ASTCT
American Society for
Transplantation and Cellular Therapy

May 8, 2025

The Honorable Susan Collins
Chair
Senate Appropriations Committee
413 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Patty Murray
Vice Chair
Senate Appropriations Committee
154 Russell Senate Office Building
Washington, D.C. 20510

Dear Chair Collins and Vice Chair Murray,

The American Society for Transplantation and Cellular Therapy (ASTCT), thanks you for the opportunity to submit public comments on the Senate Appropriations Committee hearing, "*Biomedical Research: Keeping America's Edge in Innovation.*" ASTCT is a professional membership association of more than 4,000 physicians, scientists and allied health care professionals promoting bone marrow transplantation and cellular and gene therapy through research, education, scholarly publication, and clinical standards.

For more than 30 years, ASTCT members have focused on innovative cures for patients with blood cancers and other blood and immune disorders. ASTCT members and the patients we serve rely heavily on research funding from the National Institutes of Health (NIH). Funding from the NIH to our members has directly contributed to the advancement and licensing of therapies and even cures currently available to the American public. As such, we appreciate the Senate Appropriations Committee's commitment to bipartisan support of crucial biomedical research. As was referred to in the committee hearing, **healthcare is a bipartisan issue**.

As raised by Senators Kennedy and Shaheen, we agree that there is no room for fraud, waste, and abuse within the research funding system. However, we would like to address that the majority of programs receiving federal funding do not use allocated dollars outside of the intended settings, i.e. labs and clinical trials, and the evidence provided by Senator Kennedy referred to an isolated event more than 25 years old. We would welcome a robust system of auditing and verification rather than an unmerited wholesale cut to funding support.

The proposed cut to facilities and administrative support (i.e. indirect costs) would require a massive scale back of research programs.[1] Indirect costs are not necessarily attributed to a specific research project, however, the costs are necessary for research overall.[2] This can include things such as utilities, rent, maintenance, and administrative staff: all things that are necessary for the operation of successful research endeavors. It is our duty to as physicians and scientists to provide

[1] https://www.aamc.org/media/81711/download?attachment%3Fattachment
[2] https://academyhealth.org/blog/2025-02/academyhealth-situation-report-nih-abruptly-slashing-indirect-grants-what-means-researchers#:~:text=Effective%20February%2010%2C%202025%2C%20this%20guidance%20establishes,expenses%20incurred%20from%20February%2010%2C%202025%2C%20onward

the best therapies and care possible to our patients and in order to do so, NIH funding is imperative to maintain.

As hematologic oncologists, we are concerned with the growth rate of cancer in the next 20 years. According to data from the National Cancer Institute (NCI), "In 2024, an estimated 2,001,140 new cases of cancer [was] diagnosed in the United States and 611,720 people [died] from the disease."[3] The increase in number of overall cancers demands the need for continued and increased cancer research. At the end of our letter, we included a graphic from the Global Cancer Observatory that shows the steady expected increase of cancer occurrences from 2022 to 2050.

The need for NIH funding is critical to improve survival rates and to cure American citizens and patients globally. The ways in which our field relies on funding includes:

1. <u>Innovation Saves Lives</u>

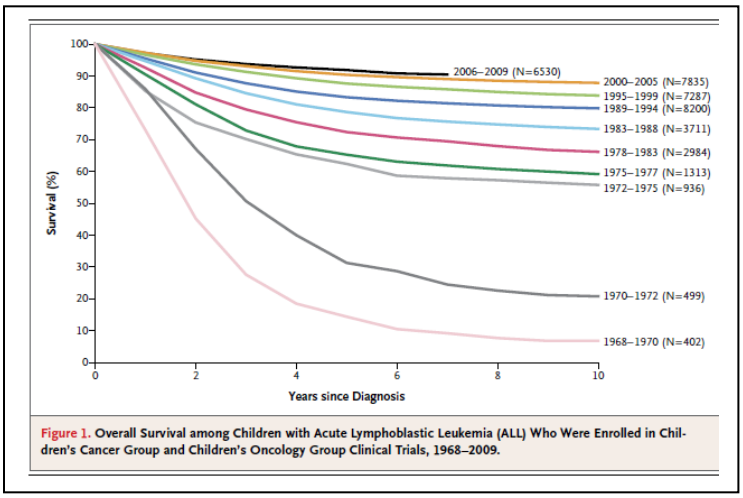

Figure 1. Overall Survival among Children with Acute Lymphoblastic Leukemia (ALL) Who Were Enrolled in Children's Cancer Group and Children's Oncology Group Clinical Trials, 1968–2009.

It is impossible to overstate the impact that decades of NIH funding for biomedical research has had. Look no further than the improvements made to pediatric leukemia treatment, for example. Just within our senior senators' lifespan, leukemia went from being essentially a death sentence for children to a condition with over 95% chance of survival; *improvements due specifically to well-crafted and publically funded clinical trials* making iterative improvements over time.[4] This longitudinal commitment from the NIH to funding rigorous biomedical research has also made possible the therapies we now employ every day, such as hematopoietic cell transplant (HSCT) and chimeric antigen receptor T-cells (CAR-T). Despite these incredible advances in our field, patients with the conditions we care for still have significant risks of death or severe complications, making ongoing research essential to continue saving lives. Cutting NIH funding will leave an immense gap in cancer research, specifically for the bone marrow and cell transplantation and cellular therapy field. ASTCT is committed to protecting the resources that drive innovation in this field because NIH-funded research dollars meaningfully translate into lives of real patients saved.

2. <u>Clinical trials enable access to lifesaving cures</u>

[3] https://www.cancer.gov/about-cancer/understanding/statistics
[4] Hunger and Mullighan. N Engl J Med 2015; 373:1541-52.

There is a direct impact to patient care when research funding is unavailable. Many of our patients participate in clinical trials funded by the NIH, partly because so many of the conditions we treat are life threatening even despite receiving state of the art medical treatments. These research trials are often the only appropriate clinical option for patients with high-risk or treatment-refractory diseases to access lifesaving therapies. When funding is removed and trials are forced to stop, this translates directly into patients with fatal disorders being unable to access lifesaving clinical trials.

3. Indirect costs are an inextricable component of research

The recent proposals to cut funding and limit coverage of indirect costs will have an immediate detrimental impact on research labs and institutions' ability to perform biomedical research. If research grants will be narrowly limited to cover resources such as cost of chemicals and specimens only, but will cut the cost of electricity that powers the entire lab, there will be real life consequences of shutting down research programs around the country. This will result in losing years of progress and momentum towards biomedical discoveries and cures.

4. Economic impacts will be far reaching

There are well-established downstream financial impacts to both federal and state economies if NIH funding is cut. In FY 2023, every one dollar of NIH funding generated approximately $2.46 of economic activity.[5] NIH is the largest single public funder of biomedical research *in the world* and in FY 2023, NIH funding generated an estimated $92.89 billion in economic activity.[6] The significance of this economic impact cannot be overstated.

One important topic that arose in the Senate hearing was for patients who receive therapies through clinical trials it is imperative for them to maintain hope for a cure. When active trials are stopped or not fully funded, patients lose hope that treatment will be possible for them which ends up affecting their overall outcomes. One recurrent theme in Mrs. Emily Stenson's testimony to the committee was the need to maintain hope. Mrs. Stenson is a mom and patient advocate for her five-year-old daughter, Charlie, who was diagnosed with Stage 4 germ cell cancer. She emphasized that if her family did not have hope for a cure, it would have been difficult for them to successfully get through Charlie's treatment. Charlie's story is one of many that we as physicians and scientists at ASTCT see every single day.

[5] https://www.nih.gov/about-nih/what-we-do/impact-nih-research/serving-society/direct-economic-contributions
[6] Ibid.

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Another important story of hope that the committee is likely aware of is the case of Henry Strongin Goldberg. Henry was diagnosed with Fanconi anemia at two weeks old and spent endless days in the hospital during his treatment. His parents, Laurie Strongin and Allen Goldberg, realized that while Henry was being treated for his medical issues, there was a lack of hope for Henry's recovery and his needs went unmet. His mother started Hope for Henry, which helps pediatric patients maintain needed "optimism and play in the lives of seriously ill children."[7] While Henry was not able to find a matched donor in his lifetime, there have been advancements in clinical trials and research studies where both pediatric and adult patients have access to receive a mismatched, unrelated donor for their transplants. Studies such as the "Access Trial" have made this possible.[8]

While optimism and hope for a cure is important, it is only possible for patients and their families to maintain hope if available clinical trials exist, which requires NIH funding so treatments and cures can continue to be discovered.

ASTCT remains steadfast in our mission to strongly advocate for policies that support bone marrow and cell transplantation and cellular therapy research and patient access to care. We continue to support efforts that drive science and bring potentially curative medical therapies to all patients in need. We will support and advocate for our members, our field and the patients who put their trust in us. As such, ASTCT remains willing to work with policymakers and stakeholders to safeguard critical funding and ensure that patients have access to the lifesaving therapies they need.

Again, we commend the Senate Appropriations committee for holding such an important and timely hearing and we appreciate the opportunity to weigh in. If interested, we would be honored to provide more compelling stories where NIH research funding lead directly to lifesaving treatment for children and adults with leukemia and other cancers. If you have any questions or need any additional information, please contact Alycia Maloney ASTCT's Director of Government Relations, at amaloney@astct.org.

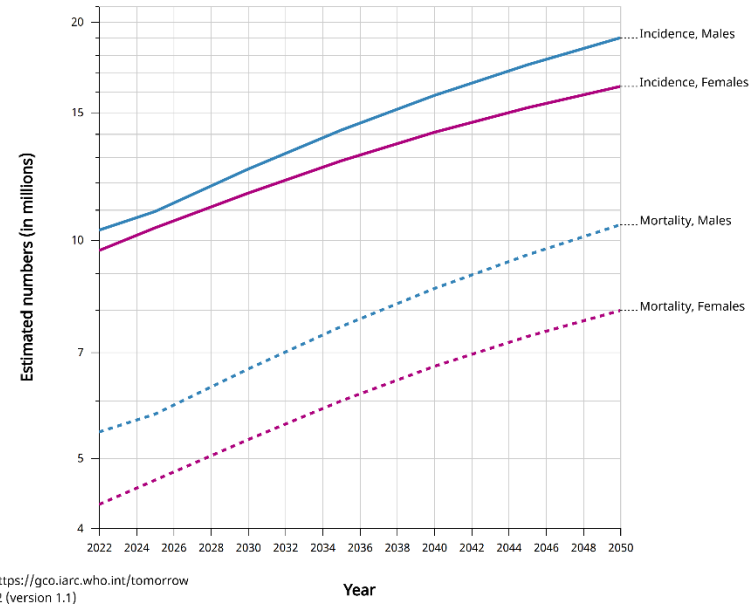
Sincerely,

David L. Porter, MD
President, ASTCT

[7] https://hopeforhenry.org/what-we-do/meet-henry/
[8] https://ashpublications.org/blood/article/140/Supplement%201/7591/487763/Access-A-Multi-Center-Phase-II-Trial-of-HLA

Estimated numbers from 2022 to 2050, Males and Females, age [0-85+]
All cancers

GLOBAL CANCER OBSERVATORY



Cancer Tomorrow | IARC - https://gco.iarc.who.int/tomorrow
Data version : Globocan 2022 (version 1.1)

International Agency for Research on Cancer
World Health Organization