

High-Cost Therapies for Pediatrics

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To meet children's health care needs, now and into the future, children's hospitals must use innovative approaches to support pediatric patients, families, providers, and facilities in accessing High-Cost Therapies (HCTs) in a safe, appropriate, and sustainable manner.

To address the national disparities in specialty drug reimbursement, we urgently need policy models that ensure equitable access and affordability. Without these reforms, pediatric patients could face severe gaps in their necessary treatments, exacerbating health inequities for kids with rare diseases and complex medical conditions.

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 incurable illnesses.

Unique Challenges for Children's Hospitals

Pediatric health care providers face unique HCT treatment challenges due to inadequate reimbursement, clinical burdens, and time-consuming administrative processes that can delay needed health services. Children's hospitals, where children come for these life-altering treatments, are concerned that inadequate reimbursement from Medicaid and/or commercial insurance will not support the therapies or the associated care required to successfully treat patients. Children must often travel long distances for these treatments, which frequently includes seeking out of state care, creating challenges for families and additional reimbursement hurdles.

Identifying Pediatric-Focused Policy Solutions

The Children's Hospital Association is working with leaders from children's hospitals to determine federal policies that could help support HCTs and ensure equitable access for children. Children's hospitals stand ready to work with policymakers on how best to explore solutions that ensure sustainable access to HCTs for children and support the long-term viability of our pediatric health care system.

Fast Facts

Pediatric HCTs, including cell and gene therapies, are innovative, potentially curative, and lifesaving or life-altering treatments for children with chronic, rare, and complex conditions. Treatment is most effective before the onset of symptoms or irreversible damage.

>HALF

of HCTs approved since 2017 are for children.

Many HCTs cost
>\$1M.

Children's Hospitals' Unique Role

Children's hospitals work steadfastly on innovations that advance knowledge and access to miraculous and transformative HCTs, for children of all ages. Our hospitals have developed clinical trials for HCTs that treat many conditions including a form of blindness, spinal muscular atrophy (SMA), and leukemia.