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August 2, 2024

The Honorable Diana DeGette
United States Congress
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Larry Bucshon, M.D.
United States Congress
2313 Rayburn House Office Building
Washington, DC 20515

Dear Congresswoman DeGette and Congressman Bucshon:

On behalf of the nation's 200+ children's hospitals and the patients and families we serve, thank you for the opportunity to respond to your request for information on this iteration of the 21st Century Cures initiative. We appreciate your efforts to ensure the nation is better prepared to modernize research, development, coverage, and access to life-saving therapies for all Americans and encourage you to prioritize the distinct needs of children—who represent 22% of the total U.S. population. We urge you to invest in pediatric-focused research and advance the development of targeted strategies and guidance that can ensure children, teens and young adults get timely access to pediatric-specific health care.

As you consider the elements of Cures 2.0 that are essential for further progress, we strongly urge you to consider the pediatric-specific implications for innovative research and development of curative and life improving therapies for America's children. We recommend that:

- Pediatric-focused innovations for Long COVID are developed, appropriately reimbursed, and available to meet children's unique needs—including existing research efforts at children's hospitals.
- The national response strategy for future pandemics—including data sharing, administration of vaccines and therapeutics, and medical supply readiness—appropriately addresses the physical, mental and developmental needs of children.
- A cell and gene therapy regulatory framework that supports the development of these therapies addresses market challenges specific to pediatrics by incentivizing research and development, streamlines the approval processes, and ensures equitable access for child patients, regardless of payer or the patient's residence.
- States be incentivized to advance policies and resources that level the telehealth playing field across the country for children, including modalities that support behavioral health services.

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 to provide the necessary care and who are compassionate and able to treat kids of all ages and from all backgrounds. It is critical that pediatric-focused innovations are developed, appropriately reimbursed, and available to meet children's unique needs.

Children's hospitals 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. Children's hospitals are hubs of pediatric medical research, exploring a wide range of issues in child development and children's health conditions, driving groundbreaking innovation in pediatric

care. 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 or who have complex medical conditions.

Below please find our detailed recommendations on specific provisions of Cures 2.0 that are most relevant for children's hospitals.

Sec. 101. Further Understanding the Implications of Long COVID

We strongly support efforts to advance pediatric Long COVID research that will inform health care providers about its unique impacts on children and help ensure that children and teens have safe and effective treatments and care. Nearly 6 million children and adolescents in the country are affected by Long COVID, but because so little is known about the wide variety of symptoms that affect these pediatric patients over time—even less than adults who have been diagnoses with Long COVID—diagnosis remains difficult. **As Congress works to bolster Long COVID research at NIH, we recommend including clear directives to ensure a significant focus on pediatric care and expertise.**

To best support the advancement of pediatric Long COVID research and treatment, it is essential to build on the foundation of the current work at NIH and at children's hospitals across the nation. The RECOVER initiative at NIH, which has enrolled more than 20,000 patients, including pediatric patients, into cohort studies is funding critical research while facilitating data sharing between grantees. Several children's hospitals are already active participants in the RECOVER Initiative that follows children and young adults from birth through age 25 for up to four years and collects data on the severity of disease, clinical impact, and the effects of vaccination and treatment. Any new research initiatives on Long COVID must both build on the important work of the RECOVER Initiative and prioritize addressing the unique physical and mental health, as well as the developmental, needs of children.

Long COVID symptoms can be very disruptive for pediatric patients and their families, negatively impacting normal activity in school, sports or other hobbies, leading to harmful consequences for their long-term well-being. In January of this year, the Senate Finance Committee held a hearing, on Addressing Long COVID: Advancing Research and Improving Patient Care, which featured the testimony of a parent whose child has struggled with Long COVID and is receiving treatment and support from their children's hospital. The testimony powerfully makes the case for greater investment in pediatric medical research focused on treating Long COVID in children.

Sec. 102. National Response and Testing Strategy for Future Pandemics

We urge Congress to strengthen the nation's pandemic preparedness and response strategy by addressing the unique physical, mental, developmental, and social needs of all children, including those with complex medical conditions. Initiatives focused on strengthening the data sharing infrastructure, administration of vaccines and therapeutics, and medical supply readiness to mitigate future pandemics and public health emergencies must meet children's needs. **The importance of pediatric-specific efforts has been made more evident over the last few years, as children's hospitals have experienced unprecedented volumes driven by a series of public health emergencies, including a substantial increase in childhood respiratory illnesses like respiratory syncytial virus and the ongoing crisis in mental health patients**.

During the recent public health emergencies, it has become evident that data reporting does not provide an up-to-date representation of hospitals' pediatric capacity**.** Congress should collaborate with a range of stakeholders to develop requirements for the administration to build out a national data infrastructure capable of efficiently sharing important public health information, including pediatric-specific information, among providers and federal, state, and local agencies. We suggest standardizing the collection of pediatric data and their public displays from state to state. Further, a real-time surveillance data network that includes available pediatric data (from hospital inpatient, outpatient and emergency departments, as well as school settings) is needed. That network must have the capability to connect data with other already existing data resources for better insight into pediatric cases. Our hospitals work in partnership with national organizations to address readiness and

response to pandemics and can provide useful data elements to support pediatric situational awareness of surges and coordination across state lines.

In addition, we ask Congress to address the distinctive needs of children and the pediatric health care system in the procurement of countermeasures and qualified pandemic or epidemic products, such as vaccines. Soliciting pediatric expertise on the development and distribution of vaccines must be coordinated across sectors to reduce duplication and fragmentation that could lead to errors in vaccine vial sizes, dilutions and related packaging and possible safety issues for children, as well as delays in access to needed vaccines. We must ensure pediatric vaccines and drugs are available with proper dosing, and that the pediatric supply chain can sufficiently provide timely access to needed supplies, equipment and medications. Formal linkages and communication systems must be established and used between key federal agencies—such as the Administration for Preparedness and Response and Food and Drug Administration (FDA)—and pediatric providers to ensure that the needs of children and their families are identified and incorporated into the planning and development of medical countermeasures.

Sec. 103. Pandemic Preparedness Rare Disease Support Program

Congress should work with stakeholders in developing public health emergency preparedness and response programs that assess how well hospital and other volunteers will be able to care for children with complex medical conditions, including those with rare diseases. **Children with chronic and complex conditions need ongoing and specific medical care, may need long-term care services, and need support from caregivers**. They also have associated or co-occurring mental health needs at an increasing rate.

In light of the nation's children's mental health emergency, it is especially critical that we ensure that children's behavioral health needs, as well as any medical needs are met to best support their health and their families during any type of emergency or disaster. We know that children with rare diseases and complex medical conditions who live through an emergency have a greater risk of having traumatic experiences. When families struggle to find mental health care, children are at greater risk for experiencing long-term impacts on their health and wellbeing.

Furthermore, pandemic preparedness plans should include mechanisms to allow for the continuation of key pediatric services in the community, including those geared towards children with medical complexity or rare diseases, such as immunization programs, child nutrition programs, and well-child services.

Sec. 203. Increasing Diversity in Clinical Trials

We urge Congress to work with the FDA to prioritize and expand pediatric participation in clinical trials. **The scarcity of clinical trials for children further delays an already lengthy approval process for new pediatric medications. Safety and efficacy information on many medicines for many childhood diseases are lacking,[1] and assessing the impact on the long-term health of children as they become adults is difficult.** The medications advanced from these studies must reflect children's physical and mental health needs throughout their development, while also taking into consideration the needs of medically complex children.

It is critical that the FDA create a data reporting framework to provide an accurate picture of child enrollment in clinical trials. We recommend that FDA be required to use this data to determine the most effective strategies to expand pediatric participation and ensure that children are appropriately represented in research. To that end, we ask you to work with the FDA to engage pediatric academic medical centers and existing clinical trial networks to identify areas of unmet need and determine the feasibility of potential study designs. In addition, pediatric clinical trial data should be broken down by pediatric

[1] Clinical trials in children - PMC (nih.gov)

subgroup (e.g., neonates, infants, children, adolescents, etc.) to provide a more complete picture of which populations of children are underrepresented.

Furthermore, we recommend the development of more effective recruitment and retention strategies based on a more complete understanding of how many, and which, children are enrolled in clinical trials. If health care providers rely primarily on data collected from adult clinical trials to guide their care for children, adverse outcomes are more likely to occur. The inclusion of children of all ages in clinical trials will ensure the development of age-specific therapies and interventions to provide the best medical treatment and long-term health outcomes.

Sec. 302. Grants for Novel Trial Designs and Other Innovations in Drug Development

Congress should prioritize the development of life-saving pediatric medications that have the potential to reduce the need for costly chronic care and eliminate health disparities for children. **Children require specialized medications and therapeutics because they are constantly growing and developing, and they react to medications differently than adults.** Pediatric care relies on uniquely formulated drugs to support proper pediatric pharmaceutical dosing, as well as practical methods for appropriate medication delivery (such as oral, pediatric auto-injectors, etc.). Due to the specialized nature of these therapies, fewer innovative drugs are available for children and many of their medications are prescribed and used off-label. The difference in approval timelines between an adult and pediatric label is nearly a decade for some products, with the need especially pronounced in neonatal intensive care. [2]

We encourage Congress to work with a broad set of stakeholders—including pediatric providers, health care associations, government agencies, manufacturers, and payers—to identify mechanisms to establish the appropriate investments in research for pediatric drug development. Public-private partnerships are essential to the development of new therapies for children, especially those aimed at treating rare pediatric diseases. Funding from government sources, such as the NIH, 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.

Sec. 303. FDA Cell and Gene Therapy

Cell and gene therapies have the potential to drastically improve the life trajectory for children, adolescents and young adults, so we ask Congress to work with the FDA and other agency stakeholders on appropriate mechanisms to address foreseeable challenges on cell and gene therapy regulation relating to pediatric health care. This regulatory framework must balance investments in research and development, manufacturing, distribution, and reimbursement structures for cell and gene therapies that meet the unique needs of children.

It is critical that Congress strengthen and stabilize equitable access to cell and gene therapies, particularly those that can be used to treat pediatric conditions. Cell and gene therapies 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 for so many Americans, especially children.

In order to improve patient access to these therapies, we recommend that Congress look for ways to measure the impact of reimbursement policies, 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 children's hospitals related to the administration of a million-dollar drug to a child when

[2] I-ACT for Children: helping close the gap in drug approval for adults and children | Pediatric Research (nature.com)

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.

Sec. 402. Strategies to Increase Access to Telehealth under Medicaid and Children's Health Insurance Program

We strongly encourage the inclusion of the *Telehealth Improvement for Kids' Essential Services (TIKES) Act* in future legislation to provide guidance to states on effectively integrating telehealth into their Medicaid program and Children's Health Insurance Program (CHIP). **Telehealth has played a critical role in addressing some of the constraints that children and their families face accessing care due to geography and other barriers to care—particularly in rural and other underserved areas.** It also has allowed children with special health care needs or complex conditions, including technology-dependent children, to forgo long and complicated trips to one or more facilities and to connect with providers located outside of their home state. As a result, patient and family satisfaction has increased, and they can engage in care more efficiently.

Furthermore, Congress should examine opportunities to encourage and incentivize states to promote telehealth access and advance national Medicaid policies that level the telehealth playing field across the country for children. We encourage you to work with states and CMS to sustain telehealth policies implemented during the COVID-19 pandemic by advancing information about state Medicaid supports, incentives and learnings to encourage more widespread and high-quality use and adoption of telehealth for children. In particular, policies that allow for parity of telehealth coverage and reimbursement, including telehealth facility fees for clinical support services and infrastructure will help sustain telehealth access moving forward. It is essential that Medicaid policies expand and protect children's access to tele-mental behavioral services using video or audio-only modalities.

Sec. 403. Extending Medicare Telehealth Flexibilities

Recognizing the interconnectedness of Medicare and Medicaid policies, The *Telehealth Modernization Act* sets an important precedent that could impact Medicaid coverage and reimbursement structures. By addressing barriers to telehealth adoption in Medicare, such as the six-month in-person requirement, licensure restrictions and originating site requirements, this bill paves the way for similar reforms in Medicaid. We believe this bill's efforts to advance telehealth flexibilities will allow the children and families we serve, particularly in rural and underserved regions, to continue to have access to high-quality, patient-centered healthcare.

In particular, we support the bill's removal of geographic restrictions that will further allow broader access to telehealth for children. During the pandemic, states chose to expand the originating site, or the site where the patient is located, to allow for the patient's home to be considered an eligible originating site during the pandemic. Before the pandemic, patients might have been required to travel to a health care facility for care via telehealth. Similarly, the distant site was expanded to include the clinician's home, allowing these clinicians to provide care while required to self-isolate or socially distance due to exposure to the coronavirus or increased risk.

In addition, we support this bill's repeal of the six-month in-person requirement. Removing this barrier would notably improve access to telehealth services for children and youth, especially for tele-mental health services, ensuring equitable healthcare access regardless of location.

Sec. 407. Expanding Access to Genetic Testing

To expand and improve access to genetic testing, we urge Congress to work with CMS and pediatric stakeholders to evaluate the impact of reimbursement policies, particularly for children covered by Medicaid, and analyze how payment policies, utilization management, and state-specific coverage restrictions affect timely access.

Strengthening equitable access to genetic testing, especially for pediatric patients with rare diseases, is critical and could be lifesaving or life-sustaining for children**. Genetic testing, including rapid whole genome sequencing, can significantly improve diagnostics, enabling tailored treatments to treat pediatric patients more effectively**. However, the high costs of these tests, coupled with state policies that do not consistently cover them, make them cost-prohibitive and reduce access for the sickest children. The solution should ensure reasonable costs and broader coverage for pediatric genetic tests, balancing investments in research, development, testing, and reimbursement.

Sec. 501 Advanced Research Projects Agency for Health

We were pleased to see the launch of the Advanced Research Projects Agency for Health (ARPA-H), which operates with its own unique research goals and processes within the NIH. As you develop the next iteration of the CURES legislation, we encourage you to engage with ARPA-H, as well as pediatric researchers and institutions to identify any barriers which may inhibit the inclusion of pediatric conditions and patients in the new agency's work.

In exploring opportunities with ARPA-H, children's hospitals have found that the grant application process necessitates a higher level of risk-taking to deliver innovations that can be commercialized. These requirements may pose challenges for pediatric hospitals that lack the required infrastructure for translational research, making it difficult to move investigation research past critical development stages. Therefore, we believe ARPA-H's potential can be enhanced by fostering collaborations between pediatric hospitals and universities with strong translational research capabilities. Encouraging these partnerships in the CURES legislation will ensure pediatric research thrives within ARPA-H initiatives.

Other Pediatric Recommendations

In addition to the specific Cures 2.0 provisions above, we urge Congress to prioritize child health interests in the NIH's clinical research programs and ensure a robust pipeline of early-stage pediatric research talent. We believe that efforts to strengthen pediatric-specific research and researcher workforce development will complement and build upon the existing Cures initiatives.

National Institute on Child Health and Human Development

The NIH, particularly the Eunice Kennedy Schriver National Institute on Child Health and Human Development (NICHD), is a critical funder of the pediatric medical research being conducted by children's hospitals across the country**. It is essential that we continue to invest in and support the NICHD—the only federal entity to solely focus on pediatric research—to bolster and expand its reach**. We are concerned about recent proposals to restructure the NIH. NICHD is a critical part of developing life-saving cures for children and we support its continuance as a separate institute at NIH. We urge you to include NIH grantees that are conducting pediatric research, including those at children's hospitals, in any discussion of reforms to NIH.

Support for the *All of Us* Program

We strongly recommend that Congress urge the NIH—and provide the needed funding in a timely manner—to finalize recruitment protocols for the *All of Us* Research Program to implement a multi-phase program geared to children. Unfortunately, the 21st Century Cures Act decreased funding for the *All of Us* Research Program, thereby preventing and delaying pediatric recruitment. **It is critical that the unique developmental and physiological needs of children are**

included in studies and clinical trials across the NIH, including the *All of Us* program. Failure to recruit children into the program in numbers that are proportional to the nation's population could lead to missed scientific opportunities, not only for children but for lifespan research.

As you know, the *All of Us* Research Program aims to recruit participants into one of the most diverse health databases ever created, to enable researchers to study how our biology, lifestyle and environment affect our health. For this database to be truly representative and to enable the study of health throughout the lifespan, the NIH must actively recruit all age groups, including children and adolescents. We recommend that Congress and NIH work with children's hospitals, given that they are regional centers for groundbreaking medical research into pediatric health conditions and life-saving treatments, on recruitment and other aspects of the child-focused components of *All of Us*.

Early-Stage Pediatric Researchers

We were pleased that the 21st Century Cures Act recognized the need for the NIH to better support early-career pediatric researchers and urge you to ensure a focus on this population as part of any Cures 2.0 bill. **Specifically, we recommend that you include a career development section that incorporates the bipartisan Pediatricians Accelerate Childhood Therapies Act (H.R. 4714).** This bill would create a new training model focused explicitly on promising early-career researchers focusing on child health, including those from backgrounds under-represented in pediatric research. Including this bill would be a tangible way to infuse training dollars into child health research training programs, helping reverse declines that have occurred over the past several years.

Thank you again for the opportunity to provide feedback on the next iteration of the 21st Century Cures initiative. We look forward to working with you to ensure the needs of children are met when addressing efforts to advance research and development. Please contact Cynthia Whitney at Cynthia.Whitney@childrenshospitals.org, (202) 753-5328, or Natalie Torentinos at Natalie.Torentinos@childrenshospitals.org, (202) 753-5372 should you need more information.

Sincerely,

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