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 some struggle 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 corporate to oversee research, managing 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 on occasion being denied 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

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

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 describe the burgeoning use in employer-sponsored health plans of alternative funding plans (AFPs) 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 plans," or AFPs, as a strategy to control the costs of prescription drugs for their employees. If an employer utilizes the services of an entity managing an AFP, they typically classify specialty prescription drugs – often expensive drugs for diseases like cancer – as non-essential. With this determination that a drug is non-essential and therefore not covered by the insurance plan, the AFP entity then attempts to find other options for providing the drug to the patient, including patient assistance, free drug from the drug developer, or other options.[2]

The use of alternative funding plans has seriously disrupted patient access to therapies prescribed to them. Patients whose drugs are declared non-covered by their plans face a change in the insurance they thought they enjoyed. They 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 typically attempt to help their patients navigate their insurance coverage 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. Employees understand that their employers are attempting to manage their health care costs and to provide employees good health insurance coverage at an affordable price, but that is small comfort when the declaration that a therapy is non-essential triggers an alternative funding plan.

Again, we describe the use of alternative funding plans to share an extreme 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 prescribed therapies. However, there are other slightly less extreme examples of management of

[2] The Food and Drug Administration (FDA) on March 2, 2023, sent 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.

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 a prescription benefit manager (PBM), is seriously broken for many Americans and for most people with cancer. As we have stated repeatedly above, even those cancer 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 plans. 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, including payers, 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 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.

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 effort 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 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