Meeting Proceedings:

Creating a Patient Centered CER Roadmap for chILD

February 27–28, 2026

Meeting Information

Project Title	**Building Capacity for Patient-Centered CER in Pediatric Rare Lung Disease**
Funder	Patient-Centered Outcomes Research Institute (PCORI)
Award Type	Engagement Award: Capacity Building Award for Small Organizations
Award No.	EASO-42389
Duration	Two Years (Year 1 complete; Year 2 commencing June 2026)
Meeting Dates	February 27–28, 2026
Venue	Magnolia Hotel, 818 17th Street, Denver, CO 80202
Next Meeting	March 6–7, 2027 (Final Year 2 Planning Meeting, Denver)

**The project employs the SEED (Stakeholder Engagement and quEstion Development and Prioritization) methodology. The SEED Method was funded through a Patient-Centered Outcomes Research Institute (PCORI) Award (#1310-07664) to Emily Zimmerman (Principal Investigator), Center on Society and Health at Virginia Commonwealth University.*

Executive Summary

The chILD Foundation convened its two-day Denver Working Meeting on February 27–28, 2026, bringing together families, clinicians, researchers, and stakeholder partners to advance a patient-centered comparative effectiveness research (CER) agenda for children's interstitial and diffuse lung disease (chILD). Approximately 50 participants attended in person at the Magnolia Hotel in Denver, with 25+ additional participants joining via Zoom.

The meeting featured nationally recognized CER experts, deeply moving family narratives from across the chILD spectrum, and intensive working group sessions designed to translate one year of family-led discovery into actionable research priorities.

The meeting also marked a significant milestone for the Foundation: the formal introduction of Holly James as the new Executive Director. Ms. James, who joined the organization one month before the meeting, framed the weekend as "two days of learning, collaboration, and planning" toward a bold future for the Foundation and its research community.

Holly James, Executive Director, chILD Foundation

DAY 1 — FRIDAY, FEBRUARY 27, 2026

Session: What Is Patient-Centered Comparative Effectiveness Research?

Moderator: Dr. Alicia Casey, Boston Children's Hospital

Presentation 1: What Is CER for Rare Disease?

Speaker: Mathew J. Edick, PhD — Director, Center for Precision Public Health, Michigan Public Health Institute

Motivated by his experience as the father of a child with a rare disease, Dr. Edick emphasized the gap between research priorities and family needs. He highlighted PCORI as a key supporter for rare disease communities through research funding and engagement infrastructure that helps patient organizations build research readiness.

Dr. Edick opened with an accessible introduction to comparative effectiveness research, with a particular focus on the rare-disease context. He defined CER as research comparing two or more real-world treatments or approaches currently in use, oriented toward the questions patients face in their clinical lives. He distinguished CER from basic science research and from regulatory clinical trials, emphasizing its relevance to the decisions families and clinicians make every day in the absence of adequate evidence.

He explained the role of PCORI Ambassadors and how organizations such as the chILD Foundation can connect patient communities with research opportunities. He noted that PCORI's Rare Disease Advisory Panel has influenced research priorities and that the rare disease community has a meaningful impact on the CER agenda.

Presentation 2: PCORI Methodology Standards, Phased Awards, and Pragmatic Studies

Speaker: Nora McGhee, PhD — Associate Director, Clinical Comparative Effectiveness Research, PCORI

Dr. McGhee summarized PCORI's funding, standards, and engagement, citing examples such as surgical versus medication comparisons, drug studies, telehealth, and care coordination, illustrating that CER extends beyond drug trials. She stressed the importance of openness in formulating research questions, focusing on decisions faced by patients and families. Key points included:

- Meaningful patient and stakeholder engagement has been central to PCORI-funded research for over a decade, involving advocacy groups and professional partners like clinicians, health system leaders, payers, researchers, and policymakers.
- With increasing competition for PCORI funding, a strong Letter of Intent (LOI) is essential, addressing the PICOTS framework (Population, Intervention, Comparator, Outcome, Timing, Setting), study design, engagement plan, investigator qualifications, and budget.
- Funding options include PLACER Awards—large-scale CER trials with an initial feasibility phase; Broad Pragmatic Studies (BPS) awards—up to $12 million across three levels; and topic-specific calls.
- Rare disease research has been a focus for years, with two dedicated annual announcements. Many rare disease studies are funded through broader calls.

PCORI's methodology standards set the minimum criteria for rigorous, patient-centered research and should be reviewed early in proposal development. Dr. McGhee urged participants to keep an open mind when developing research questions, emphasizing critical decisions faced by patients and families.

Presentation 3: Integrating Data Networks — PCORnet and PEDSnet

Speaker: Sara Deakyne Davies — Chief Research Informatics Officer, Children's Hospital Colorado

Sara Deakyne Davies introduced participants to large-scale federated research data networks and their importance to rare disease CER. As a leader in PEDSnet and PCORnet and a member of national data governance bodies, she discussed their capabilities and limitations.

Federated data networks allow researchers to query and share Electronic Health Record (EHR) data across institutions without centralizing sensitive patient information, enabling feasibility studies, patient identification, and long-term outcome tracking. For rare diseases like chILD, where no single institution has enough data for powered studies, this approach is vital.

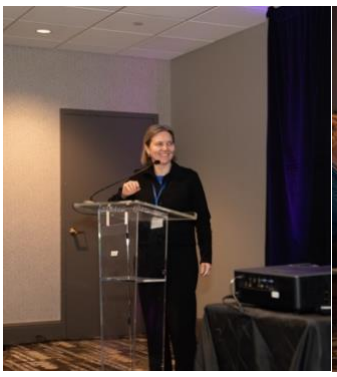
Participants noted a challenge: referral reasons in EHRs are often too broad to identify chILD patients, necessitating innovative patient identification methods. She suggested outcome-based identification—tracking patients with an ILD diagnosis—as more reliable than referral reasons.

Presentation 4: Patient-Centered CER in Pediatric IBD — A Success Story

Speaker: Michael D. Kappelman, MD, MPH — Professor of Pediatrics and Epidemiology, UNC Chapel Hill

Dr. Kappelman provided the chILD community with a concrete roadmap from his 20+ years of experience building patient-centered CER infrastructure in pediatric IBD. He described ImproveCareNow, a learning health system in which patients, families, and clinicians co-design quality improvement and research, and IBD Partners, a Patient-Powered Research Network supporting direct-to-patient outcomes research.

His recent PCORI award compares emerging IBD medications in children unresponsive to standard therapies across 30 U.S. sites using PCORnet. His key message was that CER's value lies not just in individual studies but in the infrastructure they require. Training families, compensating patient partners, developing multi-site registries, and building data network relationships are long-term investments that underpin high-impact research.



Session: Where We Are Now — Building Capacity for CER in chILD

Presenter: Ann Gettys, Project Lead, chILD Foundation

Ann Gettys summarized the PCORI Capacity Building project, its Year 1 activities, and its connection to the Foundation's broader strategy.

Year 1 Project Structure

The project used the Stakeholder Engagement in Question Development (SEED) methodology from Virginia Commonwealth University, funded by PCORI Award #1310-07664, developed by Emily Zimmerman and Sarah Cook. It is a two-year project with two cycles of family engagement. In Year 1, four groups were formed based on patient age and experience, each meeting monthly over five months:

- Ages 0–2
- Ages 3–12
- Ages 13+: Transition to Adulthood
- Undiagnosed chILD

Each group engaged in structured discussions to identify care barriers, priority outcomes, and research questions grounded in family experience. Ann then described the "heterogeneity paradox" the chILD Foundation faces: while many successful rare disease organizations focus on a single diagnosis, chILD spans more than 100 diagnoses. The Foundation's strategic response is to build infrastructure around shared needs while developing disease-specific working groups that can pursue more focused scientific agendas and attract targeted funding.

The CER Research Roadmap

A central deliverable of the PCORI project is a comprehensive CER Research Roadmap, to be completed at the end of Year 2 and disseminated. Ann described it as both an internal planning tool and an external communication instrument: "When we go to funders, we have something to say 'This is where we're going, and this is what we need.'"

Physician and Researcher Interview Findings

Presenter: Alina Brennan, Research Coordinator, Boston Children's Hospital / PCORI Engagement Award Project Staff

Alina Brennan presented findings from a qualitative study with pediatric pulmonologists and chILD researchers. The study received IRB approval at Boston Children's Hospital, with verbal informed consent from all participants. Ten individuals completed 30–60 minute semi-structured Zoom interviews, recorded (except one), and transcribed. Transcripts and notes were hand-coded, analyzed in NVivo, and reviewed by the team.

Participants included five U.S. clinicians, three U.S. researchers, and two international clinician-researchers. The main research question was: "How can we develop a sustainable, patient-centered CER agenda and infrastructure for chILD?" Four key themes emerged.

Theme 1: Fundamental Challenges in Diagnosing chILD

Physicians cited the "heterogeneity paradox" as a key obstacle. This variability impacts classification (still evolving), the balance between population and precision medicine, and the grouping of diagnoses for research.

Three chILD presentation pathways were identified: (1) severe/NICU cases with early respiratory distress; (2) recurrent subacute illness with multiple hospitalizations; and (3) chronic symptoms in older children, usually with imaging but rarely biopsies. The setting greatly influences the diagnostic process, especially for children born outside chILD centers, leading to a longer diagnostic path.

Theme 2: Treatment Challenges and Physician Resilience

Physicians identified an evidence vacuum as the main challenge in chILD treatment: “There’s no data. Our approach is mostly experience-based, and we’re unsure if it’s helpful.” This leads to prognostic uncertainty and uncertain treatment efficacy.

Treatment burden also poses a challenge. Physicians cited social impacts of oxygen and equipment, treatment fatigue, and complex medication regimens. In response, physicians use strategies like normalizing diagnosis for families, framing research as hopeful, shared decision-making, and consulting multiple chILD specialists.

Theme 3: Research Challenges and Opportunities for Innovation

Physicians cited small sample sizes, disease heterogeneity, and limited access to trial sites as the main barriers to CER in chILD. None of the eight U.S. physicians had led a CER trial despite extensive research experience.

Suggested methodological approaches included treating existing practice variation as a natural experiment, using AI to predict individual treatment response, developing patient-specific organoid models, cross-registry collaboration, and registry-based trial designs for diagnostic feedback.

Theme 4: Infrastructure Needs — Knowledge and Research

Physicians consistently identified infrastructure development as a prerequisite for meaningful CER. Expert knowledge is dangerously concentrated, with only a handful of pathologists nationwide who deeply understand chILD histology, and knowledge of genetic variants exists in the brain of somebody who’s now retired”.

Specific knowledge infrastructure needs included pathology and CT image libraries with expert annotation and AI-enabled pattern recognition; standardized gene panels; and a genetic variant database modeled after the cystic fibrosis database. Research infrastructure needs included informatics tools, biostatistical support, patient recruitment mechanisms, longitudinal follow-up infrastructure, data-sharing agreements, biorepositories, and dedicated research coordinators.

PHYSICIAN INTERVIEW ROADMAP FOR CER DEVELOPMENT

- Infrastructure (including clinical care infrastructure) is a prerequisite for rigorous research.
- Outcome measure development is essential: validated, patient-centered outcomes must exist before meaningful CER can begin.
- Methodological innovation is required: traditional CER designs will not work for heterogeneous, ultra-rare populations.
- Stakeholder engagement is foundational: the collaborative tradition of chILD must be formalized and expanded.
- Equity must be a design principle: barriers to access and research participation must be addressed from the ground up.

Family Topic Group Report-Outs

Following the project overview and physician interview findings, leaders from each of the four Year 1 topic groups presented their findings. Each presentation began with a personal family story, grounding the research priorities in human experience before presenting the aggregate themes and recommendations generated through months of structured family group work.

Ages 0–2 Topic Group

Family Story: Nicole Nefestra

Nicole, a chILD parent, shared what she described as a "textbook" clinical presentation before pivoting to its emotional reality. She described being accused by a nurse of neglect while her infant was failing to thrive; fighting insurance companies for oxygen supplies while managing tube feeds; learning to manage supplemental oxygen at home after only a day of training; and simply not knowing how to leave the house with a medically complex infant.

KEY FINDINGS — AGES 0–2 GROUP

- Trauma is recent and ongoing in this age group. Miscommunication or dismissal by physicians was common.
- Many families leave the hospital without adequate oxygen training or sick-day guidance.
- Minor illnesses and teething can trigger respiratory crises.
- Feeding issues, oxygen management, and administrative tasks pose challenges in early years.
- Mental health support for parents and caregivers is nearly absent.

Topic Group Leader: Jean Schmit-Gill

Jean Schmit-Gill, a high school chemistry teacher and mother of a child with an unknown lung growth disorder, described the 0–2 group as the Foundation's most vulnerable families—those in the diagnostic process, where "everyone is traumatized." Jean noted that the diagnostic journey has not shortened in twenty years: "I am dealing with people on the same journey I traveled 20 years ago. I don't see much difference."

The session introduced several oxygen management tools unfamiliar to many families: pre-filled sealed humidifiers, flow pens, water traps, and swivel connectors. The PAC is developing a standardized equipment supply list for families and providers.

Key Research and Program Priorities Identified by 0–2 Families

- Educate physicians on early signs of chILD in infants for pediatricians and family practitioners.
- Implement standardized diagnostic protocols applicable across care settings.
- Enhance home oxygen education before discharge: hands-on training, clear parameters, troubleshooting guides, 24/7 support.
- Conduct home evaluations before discharge to assess environment and equipment needs.
- Improve home monitoring tools for oxygen saturation and CO2 to ensure safe management.
- Establish peer mentoring for newly diagnosed families.
- Provide mental health support for parents, caregivers, and siblings.

Ages 3–12 Topic Group

Family Story: Tammy Faucheux

Tammy Faucheux shared her daughter's story, diagnosed at five months after a complex journey that began at two weeks of age. After several formula changes, a two-month hospital stay, an initial Heiner syndrome misdiagnosis, and a lung biopsy during a hurricane evacuation with inconclusive results, her daughter was finally diagnosed with chILD. Tammy recalled leaving the appointment in shock: "I can't even ask any questions. I don't even… I can't even think." Discovering a Facebook support group soon after was "life-changing." Now 13, her daughter participates in dance, baseball, and volleyball, and has a 504 plan at school.

KEY FINDINGS — AGES 3–12 GROUP

- For this age group, the biggest needs are not only medical — they are system and life skills.
- Vague provider instructions ("turn it up a little") are a patient safety issue; families need specific parameters.
- Families are learning oxygen management hacks through trial and error, sometimes unsafely.
- School accommodation is a persistent gap: IEP/504 support, staff training, and peer stigma all remain unaddressed for most families.
- Financial burden amplifies every other stressor and limits access to specialist care and research participation.
- The psychosocial strain of this diagnosis affects the entire family system, not only the diagnosed child.

Topic Group Leader and Presenter: Angel Melendez, Respiratory Therapist and PCORI Ambassador

Angel Melendez, a respiratory therapist and PCORI Ambassador who lives with asthma himself, framed the 3–12 age group: "Ages 3 to 12 is where childhood becomes social. School becomes a second home, and health needs have to fit into real life." He dedicated the presentation to the children living this reality, the caregivers who became experts not by choice, and the siblings whose lives are shaped by the same schedules, stresses, and hopes.

Theme 1: Time to Diagnosis and Access to Specialists

Families in this group described months of testing and waiting, often with worsening symptoms and no clear plan. The group's key insight: "Diagnostic delays are not neutral. Delays can mean delayed treatment, and potentially prevent further harm." One family's direct statement: "During the 5 months we waited, my daughter wasn't getting any treatment. I truly believe her lungs suffered permanent damage while waiting." Time to diagnosis is a patient safety issue, not merely an inconvenience.

Theme 2: Education Gaps Across Schools, Providers, DMEs, and Families

This theme produced one of the most impactful moments of the presentation. Angel, himself a respiratory therapist, described being "slapped in the face" by the realization that vague provider instructions like "turn it up a little" are not adequate oxygen guidance for families managing complex home care. Families reported never receiving specific parameters on when to increase flow, when to call, or when to go to the emergency room.

Theme 3: Financial Burden and Psychosocial Strain

The financial burden was the most significant systemic outcome for this group, with hidden costs including specialist travel, supplies, time off work, and lost income. The group noted regional disparities in care, where a family's location largely determines access.

The "isolation vs. socialization" dilemma was a key psychosocial theme: families grappled with balancing protection from respiratory triggers and allowing social and school experiences. Siblings often felt displaced, and parents struggled with guilt over attention imbalance. Parental mental health issues like anxiety and depression were common.

Key Research and Program Priorities Reported by 3–12 Families

- Comprehensive oxygen equipment education toolkit focused on real-world use and troubleshooting, developed from lived family experience.
- Sick-day oxygen algorithm with specific parameters and clinical decision support.
- Provider education to reduce diagnostic delay and improve pathways to specialists.
- School accommodation interventions: IEP and 504 support, staff training on comfort with medical equipment.
- Mental health interventions for children and caregivers, with attention to the full family system including siblings.
- Research awareness systems to ensure families learn about clinical trials through channels accessible to all.

Ages 13+ Topic Group: Transition to Adulthood

Topic Group Leader / Presenter: Cara Favuzza, RN, Pediatric Nurse, Boston Children's Hospital (patient)

Cara Favuzza opened with her own story. She was diagnosed at age 10 with a form of chILD following mycoplasma pneumonia. She spent much of her childhood and adolescence in and out of the hospital, working with multiple care teams and trialing treatment options.

Rather than sending her away from medicine, the experience drew her toward it. She became a pediatric nurse at the same hospital where she was treated, motivated by the desire to be the compassionate presence for other children that she had once needed herself.

KEY FINDINGS — AGES 13+ GROUP

- Most patients experience an abrupt, unstructured handoff from pediatric to adult care.
- Adult pulmonologists rarely have pediatric-onset chILD training; patients educate their new providers.
- Disease education must start early to help patients advocate for themselves in the adult system.
- Mental health in adolescents with chILD is underreported: self-harm and suicidality occur in family settings but are absent from research.
- Long-term outcome data for adults with chILD is nearly absent; a registry tracking patients after transition is urgently needed.
- Loss to care is a research emergency: without tracking, the community cannot address families' urgent needs.

Theme 1: Clinical Transition to Adult Care Programs

Most institutions outside a few centers of excellence lack formalized transition programs. Patients often describe turning 18 as being “pushed out of the pediatric hospital.” Cara recommended starting the transition at age 13. Key components include early disease education and a graduated approach to self-management skills such as prescription refills, appointment scheduling, and insurance calls.

Theme 2: Workforce Training, Self-Advocacy, and Broader Support Needs

Adult pulmonologists often lack familiarity with pediatric-onset chILD. Young adults entering adult care frequently find themselves explaining their condition better than their new physician understands. The group also identified support needs like reproductive counseling. Mental health issues, including self-harm, suicidality, anxiety, and depression, were described as severe and underreported. One participant stated: “We have a lot of severe mental health issues with teens, but we don’t talk about it.”

Theme 3: Loss to Care and Longitudinal Tracking

Once patients age out of pediatric care, the longitudinal data about their health trajectories is essentially lost. A registry specifically designed to follow patients into adulthood was identified as a critical and feasible near-term infrastructure need. Without such a registry, the chILD research community will continue to lack the longitudinal evidence needed to answer the question families most urgently ask: “what does this look like as an adult?”

Key Research and Program Priorities Reported by the 13+ Group

- Design and evaluate a formalized transition program beginning at age 13, including disease self-education and graduated self-management skill-building.
- Workforce training for adult pulmonologists in pediatric-onset chILD.
- Reproductive counseling and family planning resources for young adults with chILD.
- Registry to track long-term outcomes for adults with chILD.
- Mental health support programs specifically designed for adolescents with chronic, rare conditions.
- Peer mentorship programs pairing newly diagnosed adolescents with experienced young adult peers.

Undiagnosed chILD Topic Group

Family Story: Melanie Kirk

Melanie is the mother of a 19-year-old with unknown chILD. Her son's story began before birth—her pregnancy was complicated by hyperemesis, sepsis, and HELLP syndrome, and he was born at 28 weeks, weighing two pounds. After 101 days in the NICU, he went home on oxygen. His oxygen needs increased as he grew, and a lung biopsy at age three revealed some form of interstitial lung disease that didn't fit known criteria. He later received diagnoses including optic nerve hypoplasia, growth hormone failure, and adrenal insufficiency requiring lifelong steroids. Recently, he has had life-threatening adrenal crises at least once a year. Melanie has learned to advocate fiercely; she studied law partly to support her son's care.

Topic Group Leader: Alejandra Creixell

Alejandra is a parent of a teenage son with an undiagnosed form of chILD. She led the Undiagnosed topic group, which included six participants with children ranging from early childhood to their 20s. She highlighted the isolation these families feel—their voices are often unheard even within the broader chILD community. Alejandra described these families as living in ongoing diagnostic limbo—cycling between hope and grief with each test or potential answer, making treatment decisions without guidelines, and facing ER and urgent care providers who dismiss or panic due to a lack of diagnosis.

KEY FINDINGS — UNDIAGNOSED GROUP

- Diagnostic delays for undiagnosed children often span years, during which families undergo misdiagnoses and treatments—including steroids—that can cause lasting harm.
- These families face a unique psychological burden: chronic, unresolved grief and uncertainty, as existing mental health frameworks do not address this experience.
- Emergency tools like medical passports are largely inaccessible because non-chILD provider do not officially recognize "undiagnosed chILD" as a clinical entity and parent concerns are dismissed.
- Blood and tissue samples are re-examined only sporadically, with no systematic triggers or notifications for families about relevant new discoveries.
- Adults who were undiagnosed as children fear being forgotten by the chILD community at transition.
- Multidisciplinary case conferences, modeled on France's RespiRare approach, could lead to diagnostic changes in the undiagnosed group.
- Solving one case creates a ripple effect, opening pathways for others with similar presentations.

Theme 1: Barriers to Diagnosis — The Diagnostic Rollercoaster

Families described their diagnostic journey as a rollercoaster of hope and despair, often seeking answers from multiple specialists over years of uncertainty. The process is costly, invasive, and emotionally draining. Genetic testing is frequently outdated, and the group identified a systemic gap: no mechanisms exist to re-evaluate undiagnosed cases as new discoveries are made.

Theme 2: Chronic PTSD Without Resolution

The group challenged the idea that PTSD requires a specific past event. One parent said: “PTSD has an end. Ours doesn’t end. Every winter is going to bring problems.” Anticipatory grief is constant. The group called for mental health treatments for chronic uncertainty—approaches beyond traditional PTSD models, tailored for ongoing grief.

Theme 3: Treatment Without Evidence — Impossible Choices

Without a diagnosis, families face life-or-death treatment choices without evidence, guidelines, or a clear risk-benefit framework. The group noted many physicians are willing to try experimental treatments, which families appreciate and fear. Symptom-based management of chronic oxygen needs, feeding difficulties, and developmental delays without a diagnosis is a top research priority.

Theme 4: Dismissal and Systemic Siloes

Specialists often blame unexplained symptoms on the child’s quirks rather than underlying systemic issues. Without a diagnosis, subspecialties don’t communicate, leaving families to connect dots across unfamiliar fields.

Theme 5: Emergency Care Failure

The lack of diagnosis codes creates life-threatening emergencies in emergency settings. Without these codes, EMTs and ER staff may dismiss cases or panic. Families have been turned away from community emergency departments. The group’s stance: absence of diagnosis should not mean absence of protocols. Standardized symptom-based treatment algorithms for children with unknown diagnoses are urgently needed for EMTs and ER physicians.

Theme 6: Evolution Through Trauma

Families of undiagnosed children described a necessary transformation. One mother said: “I stopped timidly asking and started demanding. Realizing it was okay to question medical providers changed things.” The caregiver burden is intense: “We keep our kids alive. I just can’t die.” They identified provider education on this trauma as a key need.

Key Research and Program Priorities — Undiagnosed Group

- Recognize undiagnosed chILD as a distinct research category needing dedicated studies, registries, biobanks, and natural history research.
- Develop re-evaluation protocols with clear triggers like new diagnostics, clinical decline, or transition to adult care.
- Establish standards for genetic reanalysis and mechanisms to notify families of new relevant discoveries, include AI and bioinformatics for pattern recognition and data mapping in undiagnosed children.
- Create symptom-based emergency protocols not requiring a diagnostic code and improve communication systems to reduce siloing and coordinate care across specialties for complex cases.
- Develop frameworks to weigh treatment benefits against long-term risks without a diagnosis.
- Study mental health strategies for managing chronic uncertainty, grief, and anticipatory distress

DAY 2 — SATURDAY, FEBRUARY 28, 2026

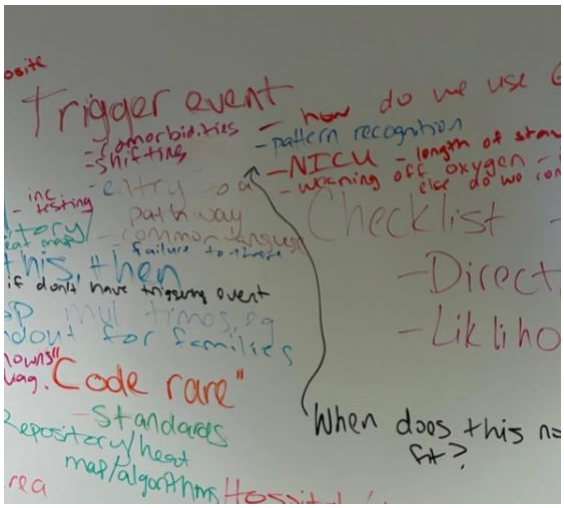
Saturday was designed as a collaborative action day titled: “Paving the Way to Cures for chILD: From Exploration to Action Items.” Participants self-selected into two concurrent session tracks based on expertise and interest. The goal was to identify the most promising, feasible, and impactful CER pathways and begin assembling the people, data, and infrastructure to pursue them. All CER questions discussed in Saturday’s sessions came directly from the Year 1 family topic groups.

Breakout Session 1A: Pathways to Improved Diagnostics

This session brought together chILD center clinicians, community-based physicians, and family advocates to examine the diagnostic journey and identify opportunities to shorten, improve, and standardize it.

Defining Triggering Events

The group devoted substantial time to defining the clinical signals that should prompt a pulmonary or chILD specialist referral. Key trigger events identified included:



- Recurrent bronchiolitis — a child should receive no more than one episode before a pulmonary referral is placed.
- Failure to thrive, particularly in infants.
- Prolonged NICU stay without clinical improvement (“not worse, but not better”).
- Unexplained infant tachypnea not responding to standard interventions.
- Persistent desaturations without clear clinical explanation.

A neonatal nurse practitioner identified NICU follow-up clinics as an untapped referral pathway. These clinics follow infants for 1–2 years and develop ongoing family relationships, but neonatologists tend to conceptualize chILD only in its most severe, ventilator-dependent forms, missing the wide disease spectrum.

Provider Education in Non-chILD Centers

Reaching physicians who will never practice at a chILD center but who are the first point of contact for symptomatic families was identified as a high-priority, feasible intervention. Proposals included:

- Sponsored Grand Rounds presentations at community and regional hospitals.
- Case-based educational content through Radiopaedia and Pathologiaedia platforms.
- A permanent, accessible online database of chILD radiology cases with confirmed diagnoses.
- Patient story-based education for primary care providers and general pediatricians.

The group discussed measuring the effectiveness of educational interventions by tracking new referral rates to chILD centers before and after implementation, using claims data or the chILD Registry as a data source.

The "Code Rare" Concept

A clinician proposed a "Code Rare" protocol in which a standardized rapid-response set of actions is triggered when a patient's presentation does not match recognized patterns and requires specialist consultation or transfer. The group agreed that this protocol should be accessible to families as well as providers, giving parents a formal mechanism to escalate care when standard reassurance is insufficient.

KEY CER PRIORITIES — SESSION 1A

- What are the optimal diagnostic triggering criteria across care settings? [High Impact / High Feasibility]
- Does targeted provider education improve diagnostic timeliness and referral rates? [High Impact / High Feasibility]
- What care coordination models most effectively reduce time to diagnosis? [High Impact / Moderate Feasibility]
- What emergency care protocols best serve chILD patients at community ERs? [High Impact / High Feasibility]
- Foundational needs: Registry infrastructure; validated imaging algorithms; standardized triggering criteria.

Breakout Session 1B: Transition to Adulthood

This session convened a focused group including pediatric and adult pulmonologists, psychologists, social workers, nurses, parents of children at various stages, and two young adults living with chILD. The presence of young adult patients able to speak candidly about their lived experiences gave this session exceptional depth.

Mapping Current Transition Programs

The group identified institutions with formal transition programs, with Boston Children's Hospital as the most advanced. It has a multidisciplinary team including Med-Peds physicians, adult ILD pulmonologists, physical therapy, social work, and psychology, covering both pediatric and adult care. Gaps remain: psychology is fully staffed only on the pediatric side, and a transition nurse coordinator is not yet established on either side. Most institutions lack similar programs.

The Challenge of 'Lost to Care'

A central concern was how to engage young adults who drift from care during or after the transition from pediatric to adult providers. This population is largely invisible to clinical care and research systems, yet their long-term outcomes—health, quality of life, educational attainment, and employment—are precisely what families of young children most urgently want to understand.

Life Transition: Beyond the Clinic

Most of the session addressed transition areas beyond clinical care: college selection, disability services, oxygen accommodations, employment disclosure, workplace planning, insurance continuity at age 26, reproductive decisions, genetic counseling, and roommate choices with home oxygen. The group suggested creating a practical college and life transition checklist covering these key areas.

Peer-to-Peer Mentorship

One of the most impactful recommendations was structured peer-to-peer mentorship. One patient, as a teenager, was matched with a peer recently diagnosed; instead of feeling helped, she felt empowered as a mentor, boosting her self-advocacy and medical knowledge. Another explained how his mother's approach—encouraging him to try activities and set his own limits—helped him become a competitive volleyball player and see his condition as "just part of me, not an affliction."

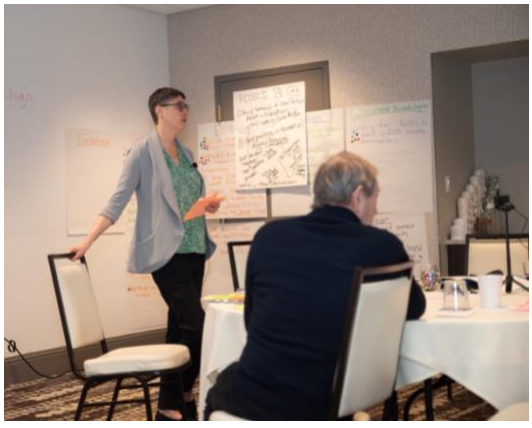
Parental Empowerment and Expectations

Multiple participants emphasized the importance of preparing parents to encourage appropriate independence rather than over-restriction. Both targeted parental guidance and peer mentorship for families of young children were identified as high-priority near-term program needs.

KEY CER AND PROGRAM PRIORITIES — SESSION 1B

- Comparative effectiveness of structured vs. informal transition programs on health and quality of life outcomes.
- Development and validation of a transition readiness assessment for chILD patients.
- Design and evaluation of a peer mentorship program for young adults and families.
- Longitudinal study of post-transition outcomes (health, education, employment, quality of life).
- Development of a practical college and life transition checklist for use in clinical practice.
- Parental guidance program on encouraging healthy independence in children with chILD.

Breakout Session 2A: Care Delivery Models and CER Infrastructure



The afternoon session shifted from exploratory discussion to structured prioritization. Participants organized the full set of family-generated CER questions from Year 1 using a two-dimensional framework: rating each question by impact and feasibility.

Coordinated Complex Care

A chILD center clinician noted the field's growing recognition that chILD is often one manifestation of broader multi-system conditions. Multidisciplinary care coordination was identified as both an urgent clinical need and a foundational research question

Prioritization Framework

High Impact: Would meaningfully improve outcomes for large numbers of families.	High Feasibility: Could be implemented with existing or readily available infrastructure.
Moderate Impact: Would address important needs for a smaller number of families.	Moderate Feasibility: Requires modest infrastructure development or partnership building.
Low Impact: Would not meaningly improve outcomes for families.	Low Feasibility: Requires substantial new infrastructure or long-term capacity building.
Foundational: A necessary building block that enables other improvements.	

Emergency Medical Passport

The group developed the concept of a clinician-authored patient document summarizing baseline parameters and specific care instructions (e.g., “do not adjust supplemental oxygen without contacting the primary pulmonologist”; “expect elevated oxygen requirements—this is the child’s norm”). A parent shared a positive experience with an endocrinology-authored version for adrenal crisis—laboratory orders and treatment protocols were followed without question.

Infrastructure Needs

Participants identified the following as key infrastructure needs:

- A permanent, accessible database of chILD imaging cases with confirmed diagnoses.
- Validated diagnostic and prognostic algorithms for chILD.
- AI tools for automated image analysis and disease tracking.
- A mechanism for institutions to share and interpret cases from other sites without creating liability or free-labor concerns.
- Dedicated research coordinators for imaging studies and bioinformatics and data support.

Registry and Data Infrastructure

Participants raised the need for: quarterly study coordinator calls to improve cross-site enrollment consistency; a searchable directory of registry sites for cross-institutional referrals; and investigation of whether existing adult ILD registries could serve as structural models or collaborative partners for the chILD Registry.

KEY CER PRIORITIES — SESSION 2A

High Impact/High Feasibility- • Does provider education improve diagnostic timeliness? (measured via referral rate changes) • Does an Emergency Medical Passport improve care quality at community ERs?	High Impact/ Moderate Feasibility- • Which telemedicine approaches best support families with limited access to chILD centers? • What care coordination models most effectively reduce time to diagnosis?
Foundational: • Patient registry infrastructure; AI imaging tools; standardized clinical algorithms. • Registry enhancement, data quality, and long-term infrastructure. • AI-assisted radiology tools for chILD diagnosis and disease progression tracking. • Standardized triggering event criteria for diagnostic escalation. • Trained patient partner cohort prepared to participate in CER.	

Breakout Session 2B: Family Support Programs

The afternoon session extended the morning's discussions to the broader domain of psychosocial and educational family support across all age groups. The session focused on identifying which support program models warrant near-term development or comparative study, and what validated tools and patient-reported outcomes should be collected systematically.

Psychosocial Support Needs

The group affirmed that mental health and psychosocial support are among the most consistently identified gaps across all chILD family experiences. Families carry significant psychological burden—from the diagnostic odyssey, from the daily demands of complex caregiving, and from repeated dismissal. One parent's comment captured a theme heard across sessions: "I just can't die"—the weight of knowing that a child's wellbeing is irreplaceably dependent on the parent's survival and vigilance.

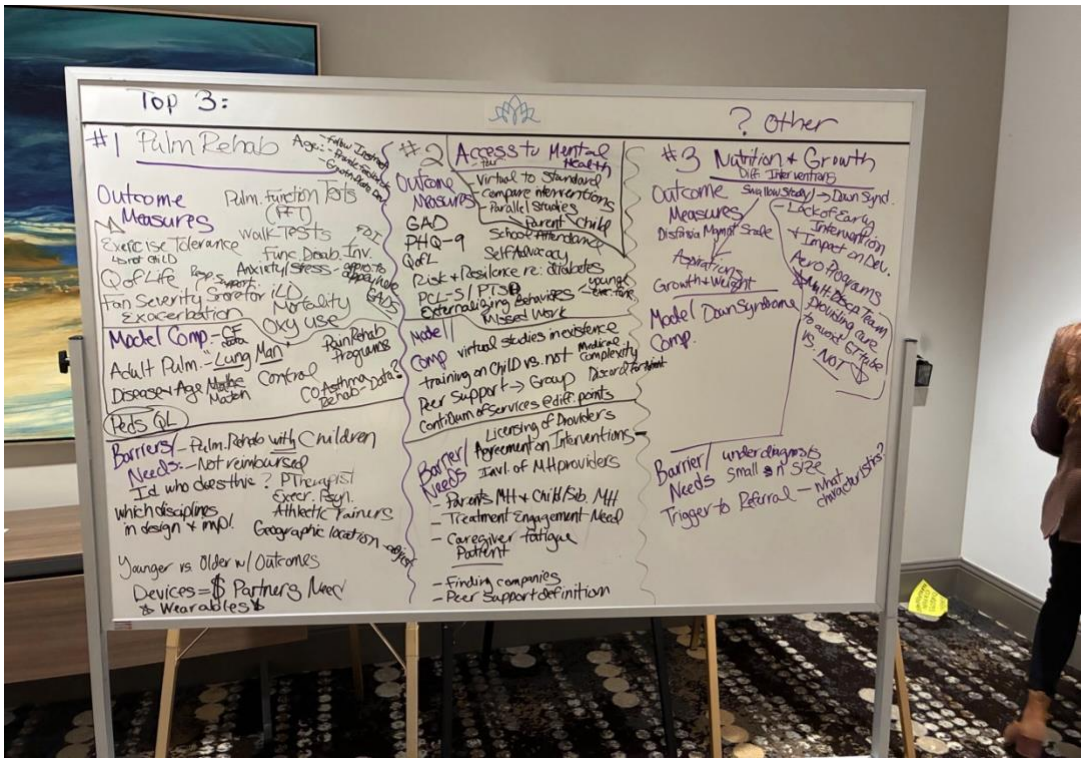
Current psychosocial support is ad hoc and highly institution dependent. Boston Children's was identified as a relatively strong model, with a dedicated pediatric psychologist embedded in the ILD program. Most families, however, have no structured access to mental health support connected to their chILD care.

Peer Support Program Design

The group endorsed a layered peer support model:

- Parent-to-parent connections: parents of newly diagnosed infants connected with parents of older children for practical guidance and realistic expectations.
- Parent-to-adult patient connections: parents of young children connected with adult chILD patients to understand long-term trajectories.
- Young adult peer mentorship: pairing newly diagnosed adolescents or young adults with experienced peers, structured to empower the mentor as much as the mentee.

The Parent Advisory Council (PAC) has already identified peer programming as a near-term priority and is planning a monthly parent support call.



Educational Resources and Self-Management

The chILD Foundation is in the process of completely revamping its website, and there are files of educational content ready for publication, but require physician review, scope definition, and design resources. Proposed topics for a family self-management curriculum included: oxygen management in home and community settings; tube feeding; insurance navigation and prior authorization; school accommodation and IEP planning; and emergency preparedness.

An education fellow position at the Foundation was proposed as a key near-term funding target, to drive both web content development and family education programming.

Young Adult Engagement Strategies

The group discussed engagement approaches for young adults who may be drifting from care:

- Social media outreach and peer-driven digital content.
- Involvement in Foundation activities as contributors, mentors, and advisors—not as recipients of services.
- Participation in research in active, compensated roles.
- Connection through college disability services and online patient communities.

KEY PROGRAM AND RESEARCH PRIORITIES — SESSION 2B

- Design and pilot a layered peer mentorship program (parent-to-parent, parent-to-adult patient, peer-to-peer).
- Develop and evaluate a family psychosocial support program model.
- Identify and validate patient-reported outcome measures for systematic collection in chILD populations.
- Build and publish a comprehensive family self-management resource library on the Foundation website.
- Design a comparative study of structured vs. unstructured family support on caregiver burden and family outcomes.
- Develop and evaluate a college and life transition checklist for use in clinical practice.

Breakout Session 3: Disease Mechanism Working Groups — Building Capacity for Year 2

The final session of the meeting launched Year 2 planning. Year 2 will shift from age-based to disease-mechanism-based topic groups, enabling more targeted CER research questions within biologically coherent disease categories. The proposed working groups are:

- Surfactant disorder (including Pulmonary Alveolar Proteinosis) and alveolar filling disorders.
- Neuroendocrine Hyperplasia of Infancy (NEHI)
- Immune-dysregulation, multi-system disorders, and pulmonary hemorrhage.
- Airway-centered diseases (including Post-Infectious Bronchiolitis Obliterans) and Environmental Exposure.
- Developmental dysregulation disorders.
- Undiagnosed

This session assessed the feasibility, stakeholder landscape, and level of interest in each proposed group. Year 2 groups will focus on capacity-building activities—developing research questions, identifying key partnerships, and characterizing data needs—rather than finalizing study designs. That work will culminate in the final planning meeting in March 2027 and the completion of the CER Research Roadmap.

Next Steps and Future Directions

May 2026	Final reports due from Year 1 SEED Cycle
June 2026	Year 2 disease-mechanism topic groups launch
2026–2027	CER Research Roadmap development (Year 2)
2026–2027	Patient partner training program development
2026–2027	Foundation website educational content publication
March 6–7, 2027	Final Year 2 Planning Meeting, Denver, CO
May 2027 and beyond	CER Research Roadmap submission to PCORI and public dissemination
June 2027	Combined scientific and physician conference (targeted every-other-year cycle)

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