CHILDREN'S HOSPITAL ASSOCIATION

Champions for Children's Health

High-Cost Therapies for Pediatrics

Children's hospitals' ability to continue to meet children's health care needs now and into the future requires innovative approaches to support pediatric patients, families, providers, and facilities in accessing High-Cost Therapies (HCTs) in a safe, appropriate, and sustainable manner. HCTs have lifesaving and life-changing potential for the children who need them, and these treatments require a substantial investment in time and resources to develop and safely deliver to pediatric patients. We support efforts to strengthen and stabilize equitable access to these therapies, particularly those that can be used to treat pediatric conditions, to help ensure that our nation's most vulnerable patients have access to safe and effective health care.

Prevalence of High-Cost Therapies in Pediatrics

Pediatric HCTs treat child patients with chronic, rare, and complex conditions and frequently come with at least a million-dollar price tag. These therapies require advanced education, special handling, and close oversight from pediatric specialists. Many of the emerging HCTs are targeted to treat pediatric rare or genetic diseases and are often most effective when administered early, before the onset of symptoms or irreversible damage occurs. Because HCT treatment is time sensitive, it may require expedited administration to prevent disease progression or meet payor coverage requirements. For example, one drug is safer and more effective when given to children before age 2 since the disease progresses rapidly in untreated infants,[1] while other therapies require administration by a certain age.

Opportunities for Improving Children's Health

HCTs have the potential to change the lives of many children and their families and in some cases cure the illnesses and chronic diseases that have shortened lifespans and challenged their health and well-being for their entire lives. They target unmet pediatric medical needs, have the potential to reduce the need for challenging and costly chronic care, may help address gaps in care and health disparities, and can be lifesaving for previously uncurable illnesses.

Role of Children's Hospitals

Children's hospitals across the United States work relentlessly on innovations that advance knowledge and access to needed HCTs that can transform care for children of all ages. Children's hospitals are at the forefront of developing and leading clinical trials of HCTs and novel clinical programs to safely implement HCTs for optimal clinical outcomes. Our hospitals have created

[1] FDA approves innovative gene therapy to treat pediatric patients with spinal muscular atrophy, a rare disease and leading genetic cause of infant mortality | FDA

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specialized, multidisciplinary centers to continue treatment advancement, bringing innovations from the lab to the bedside quickly and safely. Children's hospitals have developed clinical trials for various HCTs, including treatments for inherited retinal disease, spinal muscular atrophy (SMA), and leukemia.

High-Cost Therapy Pipeline and Approved Therapeutics

The landscape of HCTs has seen a significant focus on pediatric care, with a substantial proportion of both approved therapies and those in development specifically targeting children. Since 2017, 38 HCTs have received approval, with 21 of those important therapies targeting pediatric patients. This underscores the growing recognition of the unique needs of pediatric patients within the broader HCT landscape. Looking ahead, the pipeline remains robust, and this trend highlights the ongoing expansion and prioritization of pediatric-focused HCTs, ensuring that future innovations continue to address the pressing needs of this vulnerable population.

Challenges for Children's Hospitals

Children's hospitals face significant challenges in maintaining long-term access to pediatric HCTs due to the rarity of the diseases they treat, the small patient population, and the perception among some HCT developers that profitability may be limited. However, this perception conflicts with the reality that many of these products will have a significant impact on children's lives, making it crucial to ensure sustained access. Factors such as unpredictable and inadequate reimbursement, patients crossing state lines for care, time-consuming administrative processes, and the lack of payor policies (resulting in prolonged and challenging prior authorization and single-case agreement processes) further contribute to a healthcare system that creates care delays and inhibits sufficient and equitable access to HCTs for pediatric patients.

Reimbursement: Children's hospitals and providers face substantial and unsustainable financial risk when providing HCT treatments. HCTs require a substantial investment in time and resources to order, purchase, receive, store, and administer safely to the patient and reimbursement models do not account for this additional burden. Payors (public and private) are not incentivized to cover these groundbreaking HCTs or to develop formal strategies to support patient access. Because more than half of U.S. children rely on Medicaid as their payor, hospitals struggle with inadequate reimbursement on these therapies with such tremendous price tags.

- HCTs with a high price tag and up-front payment at time of administration create logistical and budgetary hurdles for state Medicaid programs and for providers who offer these clinical services. Many children with rare conditions, disabilities, or medical complexity rely on Medicaid coverage. Differences in coverage criteria from payor to payor are not always well documented, or easily understood, this creates additional challenges for patients and

providers alike. Medicaid coverage for HCTs require specialists to participate as providers in the patient's home state but many children travel long distances for treatment, causing many providers to participate in multiple state programs. It can be time consuming and challenging to enroll providers in out-of-state Medicaid programs. All additional reimbursement challenges have a risk of resulting in costly care delays for children.

- The FDA has not approved pediatric treatments for most diseases, forcing children's hospitals to rely on off-label medications. This practice, while necessary, comes with risks and complexities, as these medications are typically designed for adults and require careful dosage adjustments and monitoring. Even when treatments are FDA-approved, payors often still don't cover them based on lack of FDA approval. As a result, children's hospitals often undertake the time-consuming and costly responsibility of conducting their own clinical research to ensure the safety and efficacy of these treatments. However, payors don't cover the costs of these clinical trials, making it financially unsustainable to ensure access to these critical therapies for ultra-rare diseases. Without adequate financial support, hospitals struggle to maintain the necessary resources to provide these life-saving treatments, potentially limiting access for impacted children.
- Single-case agreements (contracts between a payor and a provider, focused on one therapy for one patient) are time-consuming and have a higher rate of denial, which delays needed care and eventually reimbursement.
- Payors often require prior authorization and may establish a step therapy process for a high-cost therapy that requires the provider to try other less-costly (and less effective) interventions and requires significant documentation of patient decline before the therapy can be used or may subject the therapy to peer-to-peer reviews which can further delay care as well as reimbursement.
- Payors may also require hospitals to access certain HCTs through the pharmacy benefit rather than the medical benefit, even when administered in an inpatient setting. This requirement can be inconsistent with billing requirements and applicable pharmacy regulations through forced "white bagging," requiring that the drugs be dispensed by an outside pharmacy with final preparation and administration occurring within children's hospitals. The hospital is ultimately responsible for product storage (frequently requiring specialized equipment), final drug product dispensing, patient/family education and administration, all tasks for which there is no reimbursement when "white bagging" occurs. Meaning the hospital is asked to take on all the risks without reimbursement.

Access to Care: Children often travel long distances, and sometimes to another state, to receive HCTs at a children's hospital with the specific expertise needed for their treatment. Not only is this care often provided far from a child's home, frequently requiring long hospital stays, but families

who live in rural areas and/or underserved populations may face significant barriers in accessing a needed therapy. Beyond the burden of travel, many children accessing these therapies are on Medicaid and may encounter additional social determinants of health (SDOH) challenges, such as lack of transportation, single parent living situations, and poverty. These barriers make it critical to invest in navigators, other family support, and hospital investments.

- 41 days is the minimum hospital length of stay for some HCTs as bone marrow transplants are often part of the therapy. Adverse effects from this procedure can prolong the process, creating further challenges for patients and families seeking care away from home.
- The timeframe between FDA approval of the HCTs, insurance coverage, and rate hearings can result in care delays and inability to access life-saving care. This is of particular concern when the window of opportunity during which the drug can be administered closes due to the patient aging out.

Administrative Burden: The pediatric workforce is increasingly strained by administrative burdens associated with reimbursement negotiations between the hospital and payors. Children's hospitals have hired additional full-time staff to manage the approval and acquisition management for therapies and it is not uncommon for a hospital to spend hours navigating prior approval processes and submitting multiple appeals of denials on a case-by-case basis for every patient.

- Chief financial officers, chief pharmacy officers, and other senior level executives are often included in the routine acquisition process for each HCT order because of the complexity, which compounds the associated costs to administer these therapies.
- Even after the complex approval and acquisition processes are complete, there remains a risk that the child could become ineligible for treatment, potentially resulting in a heartbreaking missed opportunity for the child's care despite the significant efforts and resources already invested by the hospital.

Clinical Burden: HCT products have unique storage and preparation requirements (throughout storage and distribution), all the way to administration to the pediatric patient. Incorrect storage and handling may result in destruction of HCTs and a financial loss for hospitals. Pediatric hospitals must adhere to strict, costly, and time-consuming protocols that are unique to each HCT to construct and acquire. These protocols can include:

- Ultra-low temperature freezers
- Cell and Gene Therapy specific clean rooms
- Unique delivery methods to maintain product stability
- Specialized clinical certifications and ongoing continuing education requirements

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Identifying Pediatric-Focused Policy Solutions

HCTs represent a transformative advancement in medical care, offering hope to pediatric patients with previously untreatable conditions. Financial and systemic challenges associated with HCTs must be addressed with solutions that ensure sustainable access to these therapies, promotes equity, and supports the long-term viability of our health care system and health of the vulnerable patients we serve.

CHA has convened a multidisciplinary workgroup focused on the unique needs and challenges of children and pediatric health care providers to explore federal policy solutions. It is critical that policymakers work with children's hospitals on how best to explore appropriate mechanisms to balance investments in research and development, manufacturing, and distribution structures for HCTs. Children's hospitals stand ready to share HCT expertise and ensure pediatric patients receive miraculous treatments in a safe and equitable manner.

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