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January 22, 2024

The Honorable Bill Cassidy, M.D.
Ranking Member
Committee on Health, Education, Labor, and Pensions
United States Senate
Washington, DC 20510

Re: Request for information on improving Americans' access to gene therapies

Dear Senator Cassidy,

On behalf of the nation's children's hospitals and the patients and families we serve, thank you for the opportunity to respond to your request for information (RFI) on cell and gene therapies. We appreciate your efforts to ensure the nation is better prepared to develop a framework for payment and access to these therapies for patients and encourage you to prioritize the distinct needs of children, who represent 25% of the total U.S. population. Our responses to relevant aspects of your RFI highlight the ways that policy solutions can meet the unique and very specialized needs of children with rare, serious, and complex medical conditions.

Cell and gene therapies have the potential to change the life trajectory for children. They address unmet pediatric medical needs, have the potential to reduce the need for costly chronic care, may help address gaps in care for underserved populations and eliminate health disparities, and can be lifesaving. Therefore, we strongly 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 safe and effective health care.

The more than 200 children's hospitals that comprise the Children's Hospital Association (CHA) are dedicated to the health and well-being of our nation's children. Children's hospitals advance child health through innovations in the quality, cost, and care delivery—regardless of payer—and serve as a vital safety net for uninsured, underinsured, and publicly insured children. Medicaid, on average, provides health insurance coverage for half of children's hospitals' patients and for some children's hospitals patient mix, closer to three-quarters. Though children's hospitals account for only 5% of hospitals in the U.S, they account for about 45% of all hospital days for children on Medicaid. We serve the majority of children with serious, chronic and complex conditions, providing 95% of all pediatric cancer care, and most children in need of major surgery. We are regional centers for children's health, providing highly specialized pediatric care across large geographic areas.

Children are not little adults. They are constantly growing and developing, and their health care needs, the delivery system to meet those needs, and support systems (e.g., schools, childcare settings) are different from those of adults. Pediatric care requires specialized medications, therapeutics, and equipment, as well as extra time, monitoring, and specially trained health care providers who are compassionate and understand kids of all ages and from all backgrounds. It is critical that pediatric-focused innovations in cell and gene therapies are developed, reimbursed, and available to meet children's unique needs.

What is the Current Practice for Patients with Ultra-Rare Diseases or Disorders?

#3. How do patient populations currently access and pay for these therapies?

Children's hospitals will be administering these therapies in many cases. When a therapy is covered by public or private payers, our pediatric patients access these therapies in a similar manner as they do any other medication whereby a team of nursing and pharmacy technicians work with insurers (public or private) to complete a prior authorization following a physician's order for the therapy. For rare disorders, patients may access them as part of an early phase clinical trial or apply for expanded access to a medication, if such a medication exists that is not readily available by standard of care means.

There are also circumstances where access is facilitated in part by the pharmaceutical companies themselves that communicate, and market, directly with families regarding steps in obtaining medications. However, in these circumstances, families may not have all the information they need about the drug, such as clinical eligibility to receive the treatment, insurance coverage for the treatment, prior authorization requirements, their out-of-pocket costs, etc. Furthermore, the lack of coordination with the child's provider who is overseeing the clinical aspects of care, as well as working with payers to navigate coverage and payment processes, can delay rather than expedite access to the needed therapy.

It is especially crucial that coverage and reimbursement barriers for cell and gene therapies in the Medicaid program are addressed to ensure timely patient access. Medicaid and CHIP are the primary insurer for the pediatric population and on average, covers between one-half to three-quarters of the children cared for by children's hospitals, it plays a critical role in children's access to these life-saving therapies. The cost of these treatments can limit patient access as Medicaid programs operate on limited or fixed budgets. Gene therapies with a high price tag and a required up-front payment at time of administration create logistical and budgetary hurdles for state Medicaid programs.

A national approach is warranted to ensure that all children – and not just those who live in certain states or areas of the country – have access to these therapies in an equitable manner. Often, a child will have to travel to another state to receive these therapies at a children's hospital with the specific expertise needed to provide high-quality cell and gene therapy treatment and services. Children who live in rural areas and/or are from underserved populations may face particular barriers in accessing a needed therapy. Not only will this care often be provided far from a child's home, but it will require long hospital stays.

In particular, administrative and payment requirements for out-of-state care under Medicaid can impede access to timely care. Providers seeking to use cell and gene therapies to treat out-of-state Medicaid beneficiaries must bill the child patient's home state Medicaid program. To receive payment, the provider must also enroll with and be credentialed by that patient's Medicaid program. Since these credentialing procedures are often time-consuming, complicated and costly, they can delay or even block the ability of a provider to treat out of state patients—many of whom may require immediate care due to severe and complex medical conditions. As a result, children who live in under-resourced areas or even some of the predominantly rural states, will likely not be able to receive treatment or will face greater difficulties in doing so.

#4. What, if any, federal or state programs do these patient populations use as they seek to pay for and access these therapies and related care? What is the specific benefit to the patient in using these programs? For example, interested parties could contemplate instances where families choose to "spend down" to become eligible for state Medicaid programs, thus ensuring coverage.

As we note above, Medicaid covers the majority of children nationwide, providing affordable coverage to children in lower-income families, children with special health care needs, and the nation's sickest children, including those in need of cell and gene therapy. Medicaid was designed with children's needs in mind and fills gaps in services not covered by private insurance.

However, Medicaid's coverage of cell and gene therapies for children varies significantly by state given their high cost and the variations in particular disease prevalence among states. For example, sickle cell disease largely impacts communities of color and southern states[1] and its treatment represents a large economic burden for state Medicaid programs. However, effective treatment greatly improve quality of life, and for children with the disease, has very positive implications for their long-term health and well-being.

It is critical that reimbursement methodologies for cell and gene therapies address the full costs of these therapies to ensure that providers, including children's hospitals, can continue to offer the treatment in a financially sustainable way and patient families do not incur exorbitant out-of-pocket costs that put the treatment out of reach. Reimbursement must cover the drug itself, as well as the costs of the procedures and hospitalizations needed to administer the treatment. For example, the recently-approved, milestone treatments for sickle cell disease-- Lyfgenia and Casgevy – require two to three hospitalizations to harvest stem cells, medication to treat and store the stem cells, multiple transfusions, approximately two months of inpatient care, possible ICU care, blood products, antibiotics, pain medication, and follow up for a number of years. All aspects of this treatment must be covered to make these therapies a life-saving and life-altering reality for patients. The life-saving drug Spinraza, which is used to treat children with spinal muscular atrophy (SMA) – a progressive neurodegenerative disease that impacts the muscles used for activities, such as breathing, eating, crawling, and walking – is estimated to cost between $625,000 and $750,000 in the first year and then $375,000 annually thereafter. Patients will need to take the medication for the rest of their lives for continued efficacy.

Providers that administer these life-saving treatments must be protected from taking large financial losses from inadequate levels of reimbursement and claims processing and payment delays and complicated procedures. Children's hospitals report that they are paying upfront for the therapies to ensure that their child patients receive needed care but then must wait months for reimbursement. Given the high costs of these therapies, the lack of timely payment places an inordinate amount of financial stress on the hospital, which is unsustainable and could lead to less access to needed care.

State Medicaid agencies are adopting a variety of reimbursement methodologies to ensure patient access to needed cell and gene therapies while addressing their high cost. Most Medicaid programs use a diagnosis-related group system to reimburse hospitals for inpatient services, where a hospital receives a bundled payment for both the services provided and any drugs administered. However, some states are implementing a payment policy where the pharmacy is paid directly for the therapy, or the hospital is paid separately for the cell preparation or drug and the inpatient administration of that drug.

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[1] Population Distribution by Race/Ethnicity | KFF

Another approach to balancing costs and patient access is value-based payment (VBP) arrangements, where performance-based contracts are linked to targets around efficacy and durability of patient response. Some states have established Medicaid pharmacy supplemental rebate agreements that provide them with legal authority to enter into VBP arrangements.

In addition, several states are using reinsurance programs to manage cell and gene therapy costs. These programs are intended to provide protection to Medicaid managed care plans, which, depending on their size, can face financial stress if they pay a higher-than-expected number of cell and gene therapy claims.

#5. What, if any, manufacturer-sponsored programs do these patient populations currently use to access and pay for these therapies? How do patient populations apply for or access these programs?

Manufacturer-sponsored assistance programs provide travel support and other resources to help pediatric patients and their families access therapies, but they are typically only available to those with private insurance. However, gene and cell therapies are usually intended for one-time administration, and as a result, manufacturers are unlikely to offer assistance programs for patients given the lack of downstream opportunity to capture patient revenue. Furthermore, government payers such as Medicaid and Tricare are generally excluded from these programs. Therefore, the large proportion of very sick children who are covered by Medicaid are unlikely to benefit from assistance programs.

When a child has commercial coverage, the children's hospital or health system team members will connect and support their families as they navigate their insurance coverage and various manufacturer programs to facilitate cost-sharing assistance. If a patient is eligible for a manufacturer's program, that program will work with the health system to input appropriate information to make the assistance program effectively a "secondary payer" in the hospital or pharmacy's billing system. Those pediatric patients may have limited out-of-pocket medical expenses to receive these therapies.

#7. What, if any, are the utilization management tools (e.g. step therapy, prior authorization) that patients are typically subject to when paying for and accessing these therapies? If not the patient, what individual or entity typically works through the process of obtaining approvals?

Congress should explore ways to help ensure that children's access to these therapies is not impeded by burdensome utilization management requirements or reimbursement processes. Children's hospitals work tirelessly on studies, trials and innovations that advance knowledge and access to needed cell and gene therapy treatments for children of all ages. However, their promising work is not always translated into accessible bedside treatments due to manufacturers' and payers' policies that can impede children's access to these very treatments. Utilization management and controls can be particularly problematic when the FDA has approved a therapy for a limited age range. A child may age out of the label approval while waiting for the payer reimbursement to be approved or payment procedures to be developed for a newer therapy.

Reimbursement for these expensive therapies is often negotiated through single-case agreements (contracts between a payer and an out-of-network provider) because children are often traveling a long distance and out-of-network for their treatment. Single-case agreements are time-consuming and have a higher rate of denial, which delays needed

care. Every intermediary in the system causes additional administrative delays as supplemental rebates and other services included in the single-case agreement are negotiated by health plans, states, and treatment centers[2].

In addition, payers often require prior authorization and may establish a step therapy process for a high-cost therapy —requiring the provider to try other less-costly interventions and document that they are ineffective—before the therapy can be used, or may subject the therapy to peer-to-peer reviews. Each payer has its own discrete approval and payment policies and procedures that hospitals must navigate and manage. Payers are also increasingly placing additional clinical monitoring requirements on treatments that delay approval of their use. Children's hospitals have had to hire additional FTEs to simply handle 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. At the end of this long, time and resource-intensive process, the payer will often approve the use of the therapy. Unfortunately, children and their families are caught in the middle with needed care delayed.

Furthermore, some manufacturers mandate white bagging, which can delay care and create safety concerns. Under a white bagging method, plans contract with a specialty pharmacy to purchase the drug and ship it to the hospital. By removing the pharmacy procurement team from the procurement function of the drug, white bagging exists outside of the standard clinical decision support processes that would normally optimize safe medication administration and effective coordination of care. There are instances when patients and families travel to a hospital for an anticipated therapy that was white bagged and the specialty pharmacy failed to properly coordinate. The family is then sent home without treatment because the white bagged medication wasn't available at the hospital yet, which further delays care and leads to adverse clinical outcomes. In addition, hospitals are not reimbursed for the time, professional services, and handling of these white bagged medications.

How Do Supply Chain Intermediaries Price and Design Contracts?

#29. What are typical contract designs between the drug manufacturer, wholesaler, and distributor as they seek to provide access to these therapies?

As noted above, contract designs typically are single-case agreements, which are very time-consuming for manufacturers, payers, and health care providers to negotiate since certain criteria must be met based on patient location and cost, among other factors. Payers are more likely to deny care under a single-case agreement, which delays care for pediatric patients in need of timely treatment for life-threatening and complex medical conditions. In order to adjudicate the denials and implement these agreements, manufacturers often need to confer with the child patient's state Medicaid agency, the out-of-state Medicaid agency, the Medicaid managed care plan, the treatment centers, and referring pediatric providers. Access to needed treatment can be further delayed by negotiations among the many players over payment issues, such as supplemental rebates and the specific services included as part of the agreement.

What special supply chain considerations have to be made for these therapies as the drug manufacturer, wholesaler, and distributor seek to distribute these therapies?

As we note above, the manufacturer or pharmacy benefit manager may mandate white bagging for cell and gene therapies, which requires increased provider time and patient family engagement and can result in improper dosing,

[2] 20231106-ARM_Medicaid-Access-Barriers-Issue_WEB.pdf (alliancerm.org)

medication errors, or impeded access to the treatment. The atypical processes, and lack of quality control that can be associated with white bagging, can put the child patient's health at risk. If drugs don't arrive to children in time due to delivery challenges, much-needed therapies for these patients can be delayed creating an undue hardship on pediatric patients and their families who already made schedule accommodations to access the treatment and leading to poorer outcomes.

#32. What are typical contract designs between wholesaler, distributor, group purchasing organization (GPO), health provider, and pharmacy as they seek to provide access to these therapies? For instance, interested parties could contemplate the value provided by exclusive or nonexclusive contracts between two entities.

There are currently no GPO contracts for cell and gene therapies, but this could change as competition increases when more therapies come to market. As we note above, most contract designs are single-case agreements between a payer and an out-of-network provider.

We caution against the use of exclusive contracts since they limit the acquisition flow of the cell and gene therapies and can increase patient risk**.** When payers dictate specific distribution methods for products in an attempt to control costs, they take away a providers' choice for their patients and their facility, and delay access.

#33. Please share any other relevant information in regard to the role supply chain intermediaries play in providing access to these therapies.

There are unique challenges in ensuring long-term access to pediatric cell and gene therapies, given the rarity of the pediatric diseases they treat, the small population (i.e., market), and developers' perception of their ability to recover profit. Because these treatments serve only a small patient population they are not mass-produced like traditional biologics. Additionally, since this is a relatively new industry, there is a shortage of skilled workers to produce and manufacture the treatments, driving up manufacturers' labor costs. At the same time, there is an increased demand for cell and gene therapies, which is forcing developers to outsource treatment production and manufacturing, leading to increased production time and costs.

Furthermore, the FDA has not approved treatments for most ultra-rare diseases. As a result, some children's hospitals are forced to use off-label medications are then likely to undertake the time-consuming and costly responsibility of manufacturing the product, implementing delivery processes, and monitoring the patient through their own clinical research system. Payers are not always willing to cover these clinical trials which means that ensuring access to these needed therapies for ultra-rare diseases can be financially unsustainable. These pediatric academic institutions also struggle to ensure that their child patients' insurance will cover their clinical trials of these therapies. For these reasons, alternative methods of financial support are needed to allow for clinical trials and sustaining production of these products for children.

In addition, pediatric-focused rollout strategies for cell and gene therapy products are also needed to ensure access. In particular, cross-border credentialing, education, and communication between states remain the highest concerns for cell and gene therapy manufacturers as they seek to launch successfully. Cross border issues are especially crucial for pediatric patients who often must travel to a neighboring state to receive a therapy as cell and gene therapy manufacturers typically launch with a small number of qualified treatment centers to balance the number of treatment sites with the number of patients they expect in each region.

Pre-launch discussions between the manufacturer and providers can support successful cell and gene therapy product rollouts for children. For example, children's hospitals have met with manufacturers before launching a cell and gene therapy – the muscular atrophy drug Spinraza being one example – to address distribution mechanisms and processes and related issues to enhance health outcomes.

What is the Future of Access for These Therapies?

#39. What is the appropriate role of the federal government in ensuring access to these therapies in the commercial market? How can any steps taken on the federal level ensure expanded access while not hurting innovation in this area?

It is critical that policymakers work with a broad set of agency stakeholders on how best to explore appropriate mechanisms to establish reasonable pricing for therapies that balance investments in research and development, manufacturing, distribution, and reimbursement structures. Public-private partnerships are essential to the development of new therapies, especially those aimed at treating rare pediatric diseases. We support a federal workgroup including payers, hospitals, manufacturers, associations, and state Medicaid agencies to work through the creation of a federal reimbursement model to support access to cell and gene therapies.

Furthermore, Congress should look for ways to measure the impact of reimbursement policies on patient access, particularly for children covered by Medicaid. It is critical that the link between payment policies and access are evaluated and documented, and that children's access is addressed in any policy solution. This analysis should look at the relationship between payment policies, utilization management, and delivery site restrictions and timely access. There are tremendous financial implications for hospitals related to the administration of a million-dollar drug to a child when there is not a guarantee of adequate payment. Some hospitals may be forced to weigh their ability to care for children who may need those therapies, which could impede a child's access to life-saving cures.

#41. What are the anticipated costs or savings to health systems, plans, payers, or patients as a greater number of these therapies become available?

As more cell and gene therapies become available, the anticipated costs or savings will vary depending on the disease state, the cost of therapy, and the treatment outcome. It is important to look at improving coordination between state Medicaid programs, manufacturers, providers, specialty pharmacies, and the FDA as new therapies come to market to prevent delays in care for children.

Congress should create avenues for early communications between all intermediaries with a focus on equitable payment and access strategies. Advanced information exchange regarding a new cell and gene therapy can help ensure that both public and private payers are not caught off guard with new market introductions and expedite pediatric patients' access. States with Medicaid managed care allow the health plans to establish the specific approval standards for access to a new high-cost therapy. The plans will typically take a substantial amount of time to develop and operationalize these utilization controls, which can be particularly problematic when the FDA has approved a therapy for a limited age range.

How Should Federal or State Governments Promote Access to New Models?

#55. How could the federal government leverage existing alternative coverage models in order to promote commercial access to these therapies? For instance, interested parties could contemplate changes to

independent, noncoordinated excepted benefits, which could allow health plans and payers to subsidize add-on benefits for these therapies.

Congress should work with CMS on ways to strengthen Medicaid and Exchange plan reimbursement for cell and gene therapies to help ensure that children have access to the care they need. Pediatric care requires specialized medications, therapeutics, and equipment, as well as extra time, monitoring, and specially trained health care providers who are compassionate and understand kids of all ages and from all backgrounds. Pricing levels and reimbursement should account for all aspects of their development as well as their delivery to the patient, including risk of handling and professional services (e.g., compounding/infusion/counseling).

As Congress considers approaches to adequately reimburse for cell and gene therapies, it is important to recognize that any changes to Medicare policies may have a downstream impact on Medicaid. Despite the low numbers of children covered by Medicare[3] its policies can affect all children, even though the policies are not developed with children in mind. Medicaid and private payers often adopt Medicare rules and procedures without evaluation of their impact on pediatrics. Therefore, it's critical that the CMS Center for Medicaid and CHIP Services, pediatric providers, and other child-focused stakeholders are consulted closely on the development of drug pricing policies that will advance child health.

Thank you again for the opportunity to provide feedback. We look forward to working with you to ensure the needs of children are met when addressing payment and access for cell and gene therapies. Please contact Natalie Torentinos at Natalie.Torentinos@childrenshospitals.org or (202) 753-5372 should you need more information.

Sincerely,

Aimee C. Ossman

Aimee Ossman
Vice President, Policy
Children's Hospital Association

[3] Some children who receive care at children's hospitals are covered by Medicare, but represent a very small percentage of our patients. Typically, these children have end-stage renal disease (ESRD). According to the CDC, fewer than 10,000 children and adolescents in the U.S. are living with ESRD and not all of them receive care in children's hospitals.