiracial or other	13 (5)	2 (8)
Not answered	13 (5)	0
Relationship to CYSHCN ^b		
CYSHCN	3 (1)	0
Parent or family member	61 (23)	6 (25)
Advocate, child or family	45 (17)	5 (21)
Health care professional	197 (73)	22 (92)
Health care professional role ^b		
Primary care	76 (28)	9 (38)
Specialty care	71 (26)	5 (21)
Behavioral health or therapist	25 (10)	2 (8)
Administrator	20 (7)	4 (16)
Policy	16 (6)	8 (33)
Researcher	40 (15)	17 (71)
Other ^c	43 (16)	8 (33)

^a All but 9 states (Alaska, Arkansas, Connecticut, Hawaii, Idaho, Maine, Nebraska, Nevada, and West Virginia) had at least 1 respondent.

^b Self-identified; respondents were allowed to pick as many as applied.

^c Roles identified in "other" included funder, public health professional, community-based services, and advocates.

- Rank 1: topics had a median in the high range (≥ 7) for all 3 domains (need and urgency, research impact, and family centeredness) and a median of >7 in at least 2 of 3 domains;
- Rank 2: median of ≥ 7 in all 3 domains and >7 in 1 of 3 domains;
- Rank 3: median < 7 in 1 or 2 of 3 domains, or median = 7 for all domains; and
- Rank 4: median < 7 in all 3 domains or any disagreement.

Finally, to determine if family stakeholder priorities were distinct from those obtained through the RAM process, we reanalyzed the results from the 8 family caregivers separately. Differences in the highest-ranked research topics between family caregivers and the whole group were summarized.

RESULTS

Research Topic Development

Survey respondents ($N = 269$) reflected multidisciplinary stakeholders from across the United States, with at least 1 respondent from 41 of 50 states (Table 1). Nearly one-quarter (24%) were CYSHCN or family members. Approximately three-quarters (73%) self-identified as health professionals, including primary (28%) and specialty (26%) care providers, behavioral health providers and therapists (10%), administrators and policy experts (13%), and researchers (15%). Because reliable denominators were unavailable for surveyed groups and respondents could identify with multiple groups, response rates were not calculated. Moreover, our goal was to achieve thematic saturation and a broad list of candidate research topics from stakeholders rather than to achieve a high response rate.

As described previously, surveys generated 2835 individual free-text

responses, which the research team synthesized and combined into 86 research topics. Nine themes emerged by consensus discussion: (1) access to services and supports (health system and community); (2) financing, payment models, and insurance; (3) caregiving at home and caregiving experience; (4) family health; (5) child health, vulnerability, and complexity; (6) care planning and coordination; (7) medical home and comprehensive care models; (8) technology and care; and (9) CYSHCN research methods innovations. Themes and representative text submissions are summarized in Table 2. Although research topics were sorted into a single theme, they did reveal overlap, with some research topics plausibly belonging to multiple themes.

RAM Expert Panel and Research Topic Ratings

After round 1, 24 of 86 research topics met rank 1 or rank 2 criteria. Experts proposed 10 additional research topics during round 1, leading to 96 total research topics. During round 2, as they reranked topics, panelists discussed tensions around breadth of research topic scope from 3 perspectives: (1) CYSHCN versus children and youth with medical complexity, (2) applicability to larger versus smaller populations, and (3) children versus youth versus adults. Guidance from MCHB team members was to focus on broadly applicable topics. Panelists were therefore encouraged to rate need and urgency lower if a research topic's scope was felt to be narrow.

The results reveal the varying degrees of each research topic's perceived urgency, impact, and family centeredness. Overall, median ratings for the family centeredness domain were >7 for the largest number of research topics (47 of 96 topics, Table 3). The domain research impact

had the lowest number of topics with high ratings, with only 10 of the 96 having median ratings >7 . Although research topics focused on financing were most often rated as having high impact, they were simultaneously the least family centered. No research topics were rated by experts as low (ie, no medians were ≤ 3) in either need and urgency, research impact, or family centeredness.

The rankings of research topics after round 2 are summarized in Table 4. The rank 1 group comprised 15 research topics emphasizing social determinants of health (SDH), family caregiving, refining clinical models, value, and youth-adult transitions. Although research topics related to these concepts were present across all ranks, the rank 1 topics highlight specific priorities within each. For example, SDH topics in rank 1 centered on research to effectively integrate SDH screening into care, understand the unique influence of rurality on CYSHCN, and develop innovative technology to reduce inequities.

Rank 1 caregiving topics were centered on families' experience of caring for CYSHCN at home. These topics centered on research to optimize care delivered by families to CYSHCN at home, use a whole-family perspective, and examine caregiving by using a strength-based approach (eg, understanding and improving family resilience as a means to achieving CYSHCN and family health). Rank 1 clinical-model refinement topics emphasized research to integrate systems, uncover key elements of effective care models, and divide "labor" (routine care and coordination tasks) efficiently among team members, including families. Value-oriented rank 1 research topics centered on research used to determine what value means to different stakeholders and then to focus on return on investment in

TABLE 2 Summarized Results From Research Topic Development

Description of Research Topics Within Each Theme	Representative Respondent Perceptions
Caregiving at home and caregiving experience Home environment as a health determinant; interventions to support caregiving at home; best practices for caregiver education and peer-to-peer support	“Medical professionals need to be aware of the home environment, what interventions are realistic in a day-to-day environment, that will let the child live as much like a typically developing child as possible...”
Technology and care Technologies to complement caregiving and communication; dissemination or implementation of technology	“Real-time symptom monitoring allowing just-in-time adjustments (like asthma meds in response to peak flows)...”
Access to services and supports (health system and community) Workforce capacity and expertise; CYSHCN, health care, and community links; durable equipment, education, and dental care	“Having more access to behavioral services helps to keep children in the community rather than having to utilize inpatient [care].”
Financing, payment models, and insurance Payment model influence on quality, access, and outcomes; compensation for families, providers, and community services	“...Funding can be used for what the family determines is most needed, even if it is for social determinants of health, housing, food, transportation, childcare...”
Medical home and comprehensive care models Key ingredients of effective models; family partnership, team efficiency, provider retention, and culturally sensitive care	“In model practices, who provides care coordination, what do they do (especially what do they do that [is] successful), what are key qualities and qualifications for this position?”
Care planning and coordination Shared decision-making and goal setting; generalizable CYSHCN health screening and comanagement approaches	“Improved family engagement through mutually defined goals (shared decision-making) that are family centered...”
CYSHCN health, vulnerability, and complexity Interventions to reduce consequences of social determinants, rural environments, mental health, and extreme complexity; health outcomes meaningful to families	“...Need research in the area of CYSHCN living in rural locations; how to provide services and supports, what is available now, etc.”
Family health Interventions to promote family physical and mental health, resilience, and self-care; health system influence on family caregiver health	“What are the caregiver benefits when they receive appropriate care support versus parents who do not receive same level of support—ability to work outside the home, caregiver stressors, and health and well-being?”
Research methods innovations Rare disease cohorts; CYSHCN as research team members; marketing findings to research consumers	“...Increased participation in research by youth/young adults with chronic health conditions. What is important to them, what barriers do they experience, what are their suggestions to improve the system?”

programs, personnel, and state-based systems. Finally, the rank 1 youth-adult transition topics emphasized interventions to effectively plan for transition and to ensure ongoing insurance and community supports into adulthood.

As illustrated in Table 5, rank 2 research topics had lower need and urgency ratings than rank 1 topics. Rank 3 research topics had lower research impact and family centeredness ratings than rank 2 topics. Rank 4 research topics (ie, those with medians <7 for all domains) were more technology focused (eg, family use of social media and application- or cloud-based care plans), were more provider centered (eg, workforce and compensation), or were more

related to research methods (eg, standardizing CYSHCN subpopulations). Complete rating details are available in the Supplemental Information.

Notably, the RAM ratings obtained from the complete panel were consistent with the ratings generated by only the family caregivers on the panel. In particular, both analyses placed 10 of the same research topics in rank 1. Ranks 1 and 2, when combined, were nearly identical in both analyses. For example, none of the rank 1 topics from the complete analysis were ranked below rank 2 in the family caregiver analysis. Only 1 rank 1 research topic from the family caregiver analysis was below rank 2 in the complete analysis (“How do delays from insurance denials affect

CYSHCN health, and how can they be minimized?”).

DISCUSSION

We adapted RAM with a professionally broad and nationally diverse group of CYSHCN experts, including family caregivers, to begin developing a national research agenda for CYSHCN systems of care. Our process resulted in a taxonomy of CYSHCN research topics depicting their varying levels of stakeholder-perceived need and urgency, research impact, and family centeredness. The highest-ranked research topics were focused on SDH, family resilience, value, and youth-adult transitions. These findings simultaneously reinforce current CYSHCN research trends and point toward important

TABLE 3 RAM Round 2 Result Summary

Theme	No. Topics	Research Topics With Median Rating >7		
		Need and Urgency	Research Impact	Family Centered
Caregiving at home and caregiving experience	11	1	0	7
Technology and care	11	1	1	5
Access to services and supports (health system and community)	14	1	0	8
Financing, payment models, and insurance	12	3	4	5
Medical home and comprehensive care models	10	2	3	5
Care planning and coordination	7	3	2	6
Child health, vulnerability, and complexity	15	2	0	6
Family caregiver health	8	1	0	5
CYSHCN research methods innovations	8	0	0	0
Total	96	14	10	47

Research topics were generated from synthesized and aggregated open-ended survey responses, following an ongoing, consensus-seeking approach. Conceptually similar research topics were sorted into themes. Each research topic was rated using three rating domains, corresponding to perceived (1) need and urgency, (2) research impact, and (3) family centeredness. Each domain was rated by using a 9-point Likert scale: 1 to 3 (low) was used when a research topic represented a rating domain to a low degree; 4 to 6 was used when a research topic represented an uncertain rating domain; and 7 to 9 (high) was used when a research topic represented a domain to a high degree.

new directions that support achieving high-functioning systems of care.

Rank 1 topics reveal how our results could promote CYSHCN research advances. For example, directly supporting CYSHCN caregiving at home emerged as a top research priority. From topic development to discussion during RAM, our process framed caregiver challenges in terms of resilience and adaptability, providing a more positive orientation than studies that are focused on caregiving stresses and burdens.²³⁻³⁰ This frameshift also aligns with recent research suggesting that strategies to support family caregivers may improve CYSHCN health. For example, emerging data support the theory that child and caregiver health are tightly interlinked³¹⁻³³ and that increasing resilience may mitigate negative effects of toxic stress.³⁴⁻³⁶ Resilience in childhood disability appears to be dynamic,³⁷ and panelists described resilience as a natural intervention target for future work. The latter echoes recent calls to apply real-time resilience measurement into routine care^{5,34,38} and is consistent with the American Academy of Pediatrics (AAP) clinical report on psychosocial factors faced by CYSHCN and families.³⁸

At present, effective interventions to support family caregiving are limited,^{39,40} and how best to achieve the rank 1 caregiving priorities across the vast array of CYSHCN conditions and family structures remains a key unknown. Promising approaches that emphasize family needs and strengths, build family capacity, and promote sustainable goal attainment have direct relevance to CYSHCN families.⁴¹ For example, the Family System-Illness Model⁴² is 1 framework for increasing family resilience in clinical settings for children with chronic illness. Strengths of the model are its linking of biological and psychosocial demands of chronic illness and its accounting for variation in patterns of illness onset, course, outcome, functional loss, and uncertainty about illness trajectory. As effective CYSHCN family resilience interventions materialize, adaptation to the different circumstances experienced by CYSHCN could lead to broadly generalizable and potentially transformative strategies.

Rank 1 research topics also extend current value-oriented CYSHCN research, such as return on investment in payments, staffing, and interventions.⁴³⁻⁴⁷ Although value-oriented research could have major

implications for financing and organization of systems of care for CYSHCN, the area remains poorly understood.⁴³ Consistent with our findings, recent research reveals the central importance of defining value for CYSHCN care through incorporation of an array of stakeholder perspectives.⁴⁵ Closing key value-oriented knowledge gaps could begin with mixed-methods studies to establish stakeholder-centered value measures and applying these with different stakeholders. Comparative effectiveness study designs could be used to evaluate the effects of various alternative payment models on CYSHCN health outcomes.

Rank 1 and rank 2 topics also highlighted integration across care sectors (home, school, community, and health care) as a CYSHCN research agenda priority. Particularly notable were numerous topics on behavioral health and the transition from childhood to adult systems. RAM participants rated more than half of all topics in these 2 areas as family centered, likely reflecting the central role of families in CYSHCN care and in building robust systems for their care. Conversely, relatively few topics in these areas were rated high in research impact. Although the

TABLE 4 Descriptions of CYSHCN Research Topics in Each Rank After RAM

Theme	Research Topic Descriptions by RAM Rank ^a		
	Rank 1 (Highest)	Rank 2	Rank 3 (Uncertainty in At Least 1 Rating Domain)
Caregiving at home and caregiving experience	Optimizing care at home	Home physical environment Parent activation Family-identified education needs Behavioral health crises Training diverse parent populations	Peer support credentials and professional standards Home nurse core competencies Independent living achievement Peer mentor education Group care models
Technology and care	Telemedicine implementation Technology to reduce disparities	Real-time symptom monitoring and treatment Technology to train parents	Parent contributing to health record documentation Data sharing across platforms and agencies
Access to services and supports (health systems and communities)	Insurance and community support through youth-adult transition	Behavioral health access Peer-peer coaching for self-management Complex care and specialty care access	Medical and nonmedical service collaboration Documentation to minimize denials Long-term supports and services across states Durable medical equipment access School integration Oral health provision Adult CYSHCN provider workforce size and competence
Financing, payment models, and insurance	Payment models that improve quality Care model return on investment Value outcomes meaningful to different stakeholders	Paying families to deliver care Paid family medical leave Payment models across life span Medicaid-managed care impact	Provider compensation Insurance delays and denials Payment models supporting youth-adult transition Child health impact from health services to parents
Medical home and comprehensive care models	Effectiveness of integrated systems Key ingredients of effective models Palliative care incorporation into routine care	Parent advisory group best practices Conducting culturally sensitive care	Staff/patient ratios Family as professional team members
Care planning and coordination	Youth-adult transition planning models Dividing labor and comanagement	Shared decision-making and goal setting Family engagement in care planning	
Child health, vulnerability, and complexity	SDH ^b SDH ^b Rurality's influence on health and care	Professional care coordinators Health outcomes meaningful to families Identifying and reducing disparities Child and family mental health screening	CYSHCN peer relationships Avoidable morbidity and mortality with youth-adult transition Youth-adult transition and mental health exacerbations CYSHCN tiering systems Family characteristics predicting acute care use Unknown diagnoses Developmental consequences that have an adult onset
Family health	Family resilience	Family health influence on child health Support for family self-care and emotional needs Family's role in value-based decisions	Parent mental health and addiction Parent stress during youth-adult transition Shared care plans' influence on family health Family health as metric of CYSHCN system effectiveness

Rank 1 (highest): research topics had a median in the high range (≥ 7) for all 3 domains and a median of >7 in at least 2 of 3 domains; rank 2: research topics had a median of ≥ 7 for all 3 domains and >7 in 1 of 3 domains; rank 3: research topics had a median <7 for 1 or 2 of 3 domains, or median = 7 for all domains; rank 4 (not shown): research topics had a median <7 for all 3 domains or any disagreement. See the Supplemental Information for complete research topics and ratings.

^a Individual research topics were ranked on the basis of RAM results. Each topic was rated in 3 domains: need and urgency, research impact, and family centeredness.

^b Two SDH research topics were combined into a single topic.

significance of this finding is unclear, it may represent concerns about being able to translate research findings from these topics to meaningful impact in the short-term.

Our explicit motivation to develop research priorities and relative rankings distinguishes these results from related efforts. For example, the MCHB 6 Core Outcomes¹⁵ and the AMCHP Standards for Systems of Care¹⁶ provide health system metrics and were developed with multidisciplinary and family input; however, neither was designed to reflect specific research priorities. Although some aspects of AMCHP and MCHB frameworks may benefit from research, others may require different initiatives (eg, policy or advocacy). As an example of this distinction, the RAM research topics in the theme of access to services and supports have substantial overlap with AMCHP and MCHB frameworks (as well as high family-centered ratings); however, none rated high in research impact. One potential interpretation of this finding is that although these topics have substantial relevance to CYSHCN, panelists were skeptical that conducting research was the best way to address the underlying challenges.

Although panelists felt that lower-ranked research topics had some

uncertainty in need and urgency, research impact, and/or family centeredness, this does not imply that those topics are unimportant. RAM does not readily facilitate interpreting why panelists gave specific ratings and is not intended to force consensus. Technology-centered research topics, despite being a focus area in the AMCHP Standards for Systems of Care, were consistently rated low or uncertain. On the basis of conversations during the RAM meeting, we speculate this was due to perceptions that technologies face important feasibility and generalizability barriers. Panelists also expressed that although technology might facilitate solutions, it might not inherently solve problems. Provider-centered research topics (eg, workforce, compensation, satisfaction, and efficiency) also tended to be lower ranked, presumably because they are less family centered, knowledge gaps are less clear, and the topics are seen as less urgent.

This work has limitations. The volume and content developed through this process precludes a comprehensive discussion of each research topic. Throughout RAM, it was challenging to be as inclusive as possible while simultaneously developing a well-defined, generalizable, and actionable set of high-priority research topics. Similarly, although we integrated

solicited literature from stakeholder organizations into RAM, we did not conduct a formal systematic literature review. Whether a systematic literature review would have uncovered additional topics or influenced the panelist ratings is not known. Because we anticipated ratings would be skewed toward the high end of the scale, we established rank priority cutoffs, which might be overly sensitive. RAM also relies on experts having the necessary knowledge to make valid judgements, which we could not verify. Despite in-person discussion, experts may have had distinct CYSHCN contexts in mind, which could differentially influence some ratings. For example, thinking about children with autism versus asthma might affect ratings about caregiving research topics. Additionally, our RAM participants were less demographically diverse than we would have liked. We also recognize that priorities will change over time on the basis of new knowledge, practice patterns, payment models, and policies.

To translate RAM results into an actionable research agenda for CYSHCN health systems, additional steps are needed. First, comprehensive reviews should be used to synthesize current knowledge and distinguish key gaps for each research topic. Second, researchers should determine

TABLE 5 Average Ratings of Research Impact, Need and Urgency, and Family Centeredness for Research Topics Within Each Rank

	Summary of Research Topics	Rating Domains		
		Need and Urgency	Research Impact	Family Centeredness
Rank 1	Social determinants, family caregiving, clinical-model refinement, youth-adult transitions, value	7.9	7.5	8.0
Rank 2	Meaningful outcomes, behavioral health, home environment, peer-peer support, family engagement, family economics	7.0	7.1	7.9
Rank 3	Linking family and child health, school and peers, dental health, youth-adult transitions, professional competencies, clinical staffing	6.8	6.4	7.2
Rank 4	Technology, research methods, provider-centered concepts	5.9	5.7	5.7

Each research topic was rated in 3 domains (need and urgency, research impact, and family centeredness) by using a 9-point Likert scale: 1 to 3 (low) was used when a research topic represented a domain to a low degree; 4 to 6 was used when a research topic represented an uncertain domain; and 7 to 9 (high) was used when a research topic represented a domain to a high degree. Shown are the average RAM round 2 ratings in each rank group. Rank 2 had lower need and urgency ratings than rank 1. Rank 3 had lower research impact and family centeredness ratings than rank 2. Average ratings of topics in ranks 3 and 4 were primarily in the uncertain range (4–6).

key barriers to progress. Do challenges result from limited knowledge about a topic or from implementation difficulties? Third, specific research initiatives that will close knowledge gaps and overcome barriers should be articulated. For some well-studied research topics, work may need to be focused on dissemination and implementation science,⁴⁸ whereas for more emergent topics, basic discovery⁴⁹ may be most needed.

Despite these limitations, this work uncovers research topics with high perceived need, research impact, and family centeredness and may offer a pragmatic guide for families, researchers, policymakers, and health system leaders interested in improving CYSHCN outcomes. The consistency of our results, whether evaluated across the complete stakeholder group or among family stakeholders alone,

suggests that they accurately reflect priorities of CYSHCN families. The synthesis of opinions of a national multidisciplinary group of CYSHCN experts, including family caregivers, represents a fundamental first step toward the development of a national research agenda to improve CYSHCN health systems. The work prioritizes the most important systems-level questions, facilitates setting research goals related to these questions, and contributes to a blueprint to guide investigators to work cohesively toward achieving an idealized vision of comprehensive coordinated systems for CYSHCN.

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ABBREVIATIONS

AAP: American Academy of Pediatrics
AMCHP: Association of Maternal and Child Health Programs
CYSHCN: children and youth with special health care needs
CYSHCNNet: Children and Youth with Special Health Care Needs National Research Network
MCHB: Maternal and Child Health Bureau
RAM: RAND/UCLA Appropriateness Method
SDH: social determinants of health

Dr Collier conceptualized and designed the study, conducted the primary data analysis and interpretation, and drafted the initial manuscript; Drs Berry, Chung, and Stille conceptualized the study, participated in data collection, analysis, and interpretation of data, and revised the manuscript; Drs Kuhlthau, Kuo, and Perrin and Ms Hoover critically interpreted data analyses and critically revised the manuscript; Dr Shelton and Ms Werner coordinated and supervised data collection and data management and critically reviewed the manuscript; Ms Thompson and Ms Garrity assisted with data collection and critically reviewed the manuscript; and all authors approved the final manuscript as submitted and agree to be accountable for all aspects of the work.

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Health System Research Priorities for Children and Youth With Special Health Care Needs

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