The Congressional Childhood Cancer Caucus Presents
Progress and Opportunities in the Fight Against Pediatric Cancer
6th Annual Childhood Cancer Caucus

Friday, September 18, 2015 9:00 – 10:30 a.m. • Congressional Auditorium, US Capitol Visitors Center

Welcoming remarks:
Representative Michael T. McCaul (Texas 10th Congressional District)
Representative Chris Van Hollen (Maryland 8th Congressional District)
Representative Jackie Speier (California 14th Congressional District)

Keynote speech:
Dr. Lee Helman, Director, National Cancer Institute

Panels:
Drug Innovation for Kids With Cancer: Roger Jeffs, PhD (United Therapeutics), Amy Fowler, MD (Dell Children’s Medical Center), and Casey and Lesley Ryan (parents, Austin, TX)
A Call to Action: Michael Link, MD (Stanford School of Medicine) and Danielle Leach, MPA (St. Baldrick’s Foundation)

Opening remarks
Scott Lenfestey

The Caucus was kicked off by Scott Lenfestey—a 7-year-old 1st grader at Triangle Math and Science Academy in Cary, NC. Scott was diagnosed with acute lymphoblastic leukemia (ALL) at age 3 and completed treatment this Spring, upon receiving 3.5 years of chemotherapy at University of North Carolina (UNC) Cancer Hospital. He has been serving as a vocal, passionate advocate for the Childhood Cancer Survivorship Treatment Access and Research (STAR) Act.

Representatives Michael T. McCaul and Jackie Speier

Representatives Michael T. McCaul and Jackie Speier also gave some opening remarks prior to the introduction of the keynote speaker. Congressman McCaul went on to say these powerful words:

“I chair the Homeland Security Committee and I try to protect the American people from the threats that I see, but childhood cancer is the #1 threat to our children”.

Congressman McCaul’s interest in childhood cancer has been fostered by his interactions with parents, as well as by the loss of his childhood best friend from leukemia. He emphasized that
we have far more work to do, while extending thanks to both Andy Taylor for his behind-the-scenes-work and Nancy Goodman for her efforts with the Creating Hope Act. He reminded the audience that the Caucus was formed because children with cancer have no lobbyists in Washington. He credited the Creating Hope Act for opening up the door to develop a new drug for the treatment of neuroblastoma. To continue this momentum, Congress is being tasked with both the reauthorization of the Creating Hope Act and the passing of the 21st Century Cures Act to increase National Institutes of Health (NIH) funding. While the House has done its job, the Senate needs to act or the Creating Hope Act will expire by St. Patrick’s Day. He also noted that a cancer research institute in his home state of Texas has increased the proportion of funding that it directs toward childhood cancer from 3% to 31%. He sees the 4% of funding from the NCI as unconscionable, hoping that the NCI will follow the lead of Texas. He concluded by noting the sadness that is inherent to childhood cancer, remarking that

“We need to stop it. We will continue to fight and eventually defeat this terrible disease.”

Congressman McCaul then introduced Congresswoman Jackie Speier, who started out by acknowledging how incredibly dysfunctional Congress has been and that people are troubled by the inability to get things done. She emphasized the need to support the STAR Act and increase NIH funding for cancer research—with a sense of indignation that we have not done these things already.

“Shame us into being functional”, said Congresswoman Speier, who also indicated that the White House will be ready to sign the legislation once it comes out of the Senate.

Following the Keynote presentation, summarized below, Congressman Van Hollen thanked the group, extending special thanks to those who have lost children to cancer. He went on the praise the Creating Hope Act for creating more hope and tangible benefits. He is very proud of United Therapeutics, which is located in his Congressional district, while also noting that Rex Ryan (a recipient of their new product for neuroblastoma) is in Congressman McCaul’s district.

“What we know is that research saves lives, but childhood cancer remains the #1 taker of kids’ lives”, Congressman Van Hollen remarked, before reiterating the importance of the 21st Century Cures and Creating Hope Acts.
Dr. Lee Helman started off by highlighting the National Cancer Institute’s (NCI) mission, to allow patients to live longer, healthier lives. While we have been making progress, Dr. Helman emphasized that “it is not time to take our foot off of the accelerator”.

Dr. Helman reviewed details regarding the NCI’s budget proposal and priorities for fiscal year 2017. The NCI is asking for an additional $355 million to study basic science, including understanding causes of cancer, genomics, and the immune system. They have also requested $205 million for bringing research to the public, which includes funding for Precision Medicine Initiative clinical trials as well as the Pediatric MATCH trial. Dr. Helman is hopeful that the Pediatric MATCH trial will open in 2016. He also emphasized the importance of basic science and continued funding that will be critical to advancing the detection, treatment, and prevention of childhood cancer.

Dr. Helman reiterated that “we have made progress”, noting that while the incidence of childhood cancer has gone up, mortality has gone down. Basic research has given unprecedented hope for improving outcomes of childhood cancer. As an example of the benefits of investing in basic research, an immunotoxin (moxetumomab pasudotox) is being studied in a randomized trial in ALL. Additionally, although using the immune system to fight cancer was once regarded as a fantasy and a dream, CD19-targeted chimeric antigen receptor (CAR) T-cell therapy is now having the biggest impact to date in pediatric ALL. A large Children’s Oncology Group (COG) study is planned for the immune checkpoint inhibitors, which are revolutionizing the treatment of melanoma, lung cancer, and bladder cancers in adults. Dr. Helman is confident that the COG will work out the ways to implement this trial in pediatric patients. Other strides are being made in the field of neuroblastoma, as evidenced by the approval of dinutuximab (monoclonal antibody ch14.18) for high-risk patients and the identification of a role for ALK rearrangements in some cases, which can be targeted by the drug crizotinib (approved for ALK-positive lung cancer).

Overall, “10 years of basic science is bearing fruit”, remarked Dr. Helman.

Some of the foundation for CAR T-cell therapy was in fact developed while studying fundamental molecular biology. Regarding the immune checkpoint inhibitors, their development represents 20 years of basic science focusing on how the immune system works. These agents are being actively studied in pediatric patients.

Dr. Helman went on to highlight the NCI Pediatric Preclinical Testing Consortium (PPTC), which is in place with the goal of finding more drugs with activity against childhood cancer at an
earlier point in their development. The NCI PPTC consists of a Coordinating Center as well as 5 research programs that perform in vitro testing of pediatric anticancer drug candidates.

It could be said that “we are at the beginning of a modern medical success story for childhood cancer”, or that we are at the end of the beginning but nowhere near the end.

There are several problems at hand and that need to be addressed moving forward:

- **Plateau in survival rates**—In the past 16 years, there have been no major improvements in mortality rates despite the increased intensity of treatment. The survival curve has been flattening, as researchers have been struggling to continue to achieve further success.

- **Survivorship**—Children who are cured from cancer continue to pay a tremendous price in terms of so-called late effects, including heart or lung disease, other health issues such as loss of fertility and cognitive declines, and secondary primary cancers. Overall, the treatments are now known to greatly accelerate the aging process. In examining cause-specific mortality among aging survivors in the NCI Survivorship Study, it is apparent that while the highest risk of death occurs from recurrent or progressive tumors in the early period from diagnosis, non-recurrence issues become a bigger threat after 20-25 years of follow-up. More specifically, childhood cancer survivors who are older than age 40 have a 2-fold increased risk of developing a second cancer and a 7-fold increased risk of cardiac-related death. Additionally, the more we look at the issue of long-term toxicity over a 25-year period, it is increasingly evident that most patients are left with side effects that affect them on a daily basis. “**We need to do better**”, Dr. Helman remarked.

Dr. Helman then shifted his talk more toward proposed solutions, those that form the basis of the Precision Medicine Initiative. For most of its 70-year history, systemic cancer treatment has relied on drugs that are marginally more toxic to malignant cells than to normal tissue. At the same time, molecular markers to predict benefit or understand therapeutic resistance in the clinic have usually been lacking. The proposed solution to these problems would be to use genomics to identify and target molecular vulnerabilities of individual cancers. This would include immunotherapy approaches, such as the manipulation of T cells.

Dr. Helman explained that better biomarkers, drivers, and therapeutic targets need to be identified:

- **Biomarker**—characteristic that is objectively measured and evaluated as an indicator of diagnosis, prognosis, normal biologic processes, pathogenic processes, or pharmacologic responses to a therapeutic intervention
- **Driver**—genomic alterations causally implicated in oncogenesis or tumor survival. Such mutations have been positively selected during carcinogenesis and often show a recurrent pattern within or across tumor types. Passenger events arise from the background mutation rate and do not contribute to oncogenesis.
- **Therapeutic target**—a molecule/protein differentially expressed in a tumor which can be used to hone in lethal therapy or when it (or its downstream molecules) is inhibited leads to tumor growth inhibition or regression or death.

Many novel drivers have been identified in recent years, yet many are not currently druggable; examples include *EZH2* and *MEF2B* in lymphomas, *DNMT3A* in leukemias, *H3F3A* and *HIST1H3B* in diffuse intrinsic pontine gliomas (DIPG), and *ATRX, ARID1A,* and *ARID1B* in neuroblastoma. He noted that he was showing an old slide, in which *IDH1* and *IDH2* were shown as non-druggable targets but are now in fact druggable/actionable targets.

Once again, he remarked that “we have to keep our foot on the accelerator”.

One issue that needs to be appreciated is that mutations in pediatric tumors are rare relative to adult tumors. Dr. Helman showed a slide of somatic mutation rates across cancers, with the lowest rate found in neuroblastoma and the highest in melanoma. “We need to keep looking”, he added.

There are several barriers to individualizing therapy in pediatric patients, including not only the low mutation rate but also that most of the variants are in non-protein coding regions (98% of the genome), with existing limitations in our knowledge of the biology of these changes. We are also faced by (1) mutations in regions of known target genes with unknown significance, some of which have been previously uncharacterized, and (2) extensive diversity between individuals and within different tumors of the same individual. Lastly, when patients are treated, new mutations are known to arise (editorial note: this occurrence of new mutations is not specific to pediatric tumors, also occurring in adult cancers).

Nonetheless, the NCI is excited about the MATCH trial, a precision medicine trial that is exploring the concept of treating patients based on the molecular profiles of their tumors. The pharmaceutical/biotech industry is providing about 30 drugs to be tested in adults. The NCI is working hard with COG to launch the similarly designed Pediatric MATCH.

As the presentation drew toward a close, Dr. Helman took the opportunity to highlight a recent Specialized Program of Research Excellence (SPORE): The Developmental and Hyperactive Ras Tumor SPORE. Awarded in 2015, this is the first SPORE grant that is non-organ specific and instead targets a pathway: hyper-activated RAS in the context of neurofibromatosis type-1 (NF-1). In addition to the co-principal investigators Drs. Kevin Shannon and Wade Clapp, as well as
NCI intramural collaborator Dr. Brigitte Widemann, the SPORE will include a project focusing on secondary cancers—led by pediatric oncologists Drs. Smita Bhatia (University of Alabama) and Jean Nakamura (UCSF).

Dr. Helman concluded by summarizing what he sees as the future of pediatric cancer trials, with precision individualized therapy of relapsed or refractory solid tumors. In this regard, treatment will be based on molecular diagnosis (amplification, mutation, translocation, overexpression, or altered sliced gene), not histologic diagnosis, to identify activated pathways to be targeted by individualized combinational therapy.

*As part of the Q&A, members of the audience asked questions pertaining to the prioritization of tumors and the current NCI allotment of 4% to childhood cancer research. Here is a synopsis:*

Per Dr. Helman, we are not doing enough for some tumors, a key example being DIPG. In DIPG, one of the problems is the difficulty in obtaining samples for genomic studies. However, we need to keep pushing, as the outcomes are unacceptable. There is far more work to be done to understand the basis for this and other diseases.

When Congressman McCaul inquired about the 4% figure, Dr. Helman responded that he would love for it to be 100% because of his career focus on childhood cancer. He noted, however, that the 4% figure is “tricky” as it does not account for broader research efforts, such as those related to understanding the immune system—which apply to both adult and childhood cancer.

**Panel Discussion**

The panel was introduced by Gavin Lindberg, who thanked the Congressional Leadership and explained that his involvement in childhood cancer is due to personal reasons. Nine years ago to the day, his 3 year old son Evan had been diagnosed stage IV neuroblastoma, which led to a brutal and heart-wrenching struggle that defined courage and strength before he passed away in October 2010. The odds were in favor of the neuroblastoma instead of his son, said Gavin.

The panel discussion was kicked off by Casey and Lesley Ryan, whose son Rex (diagnosed with stage IV neuroblastoma at age 17 months) received United Therapeutics’ dinutuximab as part of a clinical trial and is about to celebrate 1 year of remission, after 7 rounds of chemotherapy, a stem cell transplant, 12 sessions of radiation, and 6 rounds of immunotherapy. Leslie read an emotional blog post that she had written, about becoming a member of the club of parents of children with cancer; it can be found here [http://posthope.org/rexstrong/journal/187037/our-club](http://posthope.org/rexstrong/journal/187037/our-club) Both Casey and Lesley expressed their gratitude for the Creating Hope Act and the efforts of the NCI, United Therapeutics, and Rex’s oncologists and care team.
The next speaker was Dr. Roger Jeffs, PhD, President and Co-CEO of United Therapeutics, who discussed “Drug Innovation for Kids With Cancer”. Dr. Jeff agrees that it is a new beginning, and that we are in a defining time as it pertains to advancing childhood cancer research. He started off by reviewing the “Circle of Partnership”, the 5 key groups that are required for effective collaboration:

- Medical and Scientific Community
- Food and Drug Administration (FDA)
- Legislature
- Pharmaceutical industry
- Advocacy

“The science of cancer is exploding”, including our understanding of the immune system, Dr. Jeffs remarked.

In March 2015, dinutuximab (Unituxin) became the first approved therapy for patients with high-risk neuroblastoma—representing only the third drug for initial pediatric approval for cancer in 20 years. It had been studied in an NCI-sponsored clinical trial. Based on data published in *New England Journal of Medicine* in 2010, dinutuximab was shown to significantly prolong both event-free survival (66% vs 46% with standard therapy; \(P=0.01\)) and overall survival (86% vs 75% with standard therapy; \(P=0.02\)). He explained that the NCI had approached United Therapeutics about manufacturing the antibody, but they wanted more involvement beyond the manufacturing. A Cooperative Research Agreement had been put in place—with collaboration between the NCI, Children’s Oncology Group, and United Therapeutics ultimately resulting in the commercially available product.

Regarding his experience with the FDA, after 25 years of orphan drug development, Dr. Jeffs has found the FDA to be forward-thinking and flexible. Normally 2 trials would be required, but the FDA was flexible in allowing for expedited review of dinutuximab based on a single trial. The trial included 226 patients and was rigorous. He commended the FDA for doing such a good job in areas of high orphan unmet need.

Dr. Jeffs went on to discuss the critical role that the Legislature plays in drug development. United Therapeutics was awarded a voucher under the Creating Hope Act, which it has since sold for $350 million. He noted that they intentionally set a high watermark, to give United Therapeutics a “war chest” to expand use of the drug and to explore other drugs for childhood cancer. He is hoping to see bigger companies prioritize childhood cancer drug development as well.
While the pharmaceutical industry is key to daily discoveries, childhood cancer has been a secondary priority. Dr. Jeffs noted that over 900 drugs are being developed for treating cancer, yet less than a handful are being developed for childhood cancer.

“The expense is high, but the need is overwhelming,” Dr. Jeffs reiterated.

In concluding, Dr. Jeffs stressed the important role of Advocacy—in ensuring that the key players work together, not in silos.

The next speaker was Dr. Amy Fowler, MD, one of Rex Ryan’s oncologists, who practices at Dell Children’s Medical Center of Central Texas. Dr. Fowler noted that there is an increased demand for pediatric oncologists. At the Children’s Blood & Cancer Center, they have seen the number of new cancer diagnoses more than triple between 1998 and 2014, from 30 cases to 100 cases.

Dr. Fowler participated in the clinical trial of dinutuximab, which opened in 2004 for high-risk neuroblastoma patients. It was the first biotherapy trial offered at the Children’s Blood & Cancer Center. Five patients were enrolled in the study at her center. As Dr. Fowler was part of Rex’s care team, with permission from his parents, she discussed his clinical course and explained that there are some unique side effects with immunotherapy.

She welcomes further collaboration between COG and Pharma, because “we need to do more”

Next up was Dr. Michael Link, MD, from Stanford University School of Medicine, who discussed the 21st Century Cures Act, specifically the implications for clinicians and for patients.

Dr. Link started off with a background about cancer in children, a progress report of “where we stand”, and challenges and opportunities for the future.

Cancer in children is not so rare, he explained, with about 1 in 300 children diagnosed before age 20. About 12,500 new cases in this age group are diagnosed each year in the US, with 2,225 children and adolescents dying of cancer in 2014.

He showed a graph of the most frequent cancers in the US, using data from the SEER Cancer Statistics (1973-1991), in which childhood cancers in patients age 0-19 years are the 6th most common cancer type, with an annual incidence of 15.5 per 100,000. This was surpassed only by cancers of the breast, lung, prostate, colon, and bladder. For children age 0-14 years, the annual rate was 14.1 per 100,000 (8th most common category). He also showed how survival has improved, for all children younger than 20 at diagnosis and by individual types of common pediatric cancers. Nonetheless, the average years of life lost to cancer is estimated at 69.3 years for childhood cancer, which well surpasses all other cancer types. The 2nd highest is testicular cancer, with an average of 34.8 years of life lost. The lowest on the list was prostate, with an average of 9.0 years lost.
Dr. Link went on to discuss survivorship issues. Based on results of the Childhood Cancer Survivor Study (published in the *New England Journal of Medicine* in 2006), nearly 2 of 3 long-term survivors of childhood cancer have at least one chronic health condition, and more than 1 in 4 of them have a severe or life-threatening condition.

In aggregate, it is clear that better treatments are needed, yet we have made a lot of progress. Per Dr. Link, the keys to success in pediatric oncology can be summarized as follows:

- **Luck**—Pediatric tumors have relatively few mutations and they tend to be responsive to radiotherapy and demonstrate remarkable responsiveness to early drugs. Durable remissions and cures have been achievable, and empirical strategies have been successful in some cases (ie, good ideas and “best guess” strategies have worked)
- **Host with few comorbidities**, in otherwise excellent health
- Importance of collaboration
- Importance of laboratory investigations of tumor

There have also been many lessons learned from our children, as follows:

- It takes a village
- Importance of tumor tissue
- Cancers are heterogeneous collections of diseases
- Cancers are collections of rare (orphan) diseases
- Survivors suffer consequences of toxic therapies

It is apparent that while childhood cancer mortality has improved since 1975, the curve has reached a plateau over the last 10 years.

There is a new paradigm underway in cancer treatment, the concept of targeting pathways rather than tumors. Cancers are “driven” by specific, non-random mutations, and inhibition of these driver mutations can produce remarkable responses and remissions. The pathways driving cancers are promiscuous, with the number of pathways being finite and known drivers of disparate cancers. Agents that target a specific pathway should be active against several tumors that “share” that pathway. Examples of this include imatinib and related compounds in chronic myeloid leukemia (CML) and gastrointestinal stromal tumors (GIST). He also noted that a hedgehog pathway inhibitor has shown activity in a subset of medulloblastoma.

To illustrate how complex this area is, Dr. Link showed a graph of the many specific genetic abnormalities that have been identified in childhood ALL. Many occur at very low frequencies. However, this is important information, as illustrated by study results that have identified a correlation between genotype and outcome in childhood B-progenitor ALL. Dr. Link also showed the significant improvement in survival afforded by targeted therapy with imatinib for
Ph+ ALL, relative to that for historical controls. Such findings have contributed to gradual improvements in survival for children and adolescents with ALL.

Dr. Link then shifted his attention to the 21st Century Cures Act, focusing on key initiatives for children with cancer. This legislation would increase funding for the NIH, which would be extraordinarily timely given the current opportunities for cancer research. This increase in NIH funding would also serve to encourage physicians and scientists to plan careers and biomedical investigators, with additional incentives for young investigators. Beyond the NIH, many initiatives in the bill require effort on the part of the FDA, and increased funding is essential to make these things happen. It would also provide funding support for the National Precision Medicine Initiative, an important next step toward the future of cancer care.

As part of the patient-focused drug development that is part of the 21st Century Cures Act, it calls for the Reauthorization of the Creating Hope Act, an important and provocative initiative with early success but that is set to expire in March 2016 (21st Century Cures Act may not meet this reauthorization deadline). The Creating Hope Act will not be the entire answer to pediatric cancer drug development, he said, but a comprehensive assessment of its impact is warranted and being planned for. 21st Century Cures also calls for inclusion on patient perspective in drug evaluation. It is important to assess “value” to patients and families as a component of risk/benefit assessment. However, this is complicated in cases where parents act as surrogates for their children (and thus filter the assessment), and it is not designed to be designated as the primary outcome of studies. Meaningful and “hard” outcome measures such as cure rate, progression-free survival, and overall survival should always be the primary endpoints.

The 21st Century Cures Act also focuses on novel clinical trial designs, while being careful to maintain rigorous assessment of efficacy and safety. It would require the use of a Central Institutional Review Board (IRB), which should accelerate the pace of clinical trial research. It would also directly promote pediatric research, by establishing the following networks:

- **National Pediatric Research Network**—national consortium of research institutions (in cancer research, we already have the Children’s Oncology Group)
- **Global Pediatric Clinical Study Network**—resulting in harmonization among the NIH, FDA, European Union, and industry. Dr Link emphasized that “this could be huge!!” and that in childhood cancer research, Congress acknowledges that we have already learned the hard way and thus endorses a solution that will hopefully remove barriers

Dr. Link pointed out that these measures should not replace the Pediatric Research Equality Act (PREA) and its mandates to conduct trials in children where appropriate.
Another important area pertains to access to novel agents. The 21st Century Cures Act will expedite access to new drugs in various ways, including expediting approval of breakthrough therapies, having provisions to help patients navigate FDA expanded access and compassionate use processes—making it less burdensome and allowing patients and families to better assess if an agent available through compassionate use is appropriate for them. Importantly, however, these measures must ensure that the clinical trial process is not subverted, as rigorous trials remain the best method for assessing safety and efficacy and are the best route to new, more effective therapies. Additionally, use of investigational agents in the context of a clinical trial with real-time monitoring is safer for patients than via compassionate use. However, the 21st Century Cures Act is designed to address the need for earlier access to agents under development for study in children and to expedite development of novel therapies for children with cancer.

Regarding the implications for clinicians, the 21st Century Cures Act will capitalize on a new understanding of cancer with increased investment in research, both at the NIH and FDA, while providing an incentive for the best young scientists to choose a career in research. It makes an investment in precision medicine, for which cancers are the prototype diseases and that will ultimately lead to more rational, streamlined drug development. Finally, it will harmonize efforts through the NIH and FDA and encourage broad collaboration, while granting more rapid access to promising agents by children with cancer.

Dr. Link concluded by characterizing the 21st Century Cures Act as an important first step for improving research and drug development for childhood cancer. Several new initiatives for drug developers are included, but these are to be added to incentives already in place. While newer therapeutic strategies are needed, the 21st Century Cures Act does not address this issue. Dr. Link emphasized that we must be careful not to lose the incentives and regulatory mandates already in place to encourage drug development for children with cancer. Lastly, it is important to acknowledge that incentives and mandates may need to be “retooled” as our understanding of cancer is improved. He wrapped up by thanking Representatives McCaul and Van Hollen and the Congressional Childhood Cancer Caucus, as well as all of the members of Congress who work tirelessly to advance the health of children, and particularly children with cancer.

The last speaker was Danielle Leach, MPA from St. Baldrick’s Foundation, who lost her son Mason from a brain tumor. The focus of her talk was the Childhood Cancer Survivorship Treatment Access and Research (STAR) Act and necessary advocacy, which Webster’s dictionary defines as “the act or process of supporting a cause or proposal”.

As background, the childhood cancer community has 3 large goals, which are to:
• Increase pediatric cancer research
• Improve pediatric cancer drug development
• Provide necessary support to childhood cancer survivors

The process in place for new legislation includes 3 components, namely:

• Policy Roundtable
• Consensus Building
• Champions on the Hill introduce the Bill

In the House, the STAR Act is H.R. 3381, introduced by Congressman Michael McCaul (R-TX) and Chris Van Hollen (D-MD) and Congresswoman Jackie Speier (D-CA). It had over 35 bipartisan cosponsors (as of the Caucus). In the Senate, it is S. 1883. Introduced by Senators Jack Reid (D-RI) and Shelley Moore Capito (R-WV) and had 8 bipartisan cosponsors (as of the Caucus).

Danielle emphasized that legislation takes team work with our Champions on the Hill and their staff, and she extended a special thank you for their efforts. She went on to cover the main components of this proposed legislation, as follows:

• Expanding research opportunities for childhood cancer research—authorize the NCI to expand existing efforts to collect biospecimens for childhood cancer patients enrolled in NCI-sponsored clinical trials to collect and maintain relevant clinical, biological, and demographic information on all children, adolescents, and young adults with cancer

• Improving childhood cancer surveillance—authorize grants to state cancer registries to identify and track incidences of child, adolescent, and young adult cancer. This funding would be used to identify and train reporters of childhood cancer cases, secure infrastructure to ensure early reporting and capture of child cancer incidences, and support the collection of cases into a national childhood cancer registry

• Improving quality of life for childhood cancer survivors:
  o Enhance research on the late effects of childhood cancers, including a study on insurance coverage and payment of care for childhood cancer survivors
  o Improve collaboration among providers so that doctors are better able to care for this population as they age
  o Establish a new pilot program to begin to explore innovative models of care for childhood cancer survivors

• Ensuring patients access to publicly available compassionate use policies—ensure that pharmaceutical companies have publicly accessible compassionate use policies for drugs treating serious or life-threatening condition. The STAR ACT would require the FDA to finalize its guidance for industry on this issue.

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