



December 21, 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 on the introduction of H.R. 6000, The CURES 2.0 Act. 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.

Of particular note, we are pleased that the bill included Section 407, Precision Medicine Answers for Kids Today. This provision specifically addresses the rare disease needs of children with cancer. Genomic tests used to make cancer treatment decisions for children are not covered by insurance. Additionally, tests used to determine germline sequencing are also not paid for unless they are part of a research study. H.R. 6000 addresses this problem through a 15-state demonstration program to evaluate new coverage in this area which specifies coverage of "hereditary cancer testing in the presence of a suspected or confirmed cancer diagnosis."

We also support the inclusion of Section 501 which authorizes \$6.5 billion to create the Advanced Research Projects Agency for Health within NIH. The mission of ARPA-H will be to transform and improve important areas of medicine and health by funding projects that could overcome market failures through critical solutions and complement NIH's existing research portfolio and mission and the private sector's research initiatives. ***We hope to work with you on the creation, within ARPA-H, of a new public private partnership that assumes responsibility for conducting pediatric oncology drug development programs when such programs are not pursued by industry.*** By including such a public-private partnership in its authorizing section, the partnership could rapidly advance the development of emerging pediatric oncology therapies.

We believe H.R. 6000 addresses many of the individual needs of the pediatric cancer community and are pleased to share our specific comments below on the bill sections.

## H.R. 6000 Bill Sections

### TITLE 1: PUBLIC HEALTH

#### Section 103: Pandemic Preparedness Rare Disease Support Program

This policy would help financially vulnerable individuals with rare diseases and their families by requiring the HHS Secretary to develop a plan to help rare disease patients and families. 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 105: Developing Antimicrobial Innovations

This provision would establish a subscription model to pay for critically needed novel antimicrobial drugs. The policy contains investment in programs to address antimicrobial resistance, which is critical for patient care and public health.

- ***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: PATIENTS AND CAREGIVERS

#### Section 201: 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.

- ***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.***

## **Section 202: 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 increased patient and family caregiver health literacy.

- ***Americans are living longer with cancer, resulting in larger numbers of cancer survivors in the United States. The nation's 500,000 pediatric survivors of cancer 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 uncovered, is a 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 leaving the treatment state of their cancer.***

## **Section 203: Increasing Diversity in Clinical Trials**

Under this policy, HHS would be required to convene a task force on making [clinicaltrials.gov](http://clinicaltrials.gov) more user- and patient friendly. This section also requires a GAO study on barriers to clinical trial participation as well as an HHS public awareness campaign to increase awareness and understanding for minorities in this arena.

- ***CCC supports this goal. We also support increased information included in [clinicaltrials.gov](http://clinicaltrials.gov) about childhood cancer drug trials such as information about drugs whose pediatric potential companies decide not to pursue. Such a database might incentivize academia or other sponsors to further develop promising therapies for rare pediatric cancers.***

## **Sec. 205: Ensuring Clinical Trials Under Existing Standard of Care**

This section allows Medicare to pay routine costs when the enrollees are in some federally funded clinical trials.

- ***While the Medicare change would not directly influence commercial health coverage, it could influence coverage indirectly as Medicare changes often affect commercial carriers' coverage decisions.***
- ***We ask that HR 6000 go further and also address the unique needs of children and 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 to strengthen the standards for clinical trials and to enhance clinical trial participation. Furthermore, 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).*

### **TITLE III: FOOD AND DRUG ADMINISTRATION**

#### **Sec. 301. Report on Collaboration and Alignment in Regulating Digital Health Technologies.**

This provision requires the HHS Secretary to submit a report to Congress on the efforts to ensure collaboration and alignment across the centers and offices of the Food and Drug Administration with respect to the regulation of digital health technologies.

- *As stated earlier, 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 limited.*
- *ONC Study: Health care information is increasingly provided to patients through a digital platform that includes portals and other means of communication. To reiterate, pediatric cancer records of care and care plans are a critical and sometimes missing component of 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 the legislation 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 model.*

#### **Section 302. Grants for Novel Trial Designs and Other Innovations in Drug Development**

This provision provides grants in the area of innovative clinical trial design and patient experience data to further build the science in these areas.

- *Children affected by cancer face unique needs and challenges. Therapies better targeted at children's cancers can increase survival rates and reduce late effects.*
  - *The legislation could include a category of standards for the conduct of clinical trials 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 303. FDA Cell and Gene Therapy**

This section requires the HHS Secretary to submit a report to Congress regarding the current state of cell and gene therapy regulation and foreseeable regulatory challenges for the FDA in the future.

- ***The report should chronicle the barriers that impede or otherwise slow coverage for new cell and gene therapy products. 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.***

### **Section 304: Increasing Use of Real-World Evidence**

This provision requires HHS to outline approaches to maximize and expand the use of real-world evidence and establishes a task force to develop recommendations on ways to encourage patients to engage in real-world data generation.

- ***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.***

### **Section 407. Precision Medicine Answers For Kids Today**

This policy would increase access to diagnostic testing by providing federal support for the use of genetic and genomic testing for pediatric patients with rare diseases.

- ***As stated above, we strongly support this new language and hope to work with you on its enactment.***

## TITLE V: RESEARCH

### Sec. 501. Advanced Research Projects Agency for Health:

This section authorizes \$6.5B to create ARPA-H within NIH. The mission of ARPA-H will be to transform and improve important areas of medicine and health by funding projects that could overcome market failures through critical solutions and complement NIH's existing research portfolio and mission and the private sector's research initiatives.

- ***As stated earlier, we ask that the bill authors include language, under ARPA-H, to authorize a public private partnership. The January 2020 GAO report on pediatric vouchers made this specific recommendation: "A collaborative agreement to share development risk and reward between a public or quasi-public organization and one or more private developers." Subsequent to the January 2020 GAO report, the COVID-19 pandemic demonstrated the potential of public-private partnerships as in Operation Warp Speed's rapid deployment of several vaccine options. CCC recommends that H.R. 6000 creates a similar partnership to assume responsibility for conducting pediatric oncology drug development programs that may not be commercially advantageous for industry to develop on its own.***

### NEW SECTIONS FOR CONSIDERATION IN H.R. 6000

The bill authors should include the following new sections to address unmet needs of children with cancer.

#### **Data Collection**

We recommend creating a new provision similar to Section 411 (Ensuring Availability of Medicare Claims Data) that would promote adequate information about pediatric cancer survivorship and late effects resources when coverage is 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.***

## Medicaid Coverage for Children with Rare Diseases

- ***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.***
- ***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.***

Thank you for the opportunity to comment on H.R. 6000. 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](mailto:swosahla@childrenscause.org) or 703-398-7110.

Sincerely,

A handwritten signature in black ink, appearing to read "Steve Wosahla". The signature is fluid and cursive, with the first name "Steve" being more prominent than the last name "Wosahla".

Steve Wosahla  
Chief Executive Officer