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March 11, 2022

CHA Response to Healthy Future Task Force Treatments Subcommittee - Request for Information

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 medical innovation. Our response focuses on critically important pediatric-specific considerations that must be addressed in any policies to support the development and availability of life-saving treatments, devices, and diagnostics, and address rising costs.

The more than 220 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 delivery of care—regardless of payer—and serve as a vital safety net for uninsured, underinsured and publicly insured children. 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. Pediatric care also typically involves other family members or guardians. Children's hospitals, unlike adult-focused medical facilities, are equipped to provide this child-centric care.

Our responses to relevant aspects of your RFI highlight the ways that your policy solutions can meet the unique needs of all children, including the very specialized needs of children with complex conditions.

Goal 1: Evaluate potential innovative payment solutions for expensive curative therapies in Medicare and Medicaid

Questions:

- **How should government payors use innovative payment methodologies to pay for expensive new drugs, diagnostics, and devices?**
- **Would a singular model for reimbursement of curative therapies help or hurt development?**
- **How would setting the price of certain drugs in Medicare impact future curative therapies?**

We encourage the task force to address the unique challenges that children's hospitals face in ensuring that their child patients have timely access to needed high-cost therapies when it considers innovative payment methodologies to pay for new drugs, diagnostics and devices. Those challenges and related recommendations are discussed below.

- Early development of payment and access strategies. It is important to look at improving coordination between payers (including state Medicaid programs), manufacturers, patients, specialty pharmacies and the FDA as new high-cost therapies (HCTs) come to market. Congress should create avenues for early communications between all players with a focus on payment and access strategies. Advanced information exchange regarding a new HCT can help ensure that both public and private payers are not caught off guard with new market introductions and expedite patient access to those therapies.

When a new curative drug enters the market, it can take months for payer procedures to be developed, which can lead to delays in patient access. For example, under Medicaid—which covers close to half of all children, including 3.4 million children in military families and 1.7 million who qualify due to a disability—states have flexibility to administer and manage their pharmacy benefit. States with Medicaid managed care allow the health plans to establish the specific approval standards for access to a new HCT. 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. A child may "age out" of the label approval while waiting for the payer procedures to be developed. A HCT is of little value if it cannot be accessed by our patients.

- Drug development and pricing. We encourage Congress to work with a broad set of stakeholders to identify mechanisms to establish reasonable pricing for new therapies that balance investments in research and development, manufacturing, distribution, and reimbursement structures. For example, public-private partnerships are essential to the development of new therapies, especially those aimed at treating rare pediatric diseases. Funding from government sources, such as the NIH, as well as children's hospital research foundations complement private investment throughout the pediatric drug development and clinical trial process. Examples of drugs that were developed through partnerships with children's hospital researchers include the life-saving drugs Spinraza and Zolgensma, which are used to treat children with spinal muscular atrophy (SMA). SMA is a progressive neurodegenerative disease that impacts the muscles used for activities, such as breathing, eating, crawling, and walking. It is the number one genetic cause of death for infants. Spinraza is estimated to cost between $625,000 and $750,000 in the first year and then $375,000 annually thereafter[1]. Patients will need to take the medication for the rest of their lives for continued efficacy[2]. It is essential that the pricing, distribution and reimbursement structures for these and other life-saving drugs do not jeopardize patient access or health outcomes.

- Adequate reimbursement levels. Congress should work with CMS on the establishment of appropriate levels of reimbursement for HCTs to help ensure that children have access to the care they need. The determination of an adequate price and reimbursement level for HCTs must account for all aspects of their development as well as their delivery to the patient, including risk of handling, professional services (compounding/infusion/counseling) etc. Inadequate reimbursement from payers risks disincentivizing timely treatment and may hinder access to care for children with medical complexities. For example, two CAR T-cell therapy drugs, Kymriah and Yescarta, have received approval from the FDA, but with list prices at $475,000 and $373,000 respectively[3], are resource-intensive.

- Setting drug prices in Medicare. We support efforts to lower drug prices and welcome the opportunity to partner with Congress to reduce unnecessary drug spending. As the task force examines drug pricing policies under Medicare, including their impact on the development of future curative therapies, it is important to recognize their potential implications for pediatrics. Despite the low numbers of children covered by Medicare[4] 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. We urge the task force to

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[1] Katie Thomas, *Costly Drug for Fatal Muscular Disease Wins F.D.A. Approval*, New York Times, Dec. 30, 2016, https://www.nytimes.com/2016/12/30/business/spinraza-price.html.

[2] *Id.*

[3] Mary Caffrey, *With Approval of CAR T-Cell Therapy Comes the Next Challenge: Payer Coverage*, American Journal of Managed Care, Feb. 21, 2018, http://www.ajmc.com/journals/evidence-based-oncology/2018/february-2018/with-approval-of-car-tcell-therapy-comes-the-next-challenge-payer-coverage.

[4] 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.

work closely with the CMS Center for Medicaid and CHIP Services, pediatric providers and other child-focused stakeholders on drug pricing policies that will advance child health.

- Value-based payments**.** We encourage the task force to explore the use of value-based payments for HCTs. One approach could be to allow state Medicaid programs to enter into time-limited risk-sharing value-based agreements with manufacturers of certain drugs, such as one-time use gene therapies, subject to CMS approval. Under these arrangements, a state could stop payment if the therapy does not meet certain clinical expectations.

- Payers' distribution methods. Increasingly, payers are dictating specific distribution methods for products in an attempt to control costs, taking away a hospital's ability to pick what works best for their patients and their facility, and delaying access. We encourage Congress to consider ways to limit payers' ability to use delivery methods that compromise patient care and can lead to inadequate provider reimbursement.

  There are often two methods used to receive a drug: "buy and bill" and "white bagging." For buy and bill, hospitals purchase, stock and prepare the drug. Then the hospital seeks reimbursement for the drug and related administering fees. Under a white bagging method, plans contract with a specialty pharmacy to purchase the drug and ship it to the hospital.

  Some payers are beginning to dictate coverage through a white bagging-only method, while some states and hospitals are limiting the use of white bagging due to legal and quality concerns. For example, administrative and delivery delays, inadequate storage systems, shipping safety protections, lost shipments and a lack of coordination between the receipt of a drug and its dose administration schedule can result in improper dosing, medication errors or impeded access to the treatment. In addition, hospitals are not adequately reimbursed for the time, professional services, and handling of these white bagged medications. Often, there is no reimbursement at all.

Question: What other policies should we consider to lower costs while maintaining access to lifesaving cures?
Children's hospitals are at the forefront in delivering new cures to patients and, as a result, diseases and conditions that were once seen as untreatable are now being cured or greatly improved. Congress should explore ways to help ensure that children's access to these exciting new therapies is not impeded by payers' burdensome utilization management requirements or reimbursement processes.

- Utilization management systems. We encourage Congress to consider how technology could be used to improve the stringent utilization control procedures that payers have adopted in relation to HCTs. We agree with the importance of assuring the appropriate use of a therapy for a particular child patient, but note that these processes can be extremely time and resource-intensive and put enormous stress on the families and patients in need of a proven therapeutic.

  Payers often require prior authorization, may establish a step therapy process for a HCT—requiring the provider to try other less-costly interventions and document that they are ineffective—before the HCT can be used, or may subject the HCT to peer-to-peer reviews. In addition, each payer has its own discrete approval and payment policies and procedures that hospitals must navigate and manage. Furthermore, payers are increasingly placing additional clinical monitoring requirements on drugs that delay approval of their use. Children's hospitals have had to hire additional FTEs to simply handle the approval and acquisition management for HCTs 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 drug. Unfortunately, children and their families are caught in the middle with needed care delayed.

- The Fair Drug Prices for Kids Act (S. 2531). Congress should expand access to affordable prescription drugs for children enrolled in the Children's Health Insurance Program (CHIP) by enacting the Fair Drug Prices for Kids Act. This legislation would give states the ability to purchase prescription drugs at the lowest price possible for children who are enrolled in stand-alone CHIP programs through the Medicaid Drug Rebate Program. This would generate immediate savings for states and the federal government. With those savings, states could provide more children with access to needed prescription drugs.

- Closed formularies. As the task force considers ways to reduce the costs of life-saving therapies, we caution against the use of commercial-style closed formularies. Pediatric-specific considerations are often not taken into account under a closed formulary structure and off-label medications are frequently prescribed to children, particularly for unique child populations, such as children with chronic or rare diseases.[5] A closed formulary could limit timely access to these types of critically important drug therapies, which may be the only available treatment option for children, even if an exceptions process is allowed. Additionally, many new gene therapies must be administered when first symptoms are identified in order to be most effective. Any delay in providing these drugs due to the need, under a closed formulary, to seek an exception could lead to worsening of the condition or result in higher costs. Closed formularies are particularly concerning for children being treated for epilepsy or a mental health condition. These child patients spend months working with their doctor searching for a drug that works with their body chemistry. Under a closed/limited formulary structure the child's current prescription may not be covered causing a potentially problematic interruption to the child's care plan.

- Measuring the impact of reimbursement on access. As new HCTs enter the market, 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. For example, 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. As a result, some hospitals may be forced to weigh their ability to administer these HCTs and care for the thousands of children who may need those therapies. When financial stressors resulting from under-payments force a hospital to limit its use of a particular HCT, access to that therapy is limited and will impede a child's access to life-saving cures.

Goal 2: Encourage innovation and make the Medicare system more flexible to be able absorb new innovative drugs, devices, diagnostics while being good stewards of taxpayer dollars

Question: What barriers to innovation in the drug, device, or diagnostic space should Congress address?
It is essential that children of all ages are included in clinical research to ensure that they benefit and do not experience unintended harm. However, we know that it is difficult to recruit children into clinical trials. We need to better understand how many and which children are enrolled in trials to develop effective recruitment and retention strategies. Congress should work with the federal biomedical research enterprise to develop a requisite data collection and reporting structure that would allow for an accurate picture of how many children are enrolled in clinical studies and trials and where gaps exist. This data should be broken down by pediatric subgroup (e.g., neonates, infants, children, adolescents, etc.) to provide a more complete picture of which populations of children are underrepresented.

In addition, we are facing a severe pediatric health care workforce shortage that is not only affecting our bedside health care workers, but also our pediatric clinical and research workers.

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[5] See https://pediatrics.aappublications.org/content/133/3/563.

As you consider how best to address barriers to innovation, we urge you to consider ways to strengthen our pediatric researcher workforce pipeline, including in the area of pediatric pharmacology. Pediatric pharmacists play a critical role in research and drug discovery, advancing therapeutics that can meet the unique needs of kids. As the workforce ages, it is critical to cultivate the next generation of pharmacologists to develop clinical trials and accelerate discovery.

Question: How can the FDA's Accelerated Approval Program be improved upon and better integrated with Medicare coverage determinations to expand access to innovative treatments, therapies, and devices while maintaining consumer protections?
Children's hospitals work tirelessly on studies, trials and innovations that advance knowledge and access to needed 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. Congress should consider ways to work with CMS to address low Medicaid and commercial reimbursement rates that make it financially difficult for children's hospitals to secure those therapies and provide them to the children who need it. Congress should also work with payers to ensure that utilization controls and payment mechanisms do not unnecessarily impede access to needed therapies and explore strategies to address quality and safety concerns related to their medication delivery mechanisms, including the mandatory use of external specialty pharmacies.

Question: What are the various categories of Digital Health that need to be recognized from the standpoint of reimbursement to begin exploring the mechanisms for coverage, coding, and payment that may already exist, and to understand where gaps remain under current regulatory and statutory frameworks?
We wish to highlight a few pediatric-specific considerations that must be taken into account in any policy initiatives related to the advancement of digital health. Any policy changes regarding new technologies must factor in the unique needs of the pediatric setting, and the challenges of assuring interconnectedness of large health information technology (HIT) systems for pediatric providers. As regional centers for children's health, children's hospitals are especially attuned to the value and need for a strong digital health infrastructure to support high-quality care across pediatric settings. Children's hospitals are working hard to preserve a balance between delivering better quality patient care and gauging the risk profile of each new technology. As such, we note that there is a critically important subset of digital health/health information technology (HIT) issues unique to pediatric hospital care that are crucial to advancing children's health, and promoting care coordination and information exchange between patients, families and providers; among providers; and with local, state, and federal agencies.

- Pediatric electronic health records (EHRs). Children's hospitals were pleased that the Office of the National Coordinator (ONC) 21st Century Cures Act: Interoperability, Information Blocking, and the ONC Health IT Certification Program rule included guidelines for pediatric EHR certification. Those guidelines and clinical priority certification recommendations represent a positive step forward for the improvement of EHRs used in the care of children. However, we believe Congress could help advance the use of pediatric EHRs by requiring pediatric-specific testing and requiring rather than allowing EHR technologies to comply with the full package of pediatric certification standards. Congress also will have a key oversight role to play as adoption of the new standards moves forward. Pediatric EHR platforms come at a high cost and require constant updating and reworking to ensure compliance with federal and state requirements and to meet the unique needs of pediatric providers—particularly in the hospital setting. The lack of standardized pediatric EHR elements even with the new voluntary pediatric standards leads to resource-intensive "work-arounds" by children's hospitals. Children's hospitals' adoption of new digital health tools will rely on a strong pediatric EHR system that can serve as the connector for patients, families, providers and community partners.

- Proxy and teen privacy considerations. Federal policies must ensure providers have the flexibility to adapt to the confidentiality needs of a patient's particular situation, including restricting access to some personal health information (PHI), when developing and implementing new digital technologies. There are unique privacy and confidentiality, proxy and safety issues that must be addressed to accommodate the role that parent/guardian

proxies and adolescent confidentiality concerns play in pediatrics. For example, children's hospitals work with—and obtain proxy consent from—parents, legal guardians, or other authorized representatives when providing care to minor children and before extending an invitation for portal access. It is not unusual for a hospital to receive a request from a parent, non-guardian, foster parent, or temporary court-ordered care provider—i.e., extended family, friends, etc.—who does not have authority to receive the child's PHI. Pediatric providers who serve large Medicaid populations, including children's hospitals, often run into a disproportionately higher number of difficult-to-address guardianship disputes, non-traditional family structures, primary caregivers who have not received formal legal authorizations, undocumented children, or other related situations. Providing PHI access to the person with parental rights for a child who may have been subjected to abuse or neglect could cause further harm to the child and would be inappropriate. These types of situations can make proxy identification more complicated.

In addition, there are certain adolescent privacy issues where parents' involvement may need to be addressed through appropriately designed digital tools to allow for confidentiality. As we noted in our comment letters to CMS and ONC on the interoperability and information blocking rules, there are numerous privacy issues specific to children and adolescents related to application programming interfaces (APIs). In particular, APIs must have functionality allowing for the flagging of individual record components as confidential and for modifications of record accessibility to adapt to the confidentiality needs of a patient's particular situation and specific state or local requirements. States may have differing requirements and procedures related to the degree of confidentiality documentation—i.e., which types of information can or cannot be shared with family members—and the point of care when an item is labeled as such. In addition, the safety and security of downloaded health records can be a concern in pediatrics when minors do not fully understand the implications of downloading and sharing PHI. We urge the task force to recognize these unique challenges when exploring innovative digital health technologies that may be accessed and used by children and teens.

- Provider training. The need for provider training and the potential for burnout as a result of workflows is a common barrier to the adoption of digital health technologies across all settings, but particularly so in pediatrics. The lack of standardization across pediatric settings and need for work-arounds necessitates significant amounts of provider training that takes those clinicians away from directly caring for patients. As federal policies are being considered to advance digital health, it is critically important to provide training resources and funding, and to include appropriate timelines for compliance to accommodate training and implementation needs.

Goal 4: Increase access to medical innovation

Question: What can be learned from the pandemic to speed up development of novel vaccines and treatments?
There needs to be a strong emphasis on research and development specifically focused on pediatric vaccines. Those efforts must include the early (i.e., prior to the initiation of the emergency use authorization process) and ongoing input of pediatric pharmacists, providers, researchers, and suppliers who can provide their expertise regarding proper dosing, packaging, distribution, procurement and administration mechanisms appropriate for children of various ages. Mechanisms to solicit pediatric input must be coordinated across sectors to reduce duplication and fragmentation that could lead to errors in dosing, vaccine vial sizes, dilutions and related packaging and possible safety issues for children, as well as delays in access to needed vaccines.