# CANCER LEADERSHIP COUNCIL

**A PATIENT-CENTERED FORUM OF NATIONAL ADVOCACY ORGANIZATIONS ADDRESSING PUBLIC POLICY ISSUES IN CANCER**

August 2, 2024

The Honorable Diana DeGette
United States House of Representatives
Washington, DC 20515

The Honorable Larry Bucshon, MD
United States House of Representatives
Washington, DC 20515

Dear Representatives DeGette and Bucshon:

The undersigned cancer organizations representing patients, researchers, health care professionals, and caregivers are pleased to respond to the Dear Stakeholder letter of June 6, 2024, in which you asked for input regarding the impact of Cures 2.0 initiatives that have advanced through legislation or executive action as well as advice regarding initiatives that were not included in Cures 2.0 but should be advanced. You also ask for guidance regarding structural reforms of agencies, offices, or programs that are part of the Cures 2.0 effort.

In the years since enactment of the 21st Century Cures Act and the introduction of the Cures 2.0 legislation, research advances have resulted in significant improvements in cancer treatment. Among the advances are immunotherapies such as immune checkpoint inhibitors, chimeric antigen receptor-T cell (CAR-T) therapy, and bispecific antibodies; radiation therapy advances including proton therapy; and minimally invasive surgical techniques. Research continues on CAR-T cell therapies for their use in solid tumors, investigation of other cell and gene therapies proceeds, immunotherapy research continues apace, and there is promising focus on personalized cancer vaccines. For some cancer patients, treatment options have changed their prognosis after diagnosis. For others, new treatment options are turning cancer into a manageable, chronic disease. However, even those who are benefiting from new research advances may face significant toxicities – financial and other – from their treatment and an increasing number of patients are struggling to gain access to therapies at all.

***Advice Regarding New Models for Development and Access to New Gene and Cell Therapies***

Some researchers, therapy developers, advocates, and clinicians have recommended that we consider innovative models for the development, manufacturing, access, and payment for new cell and gene therapies. These experts see great promise from cell and gene

therapies, including the potential for cure of currently life-threatening diseases. They also anticipate significant price tags for these therapies and the possibility of access issues related to price and the complexities of delivering certain cell and gene therapies.

Nobel Laureate Jennifer Doudna, co-inventor of CRISPR technology, has convened a 30-person expert panel through the Innovative Genomics Institute to make recommendations regarding cell and gene therapy development and access. The panel, after meeting for a year, recommended a "mixed organizational model" for developing cell and gene therapies and making them accessible and affordable. This model would include an academic institution, a nonprofit medical research organization and a public benefit corporation to oversee research, manage manufacturing, and negotiate third-party coverage.[1]

We are interested in the discussion that has been triggered by the Innovative Genomics Institute and others, and we think these ideas should be further discussed and evaluated. However, we need not anticipate coverage and reimbursement problems in the future. Coverage and reimbursement problems are already here, and as a result of them patients are increasingly denied access or experience consequential delays in access to potentially life-saving therapies or are obtaining those therapies with great financial toxicity. If, in considering issues related to access to "cures," you are inclined to consider alternative models like that proposed by the Innovative Genomics Institute, we hope you will FIRST consider solutions to the coverage and payment issues facing cancer patients at this moment.

***Current Systems of Coverage and Payment for Cancer Therapies***

The research and development efforts that have revolutionized cancer treatment for some have been accompanied by significant difficulties in obtaining access to treatment innovations. The patient suffers the most, of course, but all in the health care system are affected – biotechnology and pharmaceutical innovators, insurers, employers offering health insurance, health care professionals, benefit managers, family members, and patients most of all. Put simply, patients cannot be assured that they will have access to the cancer therapy prescribed to them and most appropriate for them.

We will reference the burgeoning use of so-called alternative funding programs (AFPs) in employer-sponsored health plans, to underscore the stresses and strains in coverage and payment for cutting-edge therapies. Employers who offer their employees health insurance through self-funded options have increasingly turned to entities that offer so-called "alternative funding programs," or AFPs, as a strategy to control the costs of prescription drugs for their employees. These entities are typically for-profit vendors who emphasize their ability to control prescription drug costs.

Although employees understand their employers' effort to control their overall health care spending, the operations of AFPs can be tragic for patients. An employer, with the assistance of a vendor, may take one of two actions to limit their prescription drug spending: 1) exclude drugs classified as specialty prescription drugs – often expensive

[1] Innovative Genomics Institute, Report, Making Genetic Therapies Accessible and Affordable, accessed on July 22, 2024, at https://innovativegenomics.org/atf-report/.

drugs for diseases like cancer – as non-essential[2] or 2) automatically deny prior authorization of covered drugs.

Either of this actions make patients "appear" uninsured or uninsured for certain prescription drugs, a classification that then permits vendors operating AFPs to seek patient assistance programs, imported drugs, or other alternative streams of funding or drug for patients.[3] At a time when patients most need the assurance of prescription drug coverage through their insurance, they are told they are uninsured or at least underinsured and that they must work with a vendor who will attempt to find coverage for their "non-essential" drugs.

The use of alternative funding plans has seriously disrupted patient access to therapies prescribed to them. Patients may ultimately obtain the drugs they are prescribed, potentially after delay that may affect the benefits of those drugs and with greater cost-sharing responsibilities than anticipated. Health care professionals are also burdened by AFPs, as they are called on to help their patients navigate their prescription drug coverage and payment issues, a process that is time-consuming for providers and their practice administrators. Those companies that have developed life-saving drugs find that coverage of their drugs essentially evaporates through a declaration that those drugs are "non-essential" under a plan's benefit structure. The use of AFPs fundamentally undermines the goals of the "cures efforts," by denying or delaying access to life-saving therapies.[4]

Again, we describe the use of alternative funding plans to share a particularly aggressive example of management of insurance benefits, a management strategy that almost never helps patients because it typically does NOT provide affordable and timely access to the most appropriate prescribed therapies. However, there are other slightly less extreme examples of management of prescription drug benefits that serve to disrupt patient access to their prescribed therapies. These include:

- Restrictive formularies;
- Formulary management tools, including limits on specialty drugs, step therapy, and prior authorization; and
- Limits on patient assistance of various forms and structures.

The process for obtaining access to prescription drugs, which for many also includes reliance on prescription benefit managers (PBMs), is seriously broken for many Americans and for most people with cancer. As we have stated repeatedly above, even those cancer

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[2] Vendors classify those drugs that are not required to be covered under 45 CFR 156.122(a)(1) as "non-essential."

[3] The Food and Drug Administration (FDA) describes alternative funding plans via a warning letter to ElectRX and Health Solutions LLC regarding the importation of drugs in connection with an alternative funding program. In this warning letter, FDA describes AFPs. Accessed on July 14, 2024: ElectRx and Health Solutions, LLC - 614251 - 03/02/2023 | FDA.

[4] For a discussion of the impact of delays in care on outcomes, see the City of Hope blog, "Why Delayed Cancer Treatment Results in Higher Death Risk, accessed on August 2, 2024 at https://www.cancercenter.com/community/blog/2024/07/delayed-cancer-treatment-risks.

survivors who do receive their prescription medicines typically do so with delay and accompanied by financial toxicity.

There are a number of legislative proposals in the current Congress that would address some of the issues that complicate patients' affordable access to prescription drugs, including PBM reforms (transparency and more), rejection of efforts to limit patient assistance, prior authorization reform, and more. There are also nascent regulatory efforts to address the patient care obstacles created by prescription drug alternative funding programs. We urge action by Congress to protect patient access to affordable prescription drugs. At the same time, we despair of action by Congress or regulatory action in 2024, which means that patients will continue to struggle regarding prescription drug access. If our pessimism is not overstated, we urge you to turn your attention in the new Congress to comprehensive action (through perhaps a cures-focused access package) to address problems in the prescription drug market that find patients, including cancer patients, struggling for access to their prescription drugs.

***Patient Experience Data and Real-World Evidence***

Many of our organizations were engaged in early discussions related to 21st Century Cures and have remained engaged with you in efforts to foster the use of patient experience data in research, care, and regulatory decision-making. Others have been for the same period of time focused on the uses of real-world evidence, including in regulatory decision-making. Overall, we are pleased with the actions taken by the Food and Drug Administration (FDA) to provide guidance related to both patient experience data and real-world evidence.

Recently, FDA issued a fourth guidance on real world evidence and described it in this way:

> This guidance (Guidance 4) is the fourth in a series of four methodological patient-focused drug development (PFDD) guidance documents that describe how stakeholders (patients, caregivers, researchers, medical product developers, and others) can collect and submit patient experience data and other relevant information from patients and caregivers to be used for medical product development and regulatory decision-making.

Despite the positive efforts by FDA to provide guidance on collection and submission of real-world evidence and patient experience data and the use of these data in regulatory decision-making, the actual use of the data lags behind our expectations and hopes. We believe that this should be a focus of your "cures" efforts on an ongoing basis. We stand ready to share our ideas about uses of these two sources of data. An immediate need is the use of these data in coverage and payment analyses; we believe that the data can provide regulators, payers, and those conducting health technology assessments information about the full benefits of new therapies in real world use and can also inform the management of side effects of innovative therapies, including long-term effects.

***ARPA-H***

In July 2021, the Cancer Leadership Council published a statement of principles, endorsed by many of our members, related to ARPA-H. That statement is included as an attachment to this letter.

Our letter is detailed, but we fundamentally recommended that ARPA-H be separate and independent from the National Institutes of Health (NIH), should be a risk-taking entity, should encourage relatively rapid turnover among its leaders, and should focus on unmet medical needs. In general, we think that ARPA-H is well organized and is honoring the principles that we felt were very important. We also think that it is simply too soon to make an assessment of ARPA-H that might lead to changes in structure or function.

ARPA-H has to date been open to cancer community stakeholders, updating us on its structure and on work supported to date. We would, however, encourage ARPA-H to consider ways to hear from cancer stakeholders about their needs and priorities instead of primarily sharing ARPA-H actions. The cancer community has benefited from a culture at the National Cancer Institute that encourages our input through formal advisory committee structures and more informally, and the Congressionally Directed Medical Research Program at the Department of Defense engages community members in its operations. These are models that might be considered by ARPA-H for soliciting information from cancer stakeholders.

On one issue the input of cancer survivors and the health professionals who treat them is especially important: unmet medical need. ARPA-H should be listening carefully to cancer stakeholders about unmet medical needs and about the shortcomings of current treatments.

***Reform of Cancer Care Delivery and Payment***

For cancer patients to receive the full benefit of "cures" they must be cared for in a system in which care is planned, well-coordinated, and provided to them from diagnosis and over the full continuum of their disease. Some of the cell and gene therapies that we have discussed above require a sophisticated system of care for the treatment to be delivered appropriately and for any side effects to be managed promptly and adequately. Moreover, all cancer patients deserve a system of care in which symptom management is provided.

As part of the Cancer Moonshot effort, the Biden Administration has proposed a program for patient navigation and the management of social determinants of health (SDOH). This initiative is being administered by the Centers for Medicare & Medicaid Services (CMS) through Medicare physician fee schedule (PFS) codes to support navigation and SDOH assessment and management. CMS has also established codes for use by private payers to support patient navigation. We strongly support this effort to pay for patient navigation and coordination of care, as one step toward a more well-integrated system of cancer care.

The PFS codes have only been in effect since January 1, 2024, so an assessment of the program is premature. CMS is undertaking Medicare provider education efforts to encourage uptake of the codes. We encourage additional efforts to foster utilization of

these codes, efforts that might include additional federal agencies and more broad-based public-private collaboration. We think CMS is doing an outstanding job to date but could use additional resources and agency collaborators to make navigation a reality for all. We believe that this cancer care program is a complement to all Cures efforts because of its potential to improve the delivery of care.

Although fee-for-service codes can make a significant difference in how care is delivered, we support continued efforts by the Innovation Center at CMS to design and implement alternative payment and delivery models that will foster better coordination of care, ensure the integration of supportive care with active treatment, and boost overall quality of cancer care. We understand that some in Congress are urging an end to the work of the Innovation Center, arguing in part that models to date have not been successful, especially in reducing health care spending. We have seen some benefits from the cancer care models tested to date and encourage continued efforts to launch alternative payment models. We recommend continued care and payment experimentation as a logical complement to the work to cure cancer, and we suggest that you lend your support as part of the ongoing cures effort. The Innovation Center should be encouraged to continue its collaboration with providers and survivors in designing and implementing alternative care models.

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As a coalition, we will also be offering comments to some of your House colleagues regarding their plans for reform of NIH. In the early fall, we will comment on an FDA draft guidance for industry related to clinical trials diversity action plans. We will share those comment letters with you, as they will address issues related to your work on fostering cures and access to those cures.

Thank you again for the opportunity to offer our collective advice on several issues.

Sincerely,

**Cancer Leadership Council**

Academy of Oncology Nurse & Patient Navigators
American Society for Radiation Oncology
Association of Oncology Social Work
Cancer*Care*
Cancer Support Community
Children's Cancer Cause
Fight Colorectal Cancer
International Myeloma Foundation
LUNGevity Foundation
Lymphoma Research Foundation
National Coalition for Cancer Survivorship
Prevent Cancer Foundation

Attachment: Cancer Leadership Council Principles for the Advanced Research Projects Agency for Health (ARPA-H), July 2021

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## CANCER LEADERSHIP COUNCIL
## PRINCIPLES FOR THE ADVANCED RESEARCH PROJECTS AGENCY FOR HEALTH (ARPA-H)

The member organizations of the Cancer Leadership Council are dedicated to researching and developing lifesaving cancer therapies; delivering quality cancer care; providing support services and educational services to cancer survivors from diagnosis and across the continuum of care as well as to individuals, families, and communities with elevated cancer risk; supporting research and programs to improve cancer prevention and early detection efforts; and ensuring equitable access to quality cancer care. We recommend principles for the Advanced Research Projects Agency for Health, or ARPA-H, to ensure that the new entity produces meaningful benefits for people with cancer, strengthens and complements ongoing cancer research efforts, advances quality cancer care for all, and reduces health care disparities.

- The new entity should not replicate the work of academic researchers supported by private research foundations and the National Institutes of Health (NIH) or the work of pharmaceutical or biotechnology companies engaged in research and development. The entity should collaborate with those partners and a wide range of government agencies. ARPA-H should focus on projects that would not be undertaken by other research entities or industry partners.

- ARPA-H should be guided by a culture of risk-taking that accepts failure. Fostering this culture will require a leader who has a broad range of research and development expertise and experience managing collaborative ventures. The ARPA-H leader should be given significant independence to achieve this culture of innovation. Congress should consider making the ARPA-H Director a Presidential appointee not requiring Senate confirmation (comparable to the National Cancer Institute Director position) to provide the leader adequate authority and independence.

- The new research entity should employ criteria for selecting projects that can be used efficiently and that do not have the limitations of some peer review programs, including the length of the review process. However, the

standards for selection of projects, the metrics for success, and the standards for continuing or terminating projects must be transparent and publicly disseminated. ARPA-H should report routinely and publicly on funded projects and their status.

- ARPA-H should support "use-driven" research, or research that is directed at solving a practical and specific problem. This research might be directed at developing a specific product or treatment, and it might also involve creating platforms, capabilities, and resources that can be used across a range of products and across many diseases.

- The use-driven research of the new entity should include efforts to develop new and improved tools for cancer screening, risk management for genetic predisposition to cancer, and early detection, especially important in the wake of the disruptive impact of the coronavirus pandemic on screening and early detection.

- ARPA-H leaders and managers should evaluate all the new agency's projects and initiatives for their potential to address health disparities. The new research agency should include use-driven research efforts that are specifically directed toward improving the diversity of clinical trials enrollees and enhancing the diversity of the research workforce and clinical care workforce.

- Improving cancer treatment should be one goal of the new research entity, and those efforts should focus not only on development of new drugs and therapies but also on strategies to improve the delivery of care and ensure equitable access to quality care. Projects to improve cancer treatment should include the development of interventions and systems of care that reduce immediate and late and long-term side effects of therapies to enhance the quality of life of cancer survivors from diagnosis through treatment and survivorship. The side effects of cancer and cancer treatment for which better interventions should be developed include but are not limited to nausea and vomiting, fatigue, mental health challenges, hair loss, sexual dysfunction, cachexia, and financial toxicity.

- The new research entity should direct special attention to diseases where there is unmet medical need, which may relate to the limited incidence of the disease, lack of basic scientific understanding of the disease, modest private sector investment in the disease, or other factors.

- The new entity should invest in cancer prevention as a means of reducing disparities in access to screening, care, disease burden, and health care costs. These efforts should include attention to the genetic predisposition to elevated cancer risk and disproportionate disease burden due to social determinants of health such as education, environmental and neighborhood factors, economic stability, health and health care, and social and community contexts.

- The work of ARPA-H should be informed by a permanent advisory council including patients, patient advocacy organizations, health care professionals, researchers, industry representatives, third-party payers, health policy experts, and other stakeholders. This panel should be utilized early to provide advice about possible projects. Although these stakeholders will be able to advise about all types of projects, their advice may be especially important regarding projects to improve health care delivery, enhance equitable access to care, and strengthen resources for clinical research. The participation of patients and patient advocacy organizations in the advisory council is of paramount importance.

- Congress should consider how intellectual property issues that may arise in connection with ARPA-H projects will be addressed. Congress should also consider how Americans will be ensured access to the products developed through ARPA-H projects.

- ARPA-H should be generously funded so that promising projects can be awarded support without delay or without funding adjustments that might affect project success. ARPA-H funding must not be provided at the expense of NIH or other federal public health agencies and programs important to cancer patients and survivors.

- NIH funding should be increased at the same time ARPA-H is launched, to boost the percentage of approved grants that can be funded and to ensure that NIH can be a strong partner to ARPA-H through aggressive funding of basic, translational, and clinical research.

These principles of the **Cancer Leadership Council** are endorsed by the following member organizations:

Academy of Oncology Nurse & Patient Navigators

American Society for Radiation Oncology
Association for Clinical Oncology
Association of Oncology Social Work
Cancer*Care*
Cancer Support Community
Children's Cancer Cause
Family Reach
Fight Colorectal Cancer
Hematology/Oncology Pharmacy Association
International Myeloma Foundation
LUNGevity Foundation
Lymphoma Research Foundation
National Coalition for Cancer Survivorship
Ovarian Cancer Research Alliance
Prevent Cancer Foundation
Susan G. Komen

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