March 24, 2021

Representative Diana DeGette
2111 Rayburn House Office Building
Washington, DC 20515

Representative Fred Upton
2183 Rayburn House Office Building
Washington, DC 20515

Dear Representatives DeGette and Upton:

We are writing to applaud your outstanding efforts in the creation of 21ST CENTURY CURES 2.0 and express our support for its advancement. The Children's Cancer Cause (CCC), established in 1999, was founded to ensure the needs and perspectives of children with cancer, survivors and their families are integrated into federal health care, research, and cancer policy. Childhood cancer remains the leading cause of death by disease for our nation's children, and the well-being of the more than 500,000 survivors transitioning into adulthood remains uncertain. Two-thirds of childhood cancer survivors suffer from at least one severe health problem, including heart and lung damage, second cancers, osteoporosis and hearing loss. Many are unaware and unprepared to face a lifetime of impaired health and quality of life.

We believe 21ST CENTURY CURES 2.0 addresses many of the needs of the pediatric cancer community and are pleased to share our comments and responses to the questions posed in the framework document. Our comments below are embedded in the sections specific to the draft of CURES 2.0.

TITLE 1: PUBLIC HEALTH

Section ___: COVID-19 Rare Disease Support Program

This policy would help financially vulnerable individuals with rare diseases and their families by providing financial assistance for COVID-19-related expenses. By providing funds to a "COVID-19 Rare Disease Support Program" to help them with these unforeseen expenses, Congress could help ensure these individuals receive the care and support they need.

- ***CCC strongly supports this program. Financial impediments and hardships are a significant barrier for those with childhood cancer and COVID likely exacerbates this issue.***

Section ___: Improving U.S. Pandemic Preparedness and Response through Support of Antimicrobial Resistance Product Commercialization

This provision would provide the Secretary of Health and Human Services, acting through the ASPR, the resources and regulatory authorities necessary to fix the commercial market for new antibiotics.

- ***Children with cancer depend on antibiotics during treatment and recovery from acute toxicities of their chemotherapies. Ensuring a stable commercial market for these medicines is critical for their survival and well-being.***

TITLE II: CAREGIVER INTEGRATION

Section ___: Educational programs and training for caregivers

This provision would authorize grants for educational programs and training for caregivers to learn skills which would allow them to augment a care team and complement, not compete with, a clinical visit. These educational programs would include specialized training in medication adherence and injections, complementary strategies to ensure adherence to physical and occupational therapy regimens, nutritional compliance, and other services provided in the home.

- ***CCC supports education and training for caregivers. Parents bear enormous burdens in caring for their children at home during cancer treatment and afterwards, as they manage the long term and late effects of their disease and therapies. Parents often need to leave jobs for the short or long term to assist with care, may need to relocate to find appropriate cancer care and appropriate transitional care from the treatment phase to primary care.***

TITLE III: PATIENT ENGAGEMENT IN HEALTH CARE DECISION MAKING

Section ___: Increasing Health Literacy to Promote Better Outcomes for Patients

This provision would require CMS to issue an RFI regarding ways the agency can work with federally subsidized health care program stakeholders to encourage and promote greater health literacy of individuals.

- ***Americans are living longer with cancer, resulting in larger numbers of cancer survivors in the United States. The nation's 500,000 pediatric cancer survivors are uniquely affected because of late effects of cancer treatment. Over 80% of childhood cancer survivors will have at least one severe, disabling, or life-threatening late effect of their disease or treatment by the time they reach 45 years of age. At the conclusion of active treatment, survivors should receive information that includes a summary of their treatment, potential risk for late effects that are associated with their treatment, and recommendations for follow up care. We believe attention to survivorship care, which is often not covered by insurance, is an essential component of improved health literacy. A recent AHRQ report likened impediments to getting survivorship care as a system inequity because childhood cancer survivors often lack literacy about their needs when they leave the active phase of cancer treatment.***

TITLE IV: CLINICAL TRIALS

Section ___: Diversity in Clinical Trials

Under this policy, HHS would be required to convene a task force on making clinicaltrials.gov more user- and patient friendly. This task force must include NIH, FDA, academic researchers, and patient organizations to focus on the administrative burden and utility of this database for all stakeholders.

- ***CCC supports this goal. We also support increased information included in clinical trials.gov about whether childhood cancer drug trials for drugs already approved for adults or are investigational and being evaluated first in children. Such information might help inform academia or other sponsors to further develop promising new therapies for pediatric cancers.***

TITLE V: FDA

Section ___: FDA Grant-making Authority and Funding

This provision would authorize funds to enable the Food and Drug Administration to provide grants around innovative clinical trial design and patient focused drug development to further build the science in these areas. Included in innovative clinical trial design is complex adaptive, Bayesian, and other novel clinical trial designs. In the area of patient-focused drug development, further study on clinical outcome assessment and patient experience data and incorporation of digital health tools and real-world data/evidence should be prioritized.

- ***Children affected by cancer face unique needs and challenges. Therapies better targeted at children's cancers can increase survival rates and reduce late effects. For more than twenty years, Children's Cancer Cause (CCC) has been working to help speed drug development and achieve access to less toxic and more effective pediatric cancer therapies.***

    - ***CURES 2.0 could develop an option category of standards for the conduct of clinical trials for rare cancers that could lead to Breakthrough Therapy Designation or Approval. Sponsors should be encouraged to pursue this pathway if the data are promising.***

    - ***FDA makes its views on drug products and classes of drugs available through guidance documents, recommendations, and other statements of policy. Improved guidance in this area could include: (1) novel trial design; (2) definition of comparison and control groups; (3) mechanisms for facilitating research partnerships with industry and academia; and (4) use of RWE available through patient registries, other academic databases or electronic health records.***

Section ___: Increasing Use of Real-World Data/Evidence

(RWE in Breakthrough and Accelerated Approval): Congress established the Breakthrough Therapy Designation (BTD) in 2012 to expedite the development and review of drugs with substantial preliminary clinical activity that treat serious conditions with a large unmet medical need. At the same time, Congress expanded the Accelerated Approval pathway to allow drugs for serious conditions that filled an unmet medical need be approved based on an effect on a surrogate endpoint or an intermediate clinical endpoint that is reasonably likely to predict drug's clinical benefit. These policies have been a tremendous success, and we believe that further incorporating RWE could help build on that success.

- ***Real world evidence suggests that companies either do not want to or cannot conduct post-approval studies. Post approval studies are especially important for children because of the impact of therapies on normal development and on health throughout adulthood. Research by the NCI-supported Childhood Cancer Survivors Study has carefully documented the late effects of chemotherapy drugs, but discussion is just starting about the need to study the late effects of 'precision oncology treatments', including targeted, cell and immunotherapies. New approaches to address in this emerging need for follow up data are critical to understand the safety and efficacy of these new cancer treatments for children.***

TITLE VI: CMS MODERNIZATION

Questions to Support Possible Policy Development

1. **General Coverage Modernization**

- Are current coverage and reimbursement rules for new medical products under federally financed health programs that are outdated or in need of reform? If so, what are they?

    - ***CCC believes that if cancer drugs are approved for adults and are part of clinical trials for children, the cost of these drugs should be covered by Medicaid. Often, insurance denies reimbursement because drugs are not approved as treatments for pediatric cancer despite the fact that they are critical to the pediatric clinical trial protocols. Such denials create hardship and barriers for children and their families at their greatest time of need.***

- Are the current coverage and reimbursement approaches to new medical products or other modern technologies adequate to keep up with the pace of innovation? If not, why?
- What barriers and issues exist for patients who transition from private insurance to Medicare?

    - ***As stated earlier, CCC believes survivors experience significant challenges as they transition from acute cancer care to primary care. Survivors have surveillance and diagnostic needs (such as mammography scanning, EKGs, and other testing) during care transitions and throughout their young adulthood - testing that patients without a cancer history may not be eligible for. Public programs do not adequately cover the cost of these services.***

2. **Cell and Gene Therapies**

- Are there barriers that impede or otherwise slow coverage for new cell and gene therapy products? If so, what are they?

    - ***These new therapies are very costly and out of reach for many families of childhood cancer patients. New payment models are sorely needed for these life-saving therapies.***

- Are there improvements that can be made under federally financed health programs to improve coverage and patient access of these therapies?

3. **Medical Products for Small Patient Populations**

- What are the biggest impediments to new cures development for these important populations? What steps can policymakers take to address these impediments if any?

    - ***Because there is little economic incentive, biopharmaceutical companies currently do not develop drugs to treat rare cancers in children or cancers that occur only in children. Legislative mechanisms could address this gap through the creation of a public private***

partnership to assume responsibility for developing drugs when there is no associated adult cancer indication to generate revenue. A public private partnership to develop drugs for childhood cancers could model ACTIV, the public private partnership to develop Covid vaccines. and ensure adequate funding for to develop new precision medicines to treat pediatric cancers.

4. **Genomic Sequencing**

- Are there barriers that impede or otherwise slow coverage of genome sequencing?

- Are there improvements that can be made under federally financed health programs to improve coverage and patient access of genomic sequencing?

    - ***Genomic tests used to make cancer treatment decisions for children are not covered by insurance. Additionally, tests used in determine germline sequencing are also not paid for unless they are part of a research study.***

5. **Breakthrough Coverage**

- Are there barriers that impede coverage of technologies and therapies approved through FDA's breakthrough technologies and therapies pathways?

- How do we expedite coverage while at the same time ensuring that additional evidence can be collected?

    - ***We ask that CURES 2.0 address the burden of incidental costs of clinical trials participation, including transportation, lodging, and food costs. Care of children with cancer often requires family members traveling long distances for their child's treatment, taking leaves of absence, or quitting their jobs, inflicting additional financial stress. CCC urges financial support for a family caregiver as a component of clinical trial participation. We recommend these elements be considered during the CURES 2.0 discussion to strengthen the standards for clinical trials and to enhance clinical trial participation. The National Institutes of Health (NIH), and particularly the National Cancer Institute (NCI), should be charged with testing models of caregiver support in coordination with the Centers for Medicare and Medicaid Services (CMS)***

Additional Areas of Opportunity

In addition to the aforementioned comments, we believe CURES 2.0 could be enhanced by a focus in the areas of Digital Technology and Data Collection. Our comments are below:

1) Digital Technology

Most childhood cancer patients are treated at major medical institutions, often far from their homes. After completing their treatment, patients and their families return to their communities, where access to follow-up care is often limited.

- ***ONC Study: Health care information is increasingly provided to patients through a digital platform that includes portals and other means of communication. Pediatric cancer care records and care plans are a critical and sometimes missing components of follow up care. We believe better data about digital platforms for pediatric survivorship care planning is needed. Every year the Department of Health and Human Services Office of the National Coordinator for Health Information Technology (ONC) submits a mandated report to Congress on health IT progress, specifically examining the hi-tech era and the future of health IT. This annual report is submitted in accordance with the law set forth by section 3001(C)(6) of the Public Health Services Act and Section 13113(A) of the HITECH Act. We recommend that CURES 2.0 require next year's annual report to evaluate the state of digital platforms for pediatric survivorship care with a focus on integrating existing models, such as the Passport for Care.***

2) <u>Data Collection</u>

Adequate information about pediatric cancer survivorship care and resources and coverage to ameliorate late effects are limited. Data limitations and longitudinal information gaps exist in the following key areas:

- ***The way children with cancer are insured under Medicaid, group health plans or other mechanisms both for their active cancer care and survivorship care.***
- ***The number of children with cancer who receive survivorship care planning, what kind of planning, for how long, and related outcomes.***
- ***Tracking could begin with Medicaid as a first step.***

We recommend increased data collection as part of the National Childhood Cancer Survivor Study but also encourage consideration of other mechanisms to study coverage gaps.

Thank you for the opportunity to comment on CURES 2.0. The Children's Cancer Cause looks forward to working with you as this important bill moves forward. If you have questions or comments please feel free to contact me at swosahla@childrenscause.org or 703-398-7110.

Sincerely yours,

Steve Wosahla
Chief Executive Officer