American Association for Cancer Research
FINDING CURES TOGETHER™

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May 13, 2015

The Honorable Fred Upton
Chairman
Energy & Commerce Committee
U.S. House of Representatives
Washington, DC 20515

The Honorable Frank Pallone
Ranking Member
Energy & Commerce Committee
U.S. House of Representatives
Washington, DC 20515

The Honorable Joe Pitts
Chairman, Subcommittee on Health
Energy & Commerce Committee
U.S. House of Representatives
Washington, DC 20515

The Honorable Gene Green
Ranking Member, Subcommittee on Health
Energy & Commerce Committee
U.S. House of Representatives
Washington, DC 20515

Dear Chairman Upton, Ranking Member Pallone, Chairman Pitts and Ranking Member Green:

The American Association for Cancer Research (AACR) is the world's first and largest scientific organization focused on every aspect of high-quality, innovative cancer research, from bench to bedside. The mission of the AACR and its more than 35,000 members in all fifty states and around the world is to prevent and cure cancer through research, education, communication, and collaboration. Our members include basic, translational and clinical researchers, physician-scientists, patient advocates and other leaders in the cancer research and care community.

We commend the House Energy & Commerce Committee for its commitment to the discovery, development, and delivery of new therapies to patients, especially those individuals who are suffering from the more than 200 diseases we call cancer. We especially thank House Energy & Commerce Committee Chairman Fred Upton and Congresswoman Diana DeGette for their bipartisan leadership of the 21st Century Cures Initiative, and we appreciate the opportunity to submit these comments in response to the second draft bill released on April 29, 2015.

Title I: Discovery

AACR applauds the increased funding for the National Institutes of Health (NIH)

First and foremost, the AACR applauds the Committee for including language that would authorize increased funding for the NIH through sustained, predictable increases of $1.5 billion per year over the next three years, and also language that would provide an additional $10 billion in mandatory funding over the next five years through the creation of a new "NIH Innovation Fund."

We thank the Committee for making NIH funding a top priority in the bill, thereby recognizing the critical importance of NIH funded-research to improving our nation's health, sustaining our leadership in medical research, and remaining competitive in today's global information and innovation-based economy.

The AACR recognizes the federal government has an irreplaceable role in supporting medical research and believes the new provisions in the bill would effectively put the NIH back on a path

of sustained, predictable growth and begin to restore funding that has been lost over the past decade through budget stagnation and outright cuts.

AACR appreciates the support for agency personnel to participate in scientific meetings

Furthermore, we appreciate the Committee's interest in ensuring that NIH and FDA staff scientists participate in scientific meetings and conferences, such as the AACR's Annual Meeting, which this year drew record attendance of more than 19,000 scientists and health professionals from around the world. Attending scientific meetings and research conferences is an important way for NIH and FDA scientific staff to stay connected with their respective communities and keep up with scientific advances. We hope the new "Sense of the Congress" language that is included in Section 1025 of the bill will help relieve some of the restrictions currently placed on agency personnel and will help facilitate the scientific collaborations that lead to breakthroughs and cures. We recommend that the Sense of the Congress language also be written to include scientific and regulatory staff from other agencies such as the Food and Drug Administration (FDA).

AACR is concerned about the potential micromanagement of NIH operations

The AACR strongly supports prudent planning and management of the overall NIH budget, which is actively taking place within the Office of the Director and in each of the twenty-seven NIH Institutes and Centers. Currently each Institute and Center establishes its strategic plan based on its specific mission and scientific opportunity. In addition, NIH Director Francis Collins stated in testimony before the Senate Appropriations Subcommittee on Labor, Health and Human Services, Education and Related Agencies on April 30, 2015, that the development of an overarching NIH strategic plan already was underway. This plan, which according to Dr. Collins will be completed by the end of this year, will link to the plans of the individual Institutes and Centers.

The AACR does not support setting priorities for the NIH through statutory language, such as is outlined in Section 1021, because it limits the discretion and judgment of the scientific leadership at the agency, and in doing so, could hinder the scientific inquiry that for many years has led to breakthroughs in the understanding of many diseases, including cancer, as well as new therapies for numerous diseases and conditions. In fact, the ten "Mission Priority Areas" could be interpreted as narrow in focus and could prevent the agency from responding to exciting scientific opportunities and/or emerging health needs. Research that, on the surface, appears to be directed towards one aspect of biomedical research can lead to major advances in other areas. An example is how discoveries related to the immune system driven by research into HIV are having a major and positive impact on development of cancer immunotherapy.

The AACR strongly believes that the NIH Institutes and Centers should continue to have the flexibility to make the type and size of awards that are best suited to advance science with the ultimate goal of enhancing health and reducing the burden of diseases such as cancer. The peer review process administered by the NIH is second to none in the world, and has set a "gold standard" for the selection of the most meritorious proposals that countries around the world seek to emulate.

AACR is concerned with provisions that are duplicative with ongoing activities

The AACR is concerned that the language in Subtitle B and specifically, sections 1021 and 1023 could establish programmatic redundancies that could decrease efficiency and lead to additional overlapping and duplicative activities. Such activities ultimately could take precious

resources away from what the agency does best—fund and promote the science that leads to new knowledge and discoveries. The NIH has been called the "crown jewel" of the federal government, serving as a beacon of international admiration and favorable opinion.

In addition, we believe the Biomedical Research Working Group in Section 1023 is unnecessary given that there are several advisory groups, including the National Science Board and the National Academies of Science, that already are addressing this issue. Furthermore, NIH already has three separate entities that oversee the grant proposal and submission process. The Scientific Management Review Board (SMRB), the Center for Scientific Review and the Advisory Committee to the Director all consider ways to restructure, streamline and simplify the submission of grant proposals to the NIH. It would appear that these entities collectively have the authority and ability to do what is being asked of this new Working Group.

AACR has concerns about the creation of a clinical trial registry and databank

The AACR also has concerns about language included in Subtitle F, Section 1101 and Subtitle G, Section 1121 that would mandate creation of a publicly available, Clinical Trials Registry and Databank to be administered by a third party entity. Federal research agencies, including the NIH, already are required to develop plans to increase public access to research data, and there is a considerable amount of work taking place at the NIH Institutes and in the private sector to determine the best approach to collecting detailed information from patients in clinical trials. We believe it is too early to mandate, through statute, a new database, as there are additional considerations that must be taken into account, such as duplication with existing registries and privacy concerns. The AACR urges the Committee to consider an interim approach, such as a pilot project, to assist the agency in moving these important efforts forward in the most efficient and effective manner. As mentioned above, we believe that legislative language that supports NIH leaders in their ability to address exciting scientific opportunities and emerging health care needs will best advance the nation's research agenda. We are concerned that legislatively directing a new program too soon would effectively "put the cart before the horse," and would result in additional and unnecessary regulations that are costly and inefficient.
It is important that any new language that changes oversight or regulation of research should support and facilitate the medical research ecosystem, not hamper the work of the NIH or its grantees.

Title II: Development

The AACR appreciates the Committee's detailed review of the framework of medical product approval at the Food and Drug Administration (FDA) in its commitment to ensuring improved medical products reach patients in an expedited manner.

AACR commends the streamlined data review and expediting patient access provisions

The AACR commends the Committee for including provisions in Sec. 2063 to streamline data review and thereby streamline the drug development process without compromising patient safety. We are also pleased to see in Sec. 2081 "Sense of the Congress" language urging continued efforts on the part of the FDA to expedite the approval of drugs designated as "breakthrough therapies." Many of these designations have already resulted in innovative, lifesaving therapies reaching cancer patients faster than they might have due to passage of the landmark 2012 Food and Drug Administration Safety and Innovation Act (FDASIA).

We applaud the Committee's efforts to develop a sensible expanded access policy for investigational drugs in Sec. 2082. We respectfully request that the Committee consider means

of including creation of a streamlined, transparent, and easily-navigable process for patients and physicians seeking expanded access to unapproved drugs from the FDA and sponsors.

AACR supports qualification and use of drug development tools

The AACR commends the Committee's recognition of the need to integrate advances in research into the regulatory process by establishing a framework to qualify the development of new tools. Qualification of these tools, such as biomarkers and surrogate endpoints, will expedite drug development, as we have already seen in cancer. However, we are concerned that Sec. 2021, if enacted, would require significant resources from the FDA. To date there is no new funding authorized in the draft legislation to assist with these mandatory activities that would be required of the agency in addition to the various product review related tasks that the agency must also carry out within defined user-fee designated timeframes.

The AACR and its members are pleased to see that the efforts to qualify new biomarkers and surrogate endpoints outlined in the bill will involve a transparent, public process that will be conducted in consultation with medical research consortia. We would be pleased to offer the AACR's broad scientific and clinical expertise to the FDA as the agency proceeds with these efforts.

AACR is concerned about the lack of resources provided to the FDA to carry out the many additional requirements that have been proposed

As it is seeking to support the NIH through additional funding, so must the Committee consider a parallel commitment to ensuring the FDA has the resources it needs to carry out its regulatory and oversight functions, as well as recruit, develop, and retain highly qualified staff with diverse backgrounds. Advances in regulatory science should parallel advances in basic, translational and clinical science. If not, promising new medical therapies may never reach patients simply because we lack the tools to recognize their potential or outmoded evaluation methods delay or deny their approval.

In fact, this draft bill would place considerable demands on the FDA, including the requirement to issue more than 15 new guidances and hold several workshops and meetings, all within a relatively short time frame. It is imperative that the Committee include language that would authorize additional funding for the agency so that the mandated requirements in the bill can be carried out efficiently without compromising the quality of medical product reviews.

AACR is concerned with provisions that could hinder FDA's ability to be nimble

Science and technology, our understanding of cancer biology and innovation in our approaches to cancer treatment are quickly evolving. The AACR agrees with the Committee that this rapidly changing environment requires flexibility and modernization of our regulatory approaches. The draft legislation seeks to address the processes by which the FDA considers and approves new therapies, and the AACR is concerned current language in some sections of Title II such as Sec. 2061 could hamper, rather than facilitate, the work of the agency. One of the hallmarks of the FDA is its ability to be flexible and employ discretionary judgment as it considers various medical product applications. This level of autonomy has allowed to agency to make risk/benefit assessments in the context of life-threatening diseases and various unmet medical needs. Thus, while we embrace the Committee's desire for a modernized regulatory framework to oversee regulation of innovative medical products and ensure their safety and efficacy, we strongly suggest that this can be achieved by allowing the agency to incorporate the most evidence-based regulatory science principles in an ongoing basis.

It is important that Congress ensure when drafting language to first “do no harm” and to provide the appropriate level of direction to the agency.

Conclusion

The AACR is pleased to be able to provide the Committee with its comments and commends its Members and staff for their bold efforts over the past year to put forward a proposal with the ultimate goal of accelerating the pace of cures and medical breakthroughs in the United States by ensuring that our laws are keeping pace with innovation.

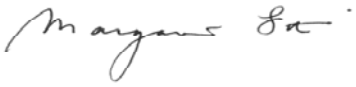
As you know, cancer remains a formidable opponent. In fact, this year it is estimated that more than 1.6 million Americans will be diagnosed with cancer, and we will lose one person, every minute of every day in the United States to this devastating disease. The rate of cancer incidence is steadily increasing; therefore, a continual effort to strengthen our nation’s commitment to medical research, cancer research in particular, is critical now more than ever.

An increased investment in NIH research and training, supporting policies that promote a patient-centered, collaborative approach to cancer research and care, and optimizing our regulatory processes through a well-funded FDA to ensure the development and delivery of innovative medical products, are all required to address the current challenges in cancer research and care, as well as across all diseases.

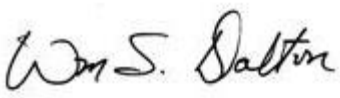
The AACR and its more than 35,000 members commend the Committee for its commitment to funding the NIH and to an ongoing dialogue. We look forward to continuing to work with you to ensure that the NIH and FDA have the resources and tools needed to continue to spur innovation and deliver hope to patients and their family members all across our great nation and throughout the world.

Sincerely,

Jose Baselga, MD, PhD
President



Margaret Foti, PhD, MD (hc)
Chief Executive Officer



William S. Dalton, PhD, MD
Chair, AACR Science Policy & Government Affairs Committee