Securing the Future of Innovation in Cancer Treatment

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PROJECT INNOVATION
Cancer Cures Are Born of Medical Innovation

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As editor of this publication, it has been a privilege to work with these organizations and our NPAF representatives in the completion of this important document, which highlights specific areas of concern that must be addressed if the United States is to maintain its leadership in research and development of innovative new cancer therapies. This initiative will require all stakeholders to work together to achieve solutions to these areas of concern and to secure “the Future of Innovation in Cancer Treatments,” assuring all people in America of a brighter future as new ways to cure and/or manage the disease are developed.

Nancy Davenport-Ennis
NPAF Founder and Chairman of the Board
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Executive Summary
Securing the Future of Innovation in Cancer Treatment

Cancer kills nearly 1600 Americans every day. In 2014, about 585,720 Americans will die of cancer, translating to almost one out of every four deaths in the United States.¹ As a country, we will spend $201.5 billion in health care costs to fight cancer and to provide treatment to the 1.6 million Americans who will be diagnosed this year.² Simply put, with these numbers, cancer impacts everyone.

Quality cancer solutions, then, require a partnership not just between patients and caregivers, not just between payers and providers, but in fact, among everyone. Improving outcomes and quality of life for cancer patients and their families is a massive undertaking that can be achieved only through substantive and cooperative investment from all health care stakeholders, including the biopharmaceutical industry, academia, health care payers, providers, patients, nonprofit patient organizations, employers and the public sector.

To respond to this challenge, the Cancer Innovation Coalition (CIC) has developed collaborative policy solutions that will facilitate innovation and promote novel approaches to foster the research and development of innovative cancer treatments. These approaches will benefit patients, reduce burden on the health care system, increase productivity and strengthen the economy at large while mitigating regulatory and financial hurdles. Above all, the goal of delivering innovative care to patients is paramount to ensure access to new and potentially life-saving treatments. Breakthroughs in cancer treatment bring tremendous personal value to patients and families, who are able to enjoy healthy and productive lives, and bring substantial economic value to our health care system.

The CIC has identified three necessary pillars of innovation:

1) Expanding on the Science of Innovation by Streamlining Logistical Obstacles

Between 1988 and 2000, life expectancy for cancer patients increased by approximately four years, which translates to about 23 million additional years of life and roughly $1.9 trillion in value added to the economy.³ Since some therapies introduced in more recent years have shown better tolerability than traditional chemotherapy, the quality-adjusted life benefit could be even greater.⁴ Society must sufficiently value and reward progressive, step-wise innovations as well as breakthroughs so that advances in breast cancer and colon cancer research can be replicated across all tumor types. Progressive gains lead to breakthrough treatments.

To ensure optimal advances in cancer therapies, both progressive and breakthrough, logistical, bureaucratic, institutional and regulatory obstacles to innovation must be reduced to the greatest extent possible. Documentation and auditing mandates, divergent local institutional review board (IRB) requirements for consent forms and protocols, duplicative and conflicting standards, increasing

regulatory requirements and delays in review decisions are often unnecessarily burdensome without providing an additional measure of safety for study subjects or a commensurate gain in the quality of clinical research. Clearing such hurdles will require a nationwide strategic plan among all stakeholders to establish priorities and coordinate uniform, or at least coherent, requirements among research institutions and across government agencies.

2) Improving the Value of Innovation by Bolstering Funding Opportunities
Investment in medical innovation will improve health care outcomes for patients and lower long-term health costs, relieving financial pressures on federal programs such as Medicare and Medicaid, and lowering costs borne by employers for the health care of their employees. However, these benefits will require all stakeholders to make a solid financial commitment to medical innovation. This commitment will necessitate increased funding for basic and clinical study – particularly through Congressional appropriations for government research – and heightened awareness by all stakeholders of clinical research opportunities. Public and private sector stakeholders need to be united in their dedication to fund the basic research that provides the foundation for the discovery of innovative treatments and therapies.

The significant, sustained research in basic life sciences led by the National Institutes of Health (NIH) is critical to the biomedical ecosystem and the Food and Drug Administration (FDA) plays a critical role in finding creative ways to promote and review medical innovation. However, further government investment is needed to increase the transparency, efficiency and effectiveness of these agencies while mitigating regulatory impediments.

3) Enhancing Delivery of Innovation through Improved Communication and Coordination Between Providers and Patients
By enhancing enrollment in clinical trials, we will ensure that medical science advances as expeditiously as possible and that patients obtain access to cutting-edge cancer treatments that are affordable, albeit experimental. To realize these benefits, physicians must be aware of clinical trials being planned and conducted, and they must share that information with their oncology patients for whom such trials are appropriate. Coordination among oncology researchers, treating oncologists and patients is essential so that all parties are educated about ongoing research.

In addition, regulatory policies must be established to ensure that payers cover clinical trial costs for patients, as required in statute and Executive Order. Payment and reimbursement for treatments must be adequate and timely, and cost sharing must be kept at a level that ensures access will not be unduly limited. Delivering innovative cancer medications in this manner can result in better outcomes and better quality of life for patients, advance cancer research and alleviate some of the long-term financial strain on our health care system.

In summary, the biggest obstacles to expanding research for cancer treatments, particularly new drug therapies, is the substantial research and development costs that must be incurred by pharmaceutical manufacturers, providers and other private research institutions in developing potential treatments. There is very little governmental or publicly funded cancer research currently in the United States. Rather, the overwhelming majority of all cancer research and development is undertaken by private entities. The CIC recommends that to alleviate these obstacles federal and state entities invest in more research, perhaps in tandem or in collaboration with pharmaceutical manufacturers and other public and private entities while developing incentives for innovative research. We also recommend that the federal government develop incentives to urge collaborative efforts among pharmaceutical companies and other researchers. In addition, the CIC recommends that patient access to clinical trials be expanded by the federal government and that there be better publication
of the availability of clinical trials in order to boost enrollment. Lastly, the CIC urges the federal government to continue to work on expedited regulatory pathways through the FDA so that potentially life-saving treatments can reach otherwise terminal patients faster.

Throughout our nation’s history, public and private stakeholders have joined forces to tackle some of the world’s largest health crises, notably polio, CML, testicular cancer, lung cancer, childhood leukemia and HIV. Melanoma, a form of skin cancer, is the latest example of a tumor type to see an increase in treatment options available due to innovation in cancer treatments, such as targeted drugs, surgeries, and immunotherapies.\(^5\)

Today, thanks to biomedical innovation, most patients diagnosed CML during the chronic period can look forward to a life expectancy similar to a non-CML patient. However, it took about 30 years of basic scientific research and progressive treatments to allow for this breakthrough therapy for CML patients. We can do better together.

Meaningful advances in cancer therapy will happen only through policies that recognize the unique role that medical innovation plays in improving the lives of Americans. Stakeholders must work together to sustain investment in innovation that will expedite research and development, regulatory review and approval processes, without sacrificing safety and efficacy.

Medical innovation saves lives, enhances personal quality of life, improves public health and productivity, creates jobs and adds economic value to society. Even more important is the “value of hope,” which exists when patients feel confident that there are treatments available to ensure they will live longer and better lives after a cancer diagnosis. A recent survey indicates that 77 percent of cancer patients prefer treatment that provides hope of breakthrough success over safer treatments with more certain outcomes. These results clearly indicate that, when determining the value of innovative treatment and therapies, the patient’s hope must be considered when determining whether an investment in innovation should be made, and when making cost/effectiveness determinations for expensive treatments.

Progressive innovation may not always constitute a cure, but it can lead to a longer length of life, in addition to reduction in pain and adverse effects, ease of administration and increase in productivity and quality of life. These gains are important for patients fighting chronic, debilitating and life-threatening diseases, and they need to be appropriately valued by all stakeholders during the funding, innovation and review processes.

The successes experienced in the field of HIV research, as well as breakthrough innovations related to childhood leukemia, have laid a blueprint that can - and should - be replicated by researchers, academics, developers, government officials, and private and non-profit stakeholders in reaching our goal of turning cancer from a deadly illness to a manageable chronic disease, and ultimately eliminating it entirely. Innovative therapies have transformed HIV from a death sentence to a manageable chronic disease; this achievement has been facilitated by truncated development times for HIV medicines.

The development of innovative cancer treatments needs to be similarly streamlined in order to control costs. Cancer treatment represents a far higher percentage of health care spending relative to the prevalence of cancer in the United States population. Drug costs will generally decrease as patented medicines lose exclusivity; generic drugs currently account for approximately 85 percent of all prescriptions dispensed, but less than a third of prescription drug spending. Between 2014 and 2016, there are three widely used oncology medications going off patent, which will lead to savings within the patient and health plan communities as generic equivalents become available. Generic drug use will account for greater savings over the next three years as billions of dollars worth of brand name medicines lose patent protection. Further savings can be realized by development of competitive innovative products within the same therapeutic categories. Regulatory latitude and incentives for progressive innovation will help mitigate this risk to innovation, and a move to smaller trials and adaptable protocols will help further. However, it is important to keep in mind that there are

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8 Economic Value in cancer care is the worth of therapy that goes beyond the price and incorporates the value of extended life (QALY), quality of life, enhanced productivity, added income made possible for the individual and decreased health system costs. From the sponsor’s perspective, economic value is often used within the context of discussing return on investment in research and development.


8 See id.


INNOVATION IN CANCER CARE – SECURING ITS FUTURE

Science of Innovation: Streamlining Logistical Obstacles

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benefits to patent exclusivity. The Institute of Medicine has studied patent exclusivity laws related to pediatric treatment and found that it has incentivized research and development in children, and increased the number of available clinical trials.\(^{10}\)

**Understanding the Risk of Innovation**

Research and development of innovative cancer treatment is an expensive though worthwhile endeavor. There is no question that the absolute costs associated with medical innovation are high; innovation is difficult, and expensive. The biopharmaceutical industry spent an estimated $33 billion on research and development in 2001, and this number has grown by an estimated 15 percent per year in the years since,\(^{11}\) with an estimated $48.5 billion spent on research and development in 2012.\(^{12}\) Approximately 40 percent of these dollars are spent on the administration of clinical trials.\(^{13}\)

In 2012, developers invested an estimated $48.5 billion in research and development, which represents the majority of all biopharmaceutical research and development spending in the United States.\(^{14}\) Even with the substantial investment and commitment to innovation, approximately 95 percent of the experimental medicines that are studied in humans ultimately fail to meet standards for safety and efficacy.\(^{15}\) A company will spend approximately one billion dollars and take an average of seven years to bring a fast-tracked drug to market,\(^{16}\) and on average only two to three of ten marketed therapies produce revenues sufficient to fund a manufacturer’s development program.\(^{17}\) Development is an uncertain process, with an estimated 19 out of every 20 drugs in development never making it to market.\(^{18}\) Unfortunately, overall government spending on health and medical research has declined by close to 20 percent since 2010, placing more of the overall financial burden for research and development on the private sector.\(^{19}\) In order to allow medical innovation to continue to thrive and facilitate breakthrough innovation, there must be greater investment by the public sector to support future development.

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\(^{13}\) Meadows at 1.


Cancer treatment accounts for approximately five percent of the total health care costs in America.\textsuperscript{20} The National Institutes of Health (NIH) estimated in 2009 that the annual costs of cancer were $216 billion, with direct medical costs amounting to $86.6 billion and indirect mortality costs—such as the loss of productivity due to premature death—totaling $130 billion.\textsuperscript{21} According to Centers for Disease Control and Prevention (CDC), each year in the United States, chronic disease such as heart disease, stroke, cancer, and diabetes cause seven in 10 deaths and account for about 75 percent of health care spending.\textsuperscript{22} In 2012, health care expenditures reached $3 trillion dollars,\textsuperscript{23} and according to the Centers for Medicare and Medicaid Services (CMS), that number is expected to rise to approximately $4.7 trillion by 2021.\textsuperscript{24} From 2001 to 2005, the annual cost of cancer treatment in the United States was approximately $48 billion per year. It is estimated that while oncology patients under active treatment represent only one percent of a payer’s patients, the care of these patients accounts for approximately 10 percent of costs.\textsuperscript{25} 

Prescription drug expenditures are expected to rise from $277 billion in 2012 to $483 billion in 2021,\textsuperscript{27} meaning the prescription drug spend as a percentage of overall health care expenditures is estimated to rise from 9.2 percent in 2012 to 10.3 in 2021. Private health insurance paid for only 36 percent of this annual expenditure.\textsuperscript{28}
**The Role of Generic Medications**

Within the prescription drug spend, generics play a role in keeping overall drug prices in check,\(^2^9\) accounting for approximately 85 percent of current market share, with some estimates showing this number rising to 92 percent by 2020.\(^3^0\) While brand medications made up about 15 percent of prescriptions in the last year, they accounted for 73 percent of the total drug spend nationwide.\(^3^1\) A bolus of generic oncology products are entering the market and will result in significant savings to cancer patients who need these medications. Oncology products that have lost or are losing patent protection between 2013 and 2015 include apreptin (Merck’s Emend), capecitabine (Roche’s Xeloda), hydromorphone (Mallincrodt’s Exalgo), imatinib (Novartis’ Gleevec), oxycodone (Purdue Pharma’s Oxycontin), temozolamide (Schering/Merck’s Temodar) and zoledronic acid (Novartis’ Zometa).

At the same time, this influx of generics will mean less revenue for sponsors to invest in future innovation through research and development. In addition, competing sponsors will continue to release multiple branded drugs, providing more brand options and, conceivably, more competitive brand drug pricing. For example, Novartis and Eli Lilly are in the process of developing a medication that will compete directly with an experimental Pfizer product for use in the treatment of breast cancer. Multiple entries within a single therapeutic category can be expected to contain costs by creating a more competitive pricing environment.\(^3^2\) Given this dynamic, absent increased regulatory latitude and incentives related to stepwise, continuous innovation, it could potentially become increasingly difficult for sponsors to raise capital for future advances within the current structure, which could have a negative impact on investments in innovation.

**Recognizing the Value of Innovation**

It is important to keep in mind the relative gains resulting from upfront investment. It takes a substantial financial commitment to produce innovative cancer medications, but the value, measured in human life, not only justifies the investment, but re-coups it as well. Progressive innovation impacts public health, and there exists a need for all stakeholders to work collaboratively to further define value in broad, 21st Century terms within the context of cancer innovation. Within value we must include longevity of life, greater productivity, and most importantly, the value of hope brought to patients and their families. It cannot merely

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30 See Wyatt, supra.


be an economic calculation. Progressive innovations extend life for patients, sometimes long enough for them to receive the benefit of a subsequent breakthrough innovation that could improve quality of life and ultimately lead to survival.

Today, thanks to biomedical innovation, most patients diagnosed with CML during the chronic period can look forward to a life expectancy similar to a non-CML patient. However, it took about 30 years of basic scientific research and progressive treatments to allow for the breakthrough therapy for CML patients. The first breakthrough in CML research came with the discovery of the Philadelphia chromosome in that city in 1960. Not only did this discovery allow scientists to prove conclusively that there is a genetic link to cancer, but it would be the basis for all future treatments of the disease. Still, until the 1980s patients with a CML diagnosis had no cause to be optimistic: the median survival rate from diagnosis was about five years, and the best drugs available caused infertility in patients. Through the next two decades, advances in interferon treatment and stem cell research led to better outcomes for patients, although treatment was still imperfect. Clinical trials in the early 1990s revolutionized CML therapy. Oncologist Brian Druker, a researcher at Oregon Health and Science University, partnered with Ciba-Geigy (which later became Novartis) to conduct the first clinical trials on a class of clinical compounds that promoted anticancer activity. As Druker noted, “[u]sually in a phase 1 clinical trial, if you see a 20 percent response rate, that’s remarkable. We had a drug that was extremely well tolerated and had a 100 percent response rate. It was incredible to see this unfold.” With this drug, later called Gleevec, the seven and 10-year survival rates for CML are now about 80-85 percent.

In addition to the invaluable impact that innovation has on hope, there are substantial economic gains to be had as a result of innovative cancer treatments. Over the course of the 20th Century, cumulative gains in life expectancy amounted to an economic gain of $1.2 million per person. Between 1970 and 2000, increased life expectancy added about $3.2 trillion per year to national wealth, an uncounted value equal to about half of average

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annual GDP over the period. The potential gains from future innovations in health care are just as large, if not larger. Even a modest 1 percent reduction in cancer mortality would be worth nearly $500 billion.36

**Innovative Treatments, Meaningful Gains**

There have been substantial gains made within the field of cancer innovation, but much progress remains to be made. Between 1988 and 2000, life expectancy for cancer patients increased by approximately four years, meaning the gains in cancer survival during this period created 23 million additional life-years and roughly $1.9 trillion of additional social value.37 These numbers suggest that the average life-year was worth approximately $82,000 to its recipient; health care providers and pharmaceutical companies assumed between 5 and 19 percent of these costs, with the remainder being borne by patients themselves.38 A study completed in 2011 shows that new cancer drugs introduced over the past 30 years have increased the life expectancy of cancer patients by almost one year, and since some products introduced in more recent years have shown better tolerability than traditional chemotherapy, the quality-adjusted life benefit could be even greater.39 Progressive innovation must be sufficiently valued so that advances in breast cancer and colon cancer research can be replicated across all tumor types. These progressive gains lead to breakthrough cures.

In addition to increased life expectancy resulting from improved treatments, innovative treatments have led to a substantial reduction in cancer mortality rates since 1990. An example of science leading to better understanding of disease and therefore improving targets for treatment is in advanced non small cell lung cancer. Ten years ago, we knew of two genetic mutations responsible for lung cancer growth; today, we know of at least twelve genomic drivers, and the list may continue to grow. Lung cancer is the most lethal cancer in the US, killing over

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37 See Lakdawalla et. al. supra.
38 See Id.
39 See Lichtenberg supra.
160,000 people in 2012 and accounting for approximately 28 percent of all cancer deaths. However, the outlook for treatment of lung cancer is more hopeful, as we have seen significant advancement due to innovative developments in detection and treatment options. Recent advancements in combating lung cancer have included targeted therapies through personalized medicine, therapeutic vaccines, new chemotherapies, maintenance therapies, chemoprevention, and increased efforts at early detection. To maintain these trends, innovation must be protected and incentivized.

Similar promise has been shown in the treatment of melanoma. Patients with advanced melanoma have seen dramatic advances in cancer treatments and drug discovery in three areas due to innovation: targeted therapies, immunotherapy, and viral oncolytic therapy. With these powerful and innovative therapies, the majority of melanoma patients are experiencing a durable major response, compared to less than a 20 percent response rate that was short-lived for all the drugs available before 2010. There are now three FDA-approved drugs in melanoma patients whose metastases have the BRAF V600E mutation. Each of these drugs increases progression-free survival when used alone. More importantly, the combination of two of these drugs was more effective than either drug alone, and interestingly, the toxicity was lower with the two drugs together than either alone.

There are now four new and effective monoclonal antibodies that are proving effective using a unique strategy of inhibiting melanoma cells or their foreign antigens that would otherwise inhibit a beneficial cytotoxic immune response. This class of agents—termed “checkpoint inhibitors”—has the most dramatic and durable reduction or elimination of advanced tumors that has been demonstrated in the history of melanoma drug therapy. The FDA has approved one antibody, while 3 other agents (anti-PD1 and anti-PDL1) are in late stages of clinical development. Finally, another strategy used viruses as a “vector” that homes to melanoma cells and delivers a knockout dose of a biological agent termed GM-CSF. The drug, called TVAC, is an investigational oncolytic immunotherapy designed to selectively replicate in tumors (but not normal tissue) and to initiate an immune response to target cancer cells that have metastasized. This drug is injected directly into tumors where it replicates inside the tumor’s cells causing the cell to rupture and die. The rupture of the cancer cells can release tumor-derived antigens that can stimulate a system-wide immune response where white blood cells are able to seek out and target cancer that has spread throughout the body. We must ensure that collaboration between developers and FDA continues, and that innovation is able to thrive moving forward, so we can continue to see these types of promising breakthroughs.

In the last 15 years, 50 million life years have been saved due to breakthroughs in treatment and innovation. These cumulative improvements in the health of those diagnosed with cancer positively impacts job creation,

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taxes paid, productivity, etc., and validates much of the upfront investment in research and development. Improvement in patients’ quality of life, along with the resulting economic benefit, needs to be taken into consideration when determining the viability of private and public sector enthusiasm for, and investment in, the research and development that is required if we are to continue adding to these tremendous gains. Some health economists have estimated that the economic gains from declining cancer mortality rates in the United States from 1970 to 2000 added value to society of more than $3 trillion a year, in addition to substantial savings to health care costs. Further breakthroughs will have similar results, with a substantial portion of health care savings to be realized within the Medicare and Medicaid programs.\(^{43}\)

**Recommendations for Improvements to Innovation**

Given the obvious value that medical innovation brings to cancer patients, more needs to be done to improve access to innovation and increase the speed with which innovative therapies are brought to the market. There is room, and precedence, for improvement: while cancer medications average nine years, HIV drugs generally average two years from discovery to approval, while cancer medications average nine years. Given the importance of treating and eliminating cancer, and its prevalence in our society, steps should be taken, in partnership with industry stakeholders and government officials, to expedite these processes.

**Collaboration among Developers**

An encouraging development within the pharmaceutical industry is the announcement of a collaborative effort among developers to share research in order to expedite the development of breakthrough treatments. A not-for-profit entity named TransCelerate\(^{44}\) seeks to increase the number of innovative medications, and improve efficiencies within research and development, with the goal of lowering costs by eliminating repetition and executing streamlined clinical trials. This effort could be the first step in a more efficient and less costly development process, which will benefit patients both through expedited access to breakthrough therapies, and lower out-of-pocket costs.

A specific example of such collaboration is a recent announcement that Merck, Pfizer Amgen and Incyte will collaborate to determine how Merck’s immunotherapy cancer drug performs in combination with other treatments produced by other developers.\(^{45}\) This endeavor comes on the heels of findings showing that while some immunotherapies have generated good results on their own, combining these treatments could produce an even greater result. This type of collaboration is necessary as we move to a future where breakthrough combination therapies become more prevalent, and more effective in treating cancer.

The Institute of Medicine has supported\(^{46}\) efforts by the NCI to build on their progress, in collaboration with the Patient-Centered Outcomes Research Institute (PCORI), created as part of the Affordable Care Act to work with

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\(^{43}\) See Murphy and Topel, supra.


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government and private stakeholders to develop a common set of data elements to capture patient-reported outcomes in clinical trials and other studies, to be used to further advance research and development. This is yet another example of the type of collaboration that will greatly benefit patients’ access to breakthrough treatments and innovative therapies.

**Recommendations for Improved Regulatory Pathways**

Any effort to expedite the approval process\(^{47}\) will require early and regular engagement between trial sponsors and the FDA.\(^{48}\) Using more standardized review processes would allow for an element of predictability that will translate to more certainty for innovators, and should in theory lead to quicker approvals, and denials.\(^{49}\) It has been observed that the FDA appears to be “approving more drugs, even working with companies to help remove red tape and speed drugs for particularly serious diseases to market.”\(^{50}\) It is important that stakeholders continue to work with FDA to build on this progress, and to continue to reinvent their own processes as well. Many companies are currently working to restructure their research and development laboratories\(^{51}\) with an eye toward a more static, streamlined process that should ultimately lead to quicker approvals.

Expediting the approval process is important, but safety and efficacy must remain at the forefront of any process or policy modifications related to the approval process. The requisite data need to be provided to FDA in order to gain approval, and FDA must make these review processes as collaborative and user friendly as possible in order to encourage availability of breakthrough treatments while also protecting patients. FDA has enhanced its offerings related to the review and approval of breakthrough drugs, but there are still legislative and regulatory vehicles that need to be examined in order to further expedite and streamline the processes through which these breakthroughs are provided to patients. The agency has developed Subpart E and Subpart H procedures, parallel studies, compassionate INDs and other processes to expedite patient access to innovative new therapies before they are approved for general use. The challenge is to allow patients expedited access without undermining enrollment of subjects in formal randomized clinical trials.

One way to improve the approval process and expedite the development of innovative cancer therapies is to develop a system through which outcomes data could be shared among government and non-government stakeholders. The creation and maintenance of a more useful, centralized data hub wherein data can be accessed and shared among public and private sector stakeholders could facilitate additional research and innovation. Even outcomes and data related to failed trials would provide useful information that could lead to

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\(^{50}\) Herper, supra.

\(^{51}\) Id.
future breakthroughs. Questions remain regarding such data sharing. Specifically, what would the data look like? It would likely need to be more substantive than the publicly facing data currently available on the government’s clinical trials website, but how willing would developers be to make proprietary data available for public viewing? Additionally, in what format would the data need to be made available to ensure that it would be both accessible and usable for researchers and sponsors in order to facilitate innovation? Current law allows FDA and CMS to conduct concurrent, parallel review processes (data sharing), although this process has not yet been well defined and there exist issues related to patents that should be clarified in order to improve results.

One promising development is the recent news that the NIH and 10 major pharmaceutical companies are planning a five-year, $230 million collaboration to exchange data related to several disease areas. This represents a unique agreement since pharmaceutical companies generally are hesitant to share their research. Under the collaboration agreement, “no pharmaceutical company can use any discoveries made under the project to further their own research on medications until the project reveals that discovery data to the public.” The collaboration, coordinated by NIH, ideally will set a precedent as a model option that can be considered in order to facilitate expedited cancer care innovation.

**Creative Regulatory Approaches to Innovation**

Creative thinking among stakeholders could lead to new and additional innovative ideas to accelerate research, increase patient enrollment in clinical trials and improve access while keeping in place safeguards to ensure safety and efficacy. Through the coordination and collaboration of the Cancer Innovation Coalition, we hope to begin addressing specific proposals put forth by national and international stakeholders.

Greater coordination and integration with sponsors and other stakeholders, and more engagement from FDA on progressive advances, will expedite the process through which innovative treatments become available for patients. Such advances could include research strategies for treatments that offer progression-free outcomes and improved quality of life. Advances are also needed to expedite research into specific medications targeting specific types of tumors. Personalized medicine, genetic biomarkers, etc. could increase the speed with which trials are conducted and safe, effective therapies can be approved and brought to the market. The Novartis Simplified IRB process, discussed below, is a good example of such coordination and integration and could serve as a model for other developers.

**Public and Private Partnerships**

Strengthening public and private partnerships will remain the most important element of facilitating greater innovation in cancer care. Patient groups, industry stakeholders, payers, and government agencies - including NIH, CMS and FDA - need to collaborate more with respect to data sharing related to outcomes, patient quality of life and other relevant variables. It is equally important to pursue improved data sharing and publication of

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53 Id.
trial outcomes when trials fail to produce positive results. This information is critical to advancing scientific knowledge and, thus, innovation.

The valueofinnovation.org project is one example of a collaborative effort to improve innovation, managed by the Center for Medicine in the Public Interest. The group’s mission is to bring stakeholders together and facilitate innovation, and the project is seeking to gain as many partners as possible. Building upon the idea that a more collaborative environment and more investment from stakeholders will result in better medicine and improved access for patients, the group seeks to foster this collaboration in an effort to expedite the speed at which innovative cancer treatments are developed. Such a partnership will be successful only if it includes input from academic, government, legislative, nonprofit and industry stakeholders, all participating equally and concurrently in an effort to make contributions toward new innovative treatments and, ultimately, a cure.

**Recognizing the Value of Incremental Progress**

A general and widely applied definition of what is considered “breakthrough enough” is inappropriate in that it fails to adequately consider the varying science and complexities of particular types of cancer. An approach that relies exclusively on a breakthrough benchmark may have the unintended consequence of discouraging research and development into particularly rare tumor types for which the prospects for breakthrough innovations are comparatively small. There is no question that expedited approval leads to innovative treatments and future breakthroughs. Building on the successes of treatments will lead to even greater innovation, as subsequent use of medications for specific cancer types will ultimately lead to future breakthroughs or additional gains based on the results gathered from the use of those medications in certain populations.

There are additional steps through which innovation can occur more rapidly without sacrificing safety and efficacy, such as approvals of therapies for specific types of cancers. Faster approval of medications for specific tumor types could expedite access to medications and provide patients with additional treatment options, while still ensuring that such innovations can be used safely to treat these specific conditions. It is important to work with regulators and legislators to emphasize that a strict interpretation of the success of a particular treatment cannot be based on a generic measure of success. There are many instances where in a particular tumor type has not seen any advancement in years, or even decades, so a progressive innovation in these instances, even if small, constitutes a major development within the context of that particular tumor.

A system wherein FDA could target approvals for specific tumor types would require substantial education and involvement of providers; they would need to be educated about possible technical use of rare medications appropriate for a very specific segment of their patient population. It also would be important for the nonprofit patient advocacy community to remain involved and engaged on this issue to help ensure that safety and efficacy remain paramount. A reeducation of physicians to ensure understanding of any new trial processes will be necessary, as will a continuing educational program to ensure that providers are aware of the most recent developments.

**Value of Medical Innovation: Funding for Innovation Opportunities**

We cannot realize the benefits of medical innovation without first making the commitment to adequately invest to ensure that innovation can continue to occur. Public and private sector stakeholders need to be united in their commitment to fund innovative treatments and therapies, bolstered by the knowledge that an investment
made today will allow us to realize additional gains tomorrow. We must ensure that funding for clinical trials is available in order to expedite the process through which safe and effective treatments are moved to the market. Further, we must ensure that patients have the opportunity to enroll in such trials without having to worry about cost. Investment in medical innovation will improve health care outcomes for patients and lower long-term health costs, relieving financial pressures on federal programs such as Medicare and Medicaid, and lowering costs borne by employers for the health care of their employees.

The Food and Drug Administration Safety and Innovation Act (FDASIA) has substantially focused attention on the importance of therapeutic innovation since its enactment on July 9, 2012. The outstanding question is whether FDASIA’s provisions can be expanded such that they serve to modernize the accelerated review process in a way that enhances medical innovation. Breakthrough Therapy Designation, FDA’s new expedited drug development tool, should better enable FDA to assist drug manufacturers to expedite the development of drugs that hold promising preliminary clinical evidence of superiority to available therapies for patients with serious diseases. However, it remains to be seen whether this designation is used to bring novel therapies to patients quicker. Other FDASIA provisions, including incentives for antibiotic development, increased communication, New Molecular Entity review processes and novel approaches to risk-benefit assessment all hold similar promise. Again, the question remains: will these provisions result in less regulatory burden and quicker approvals?

**Investment Now for Returns through the Workforce**

A healthier workforce leads to increased productivity and lower health costs for both employees and employers. Therefore, it is important for employers to have a vested interest in the availability of and reimbursement for clinical trials and innovative therapies. Approximately 40 percent of all Americans diagnosed with cancer each year are working adults. 54 NIH estimated the dollar value of lost productivity due to premature cancer deaths in the United States in 2005 at $134.8 billion (estimating about 600,000 cancer deaths that year). 55

The Affordable Care Act (ACA) has encouraged employers to adopt wellness initiatives aimed at encouraging healthier employee lifestyles and preventive care, which leads to lower long-term costs incurred during treatment of chronic disease. Government is at the forefront of wellness initiatives in the Federal Employee Health Benefits (FEHB) program, with pilot programs at agencies such as the Department of Health and Human Services (HHS) and the Office of Personnel Management (OPM) aimed at lowering long-term health care costs by incentivizing wellness, encouraging preventive care, and coordinating costs to improve utilization.

A 2009 study of 64,000 employees with cancer outlined the major financial impact on employers and employees due to missed work for cancer treatment. 56 On average, employees missed 26 workdays because of traditional chemotherapy or radiation treatment, and 18 days due to treatment and management of side effects. Employees who serve as caregivers to cancer patients are often forced to miss work in order to transport and accompany loved ones to appointments. An estimated 77 percent of cancer patients report being accompanied

by a caregiver when attending scheduled treatments. If patients can gain wider access to oral anticancer medications, and are able to take these medications at home and perhaps even continue working during treatment, employers will have more options to avoid the costly issues of absenteeism, presenteeism, turnover, short and long-term disability and worker replacement costs. It is for these reasons, along with the general well-being of their workforce, that employers must continue to emphasize wellness among their employee populations, and provide avenues through which employees can receive affordable treatment when faced with a disease such as cancer.

Access to innovative therapies improves outcomes and quality of life, and with that comes improved productivity and retention for employers, both of which result in measurable economic benefit. The economic gains realized as the workforce remains healthy and productive is sufficient enough to justify the upfront cost of providing comprehensive health coverage packages, and incentivizing wellness and prevention. Employers’ investment in proper treatment will improve stability in the workforce, and the value of such stability is realized directly by not only patients through improved quality of life, but by the employers themselves in the form of economic gains related to productivity.

In “An Employer’s Guide to Cancer Treatment and Prevention,”57 the National Business Group on Health made a number of recommendations for pharmacy benefits to ensure that employees would receive optimal care and increase their chances to return to work and remain productive and healthy. The Guide calls for “Reasonable out-of-pocket thresholds to ensure that “cost is not a barrier for patients to obtain medications needed to treat their condition, including maintenance and supportive care drugs.” The Guide also recommends that “per-prescription copayment and/or coinsurance requirements should be established at a reasonable level.” To further enhance adherence rates, the Guide recommends that specialty pharmacy vendors “implement programs to counsel individuals who are prescribed oral oncology drugs or self-injectables to reduce the prescription abandonment rate” and “monitor patients on long-term treatment regarding failure to fill or refill prescriptions.”

Did you know that:

- Patients and sponsors spend a substantial amount of money on research and development, sometimes with no gain to show for it.
- NIH does not develop the cures, and sponsors pay a substantial amount of money on research and development, sometimes with no gains to show for it.
- Therefore, stepwise, progressive innovation is critical and its importance should be highlighted more than it is, and supported through enhanced policy emphasis as well as increased funding.
- There are many times when research and development does not lead to its intended goal, despite substantial financial investment.
- However, the discoveries made during these failed endeavors often lead to incremental gains, so their importance cannot be disregarded.
- Though incremental innovation does not directly result in a breakthrough therapy, breakthrough therapies are often the result of several progressive innovations that make the larger discovery possible.
- In addition, if progressive innovation offers the possibility of improved quality of life for terminally ill patients, or the potential that someone suffering from a life-threatening disease will survive long enough to make it to the next breakthrough; it is difficult to argue that it is not worth an upfront investment.

The role of disease management remains as important to patients as the need for improved access to breakthrough therapies. Often, it is the ability to manage chronic conditions, or prolong life through stepwise therapies, that allows patients to live long enough to benefit from a breakthrough therapy or new clinical trial that was unavailable when they were first diagnosed. As we continue to build on the progress made in managing certain types of cancers such that patients are able to live fulfilling, productive lives as they progress through coordination of care and adherence to protocol, we should keep in mind the added value that this improved quality of life brings to patients, and the added years of life they are able to gain as a result. We also must remember that these treatments serve as a bridge to future protocols that could ultimately lead to a cure.

Stakeholders need to work collectively to re-define what is considered “innovation” so that the word is not misunderstood as being limited to breakthroughs. Progressive innovations do indeed produce life-saving treatments for many patients. FDA must keep this in mind during trial design and the review process, payers should keep this in mind with respect to reimbursement, and patients should keep this in mind when seeking treatment. All stakeholders must have a voice in speeding innovations. Collaborative efforts should be made to preserve access to clinical trials and innovative/breakthrough treatments, in addition to working to speed the process. Telling the story of continuous innovation and advances and relating it to real patient experiences is what will convince stakeholders at large that the upfront investment is worth making. Bolstered by the nonprofit community, an even stronger patient voice is needed within the context of discussions related to access and availability of clinical trials, because the risk associated with clinical trials and innovative products and procedures is borne by patients. As such, they deserve a seat at the table when discussing these issues.

**Increasing Investment in Research to Facilitate Innovation**

There is more to the development of innovative cancer treatments than an expedited review process; innovation requires investment.

Investment by venture capitalists in the biotechnology sector has declined since 2007, and government investment has declined in the last several years to levels not seen since 2004.58 It is important to continue to encourage the involvement of venture capital in cancer research, to mitigate the difficulties resulting from current limitations in government funding and future uncertainty with respect to government appropriations. A good example of such an endeavor is the Cancer Research Clinical Accelerator program,59 which allows investors to provide capital to promising clinical trials in exchange for the potential for future earnings based off the success of the product. These programs are vital in ensuring that research dollars are available today to facilitate the progressive and breakthrough

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treatments of tomorrow. This program, along with similar endeavors, seeks to “accelerate the development of better immunotherapies for more types of cancer.”

While most of the attention is given to breakthrough innovation, progressive innovation remains almost equally important. Progressive innovation leads to breakthrough innovation and can extend life for patients, sometimes long enough for them to receive the benefit of a subsequent breakthrough innovation that could improve quality of life and, ultimately, survival. What are stakeholders – government and private – willing to invest today in order to save lives and produce health care savings tomorrow? Moreover, attempts at breakthrough innovations often fail to achieve the stated goal, but end up leading to progressive innovation. It is for these reasons that progressive innovation should be rewarded and better understood. Better communication between stakeholders and FDA/CMS officials is needed for the parties to be better able to

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60 See Id.
61 Id.
understand the extent to which progressive innovation benefits patients, and justifies the cost of development. Many of today’s breakthroughs originate from investments made ten years ago.

Percent of New Cancer Cases by Age Group:64

There are a substantial number of patients willing to enter clinical trials, so theoretical enrollment generally is not a problem. From a patient perspective, once a diagnosis is received there are several hurdles related to access to innovative cancer treatments that must be addressed. Adding to the problem is the fact that there are major disparities in cancer outcomes in minority and among those with low socioeconomic status, as well as those who are underinsured or uninsured.65 One of the barriers to treatment is the lack of cooperation between sponsors and payers with regard to responsibility for reimbursement for certain treatments.66 With respect to clinical trials, discussions between payers and sponsors are needed to determine who should reimburse certain services. There is general agreement among stakeholders that the patient should not be faced with substantial out-of-pocket costs as a result of treatments incurred within a clinical trial, so the reimbursement structure needs to be resolved.

**Paying for the Administration of Innovative Treatments**

Reimbursement for costs incurred is a real issue, and the question is whether sponsors or payers should cover these costs. There are costs for treatments and procedures that are unique to trials, and should arguably be borne by sponsors, but there are also more basic costs, such as testing and monitoring, which are generally reimbursable outside of the context of a clinical trial, yet generally will not be covered during a trial.

since a payer will deem them outside the standard of care. The question regarding who pays may be a difficult one, but the patient should not be forced to absorb these costs.

There already exists much confusion for patients in regard to current payment obligations. For example, many patients do not understand that under certain plans there exist separate deductibles for medical benefits and pharmaceutical benefits, causing patients to pay more than they previously expected to be responsible for. Furthermore, currently these deductibles can both be quite large, totaling up to $12,700 for patient responsibility in the commercial market, with this being reduced by half in 2015 through the ACA. Complicated payment systems in the commercial market that have separate deductibles based on type of treatment receive makes receiving a difficult process such as receiving cancer care even more confusing, expensive, and difficult. These concerns are not limited to commercial products, with a similar dynamic existing for Medicare Part B and Part D beneficiaries.

The Role of the Regulating Agencies

In order to facilitate a reasonable structure through which payments and reimbursements can be determined for the betterment of patients, regulators need be involved in these discussions. A recent FDA guidance document states that, “although FDA determines whether a sponsor may charge for an investigational drug used in a clinical trial or expanded access program, FDA does not decide how that charging is to be carried out. FDA anticipates that the sponsor would ordinarily charge a patient directly, or would charge a third party payer if reimbursement were available. FDA notes that it has no authority to require that the Centers for Medicare and Medicaid Services (CMS) reimburse for investigational drugs for which FDA has permitted charging. Similarly, FDA has no authority to dictate reimbursement policy to private health insurance providers.”

Medicare pays for routine costs of items and services in covered research studies. According to CMS, covered items and services include room and board for a hospital stay that Medicare would pay for even outside a study, operations to implant an item being tested, and treatment of side effects and complications that may occur as a result of the study. Medicare will not pay for the new item or service that the study is testing unless Medicare would cover the item or service outside a study, items and services the study provides, items or services used only to collect data and not used in direct health care, coinsurance and deductibles.

Delivery of Innovation to Patients: Access, Communication and Coordination

The success of the above strategies will be possible only to the extent that all stakeholders, including patients, have the capacity to become aware of the developments and the new treatment options. Patients also need to be able to afford and have access to the medications that result from these innovations.


68 FDA Guidance for Industry, supra at 3.

Payment and reimbursement for treatments must be adequate and timely, and cost sharing must be kept at a level that ensures access will not be unduly limited. Delivering innovation to patients will result in better outcomes and better quality of life, while simultaneously serving to alleviate some of the long-term financial strain on our health care system. Improving the payment structure such that innovative treatments and therapies are reimbursable, and therefore accessible, to patients is critical. Empowering patients to make decisions about their health care, and choose safe and effective innovative treatments, will improve long-term outcomes, and facilitate progress in the search for a cure for cancer. An example of problematic policy is the Oregon Medicaid approach related to costly end-of-life treatments, wherein treatment decisions are made not based on patient need, but based on an analysis of cost and effectiveness. This approach does not take into account the need for patient engagement, nor does it capture the value of extending and improving quality of life, perhaps long enough to reach the next breakthrough therapy. There are examples of successes in the areas of polio, HIV and childhood leukemia; drawing from those successes, a similar result can be achieved in the fight against cancer.

The Role of Payers and the Need for an Appropriate Coverage and Reimbursement Framework

It is imperative that payment reform value and provide incentives for innovation. Specifically, payers need to ensure that reimbursement and benefit design allow patients and physicians to select the therapies that are optimal on the individual patient level, taking into account the patient’s treatment goals and preferences. Excessive cost sharing, utilization review techniques such as step therapy and fail-first restrictions inhibit patients’ ability to access needed medications and create barriers to innovation.

Timely coverage should be available for both incremental and breakthrough innovations. Medicare Part B drug coverage is an example of a policy paradigm that has some important protections that should be maintained, where cancer patients have coverage for HCP-administered therapies that have been approved by FDA as safe and effective for specified uses, as well as for additional uses that are supported by compendia and peer-reviewed literature. Within the Medicare Part D program, it remains important to retain protected classes for anti-cancer drugs where substantially all need to be covered on formulary. These types of policies that ensure patients have access to a broad range of proven therapies are also critical within the commercial market, where it remains important to avoid reimbursement policies or utilization techniques that could limit therapy choices for patients. A framework wherein narrow formularies are designed, for example those covering one product

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A Complicated Coverage Challenge

A 59 year-old female patient with rectal cancer tried and failed on all standard treatment options, and her insurance company denied pre-certification for the treatment on the grounds that it was off label. Working with the oncologist’s office, a Patient Advocate Foundation case manager appealed the coverage denial, but the appeal was denied. The case manager re-filed a letter of appeal, citing the Foundation One test results and the oncologist’s recommendation. The second appeal also was denied.

While the second appeal was being processed, the case manager initiated a compassionate use request to the manufacturer pending the outcome of the second appeal. The compassionate use request was granted, and the patient was able to access the medication with a cost savings of $120,000.
per drug class, is an example of a policy that is severely limiting for cancer patients if coverage exceptions are not allowed. To help ensure that patients have coverage for cancer treatment that is evidence-based, using consistent standards of evidence the National Business Group on Health also recommends “Plans to cover evidence-based cancer treatment, whether paid under the medical or pharmacy benefit.”

We also need to keep at the forefront of any payment reform discussions the need to ensure that patients are able to access treatments without being overly burdened by unreasonable out-of-pocket costs. Benefit designs that place more financial burden on patients, for example benefit designs with high deductibles and co-insurance, can have a disproportionate impact on patients with the highest disease burden such as cancer patients. Benefit design needs to be done in a way that keeps patients in mind, and avoids co-payments or coinsurance that will preclude a patient from receiving needed care. Limitations on cost-sharing, such as the elimination of specialty tiers within the commercial and Medicare space, would improve access to innovative, life-saving care, and lessen patients’ financial burden as they move through treatment.

**Recommendations for Access to Appropriate Treatment Options for Individual Patients**

Access to innovative treatments must be improved if patients are to fully benefit from these breakthroughs. Cost-sharing should be equitable across different benefits and settings of care to ensure that patients have equal access to the most beneficial and appropriate treatments. For example, parity in reimbursement for orally administered chemotherapy as opposed to chemotherapy provided via intravenous (IV) administration continues to be an important issue for patients. Even as access to such treatments is inhibited, there remains no question that the use of oral anti-cancer medications, when medications can be administered in the home, minimizes travel issues and can lower costs to patients.

In “An Employer’s Guide to Cancer Treatment and Prevention,” the National Business Group on Health made a number of recommendations for pharmacy benefits to ensure that employees would receive optimal care and increase their chances to return to work and remain productive and healthy. The Guide calls for “Reasonable out-of-pocket thresholds to ensure that “cost is not a barrier for patients to obtain medications needed to treat their condition, including maintenance and supportive care drugs.” The Guide also recommends that “per-prescription copayment and/or coinsurance requirements should be established at a reasonable level,” that a single “out-of-pocket maximum that applies to combined medical and pharmacy expenditures,” and “parity of patient cost sharing between the medical and pharmacy benefit.”

To date, the most successful payment reform initiatives have moved away from traditional fee-for-service models, while also ensuring that patients are at the forefront of care, with providers held accountable if that is not the case. This increased accountability and transparency has the goal of improving quality of care and patient engagement, as well as improving outcomes and lowering costs. If payment reform is to move forward, and be applied to oncology patients, appropriate quality measures need to be established, and safeguards are required to ensure that bundling does not occur at the expense of quality treatment and access to innovative medicines for patients.

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These patient care issues must be addressed given the lack of evidence to date. A recent study by RAND found that “bundled payment programs that incorporate a quality component are ... new, and there is virtually no evidence on whether they can be successfully implemented and what their effects are.”71 This quality measure component is a cornerstone of any new payment and delivery reforms, to help ensure that quality patient care is not compromised by cost considerations. Quality measures attached to new payment and delivery reforms should provide a more complete picture of the care provided, including longer-term outcomes, rather than process-focused measures, and should consider the aggregate costs of care. Inadequate quality measures can create incentives that undermine the needs of individual patients who require specialized and individual care, ultimately reducing both quality and value.

How do we define “value” across the spectrum of innovation, in a manner that will be uniformly adopted by patients, payers, providers and investors? That is an overarching challenge that will benefit from multi-stakeholder input but needs to recognize survival improvement, quality of life, ability to continue working, as well as other benefits of medical innovation and therapies.

Further discussions should be held with government officials to discuss how to include patients in any conversation related to payment models72 so that any system that is developed will be both cost effective and deliver quality patient care.73 Adequate and stable reimbursement mechanisms that ensure patient access to innovative therapies remain critical. Many payment and delivery models are under consideration and being piloted. The development of quality measures and benchmark performance data should be prioritized as the foundation of any payment reform effort, and particularly if the payment reforms place increasing risk on providers. A technical expert panel convened to provide input on payment reforms in oncology found for example “that one of the linchpins of any bundled payment approach is robust quality measures to ensure the proper standard of care is being delivered to patients. Patient protection is an important factor in this model, in light of the potential for the perverse incentive of under-providing care. Members agreed that measures should be both robust and validated, though there was discussion on whether quality measures alone are a sufficient quality floor in this model, since they may not represent all the important dimensions of care.”74

Cancer payment and delivery models should be robustly studied and tested before broad implementation to ensure that patient access to care, and quality delivery of care, is not compromised. To date, the scope of bundling has not been determined effectively or efficiently within the field of oncology. More generally (and not specific to oncology), several studies have cited difficulties with bundling related to chronic disease and the paucity of evidence regarding the impact of bundled payment programs and whether they can be successfully implemented.75 It is also important to include patients in any conversation related to payment models so that


75 See Damberg.
any system that is developed will be both cost effective and beneficial to the patient. The Consumer-Based Cancer Care Value Index (CCCVI) project seeks to answer the question: how do patients measure value in cancer care? An emphasis needs to be placed on creating new payment models that “reward cancer care teams for providing patient-centered, high-quality care and eliminating wasteful interventions.”

**Recommendations to Preserve Access to Patient-Centered Care**

Chronic diseases, such as neurodegenerative diseases, cancer, and diabetes, account for more than 75 percent of overall health care spending in the United States. A study that evaluated seven of the most common chronic diseases estimated that these conditions cost the nation over $1.5 trillion annually, seven-fourths of this burden was related to economic loss as a result of lowered productivity.

Patient decision aids for some health conditions, for which treatment decisions are highly sensitive to both patients’ and physicians’ preferences, may reduce rates of elective surgery and lower costs.77 Informed consent “refers to the process and documents associated with educating individuals on the details of a clinical trial and potentially gaining their consent to participate in clinical research. Obtaining informed consent from each subject in a clinical trial requires a significant amount of time. The informed consent process includes developing appropriately worded consent documents, discussing the documents and the clinical trial process with individual patients, obtaining the required patient signatures on the documents, and keeping track of the paperwork generated throughout the enrollment process.”78 The informed consent process should be streamlined to be more understandable and patient friendly. While the consent forms contain necessary disclaimers and legal information and must continue to do so, the length and complexity of informed consent forms are difficult for patients, and should be presented in a more consumer friendly manner.

Some stakeholders suggest that involving patients in their health care decisions decreases conflicts, increases patient satisfaction, and improves their knowledge and short-term adherence to care protocols. Patient-reported outcomes should be further discussed. The environment exists but the data collection methods must be improved in order to make this a useful endeavor that will benefit patients. As more patients participate in the treatment decision-making process, more potential data becomes available.

**Recommendations for Facilitating Physician, Patient and Stakeholder Communication to Sustain Quality Patient Care**

Physician education is important, in furtherance of informing and empowering the patient. Patients should be given all relevant information and potential courses of treatment when they are seeking care. This includes access to and information on available clinical trials. Patients have a vested interest in researching all available options and becoming informed. However, patients’ treatment regimens should not be dictated by their level of knowledge; the physician-patient relationship should be a partnership, and providers’ knowledge should prevail. A recent survey of cancer patients indicated that “85 percent were unaware that participation in clinical trials

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78 Institute of Medicine, supra.
was even an option. Of the patients surveyed, 75 percent said that if participation in a clinical trial had been offered, they would have been receptive to the idea. Of those aware of clinical trials and offered the possibility of participation, 71 percent chose not to participate. However, almost all who participated were satisfied with the experience. Thus, according to these survey results, patients’ preconceived notions about trial participation could pose a barrier to clinical trial enrollment.\(^79\)

The Institute of Medicine has recently advocated for a “learning health care system”\(^80\) that would allow for continuous updating of best evidence and clinical practices. The system would allow for collection and sharing of large data and would facilitate translation of research into treatments that can be used in the clinical setting. This improved collection and sharing would have the added benefit of empowering patients by making it easier to understand and make decisions about their treatment options. The IOM further recommends the collaborative development of a digital infrastructure to house this data and facilitate its access and ease of use for all stakeholders.\(^81\)

**Historic Successes of Innovation: Recommendations for Coordination among Stakeholders**

Success with diseases such as HIV, polio and childhood leukemia and other life-threatening diseases show that collaborative efforts to eradicate disease can succeed if all stakeholders work together. Through government involvement and funding; grassroots lobbying and organization; and coordinated, aggressive research and education efforts; the fights against these diseases have seen successes that could – and should – be duplicated in the fight against cancer.

**HIV/AIDS**

Development and approval of new antiretroviral drugs has led to improved successes in managing HIV and brought society closer to a cure.\(^82\) This was made possible only because a group of passionate, educated stakeholders from the advocacy community, provider community, elected and unelected government officials, media and many others, took the time to understand the illness and what needed to be done, and utilized that knowledge to successfully lobby the federal government to work toward, and fund, the elimination of this disease.

In the 1980s, the issue resulted in hearings held in the United States Congress, in response to demand from the public to pursue a solution to this increasingly pervasive epidemic. This increase in awareness resulted in increased funding and research in pursuit of a cure. In 1985, HHS and the World Health Organization (WHO) hosted the First International AIDS Conference in Atlanta, and soon after, the National Institutes of Mental

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\(^81\) See Id. at 258.

Health AIDS Center grant designed to boost AIDS prevention research. In 1990, Congress passed the Ryan White Care Act, creating programs that have become the largest provider of services for people living with HIV/AIDS in the United States.

Since this aggressive government lobbying was conducted by citizen stakeholders in the 1980s and 1990s, government funding for research and development of HIV/AIDS vaccinations, medications and treatments has remained relatively secure, buoyed by private sector and non-profit stakeholders who see the goal of curing HIV/AIDS as one that is both necessary and attainable. The grassroots movement also convinced the FDA to alter its clinical development requirements to truncate the process so that approval of life-saving innovations could be expedited.

Polio

Similarly, the eradication of polio was the result of an aggressive mobilization of people who demanded change from their government leadership and private research communities. In the late 1940s to the early 1950s, polio crippled an average of more than 35,000 people in the United States each year. Thanks to the development of an effective vaccine, the United States has been polio-free since 1979, and the virus has been effectively eradicated from the world. The National Foundation for Infantile Paralysis (NFIP), now known as the March of Dimes, was a leader in the efforts to eradicate polio. Public awareness, coordination and education were made easier because of its founder, Franklin D. Roosevelt, whose inability to walk on his own after contracting polio raised public awareness and translated into a widespread effort to learn more about the disease and make efforts to find a cure. The NFIP led the charge to eradicate polio in the United States along with countless volunteers, educators and medical researchers supported by March of Dimes grants.

A breakthrough occurred at the University of Pittsburgh, when Jonas Salk, MD, created a vaccine that spelled the end of polio in a matter of years. “Tested in a massive field trial in 1954 that involved 1.8 million schoolchildren known as ‘polio pioneers,’ the Salk vaccine was licensed for use on April 12, 1955, the very day it was announced to the news media as safe, effective, and potent,” according to a history of polio. Within a few years, reported cases of polio in the United States declined from tens of thousands per year to almost none. This success would not have been possible without the coordinated efforts of millions of volunteers throughout the nation. Public outreach and education created an environment that resulted in the polio epidemic moving to the forefront of the nation’s consciousness, such that government funding and private donations were available, and volunteer science and medical professionals were ready and willing to donate their time to this effort. It was united energy and advocacy around a common goal, and coordination among stakeholders, that led to this success.

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86 See Id.
**Childhood Leukemia**

Initial research on childhood leukemia led to breakthroughs that aligned stakeholders, got groups engaged and working in concert, and led to trials and advances that have saved countless lives since the 1950s. In the mid-1950s, three children’s cooperative groups—Acute Leukemia Group A (eventually Children’s Cancer Group [CCG]), Acute Leukemia Group B (which became Cancer and Leukemia Group B [CALGB]), and the Southwest Cancer Chemotherapy Study Group (which evolved into the Southwest Oncology Group [SWOG])—formed in rapid succession. Internists also soon joined and began to enter patients into clinical trials. Multi-institutional collaborations were developed to ensure that researchers were collaborating and coordinating their research. As noted by LLS, the first chemotherapy drugs were developed for lymphoma and leukemia patients, including children. A group of prestigious researchers took the lead on coordinated research, and two of these doctors, Drs. Hitchings and Elion, received Nobel prizes for their work, and helped guide LLS research funding in the early years.

Recognizing the promise of the research, substantial funding was put forth by the U.S. National Cancer Institute. Expanded use of clinical trials has helped doctors refine their use and dosage of drugs, resulting in greater survival rates. Research found that selecting therapy based on patient- and disease-specific prognostic factors resulted in significant improvement in outcomes for childhood leukemia and, in an example of a progressive innovation leading to a potential breakthrough, this novel approach has been adopted for adults in recent years, and is showing favorable results to date. According to NIH, recent progress in risk-adapted treatment for childhood acute lymphoblastic leukemia has secured five-year event-free survival rates of approximately 80 percent and five-year survival rates approaching 90 percent.

**An Analysis of Clinical Trials: The Crux of Cancer Innovation**

The National Institutes of Health, academia and independent researchers conduct basic research that leads to discoveries of the biology of diseases like cancer and understanding of the ways in which disease pathways are activated or blocked. With this information other researchers, primarily biopharmaceutical research companies, apply that basic scientific research to the task of discovering, designing and developing novel chemical compounds and biologics that can operate through those disease pathways. Evidence of the safety and efficacy of such innovation generated through clinical trials is crucial to the advance of medicine. Clinical trials are

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92 See Id.
93 See Seibel supra.
integral to each of the three pillars of innovation discussed in this paper: streamlining logistical obstacles, bolstering funding and improving access to innovation through enhanced physician-patient communications.

Streamlining the many logistical obstacles to the conduct of clinical trials will go far to expedite the development of innovative cancer therapies. Twenty-four out of twenty-five drugs that enter clinical development will ultimately fail to reach the market, and clinical trials represent the largest cost associated with drug development for pharmaceutical companies worldwide. Over 40 percent of the total research and development dollars are spent on clinical trials, which results in a global expenditure of approximately $30 billion per year.

There are some regulatory requirements that may make it more difficult for oncologists to focus on delivering treatment to patients. A recent article published in the Journal of Clinical Oncology states: “Trials of new drugs, especially in oncology, have become a long row to hoe. For the last 25 years, well meaning bureaucratic functionaries have introduced countless new regulations without field testing or consultation with clinical investigators. The resulting proliferating complexity and unnecessary formalities involved in developing and testing cancer therapies have stifled innovation, driven up costs, and delayed development of new treatments—factors that may ultimately harm patients.”

**Challenges to Access: Moving Clinical Trials Overseas**

Increasingly, biopharmaceutical companies are conducting clinical trials outside the United States “as industry and government sponsors in wealthy countries move trials to less wealthy countries.” Sponsors save substantial development costs by conducting trials overseas so are increasingly moving phase 1 and phase 2 trials to other countries, and there are concerns about the applicability of these results to patients in the United States, given the different demographics and other variables involved.

Although there is universal agreement that safe and effective medications should be moved to market as quickly as possible, it is generally considered more difficult to conduct a clinical trial in the United States than overseas, as evidenced by the fact that 70 percent of current clinical trial enrollment is non-domestic. While well-designed randomized clinical trials are the most reliable way to get unbiased information to support development of new therapies, our current system of conducting clinical trials is often paper-based, slow, and costly. Poor quality and inefficiency in clinical research can seriously limit the number of questions that we can answer about the appropriate uses of approved or licensed medical products and significantly delay access to new therapeutic innovations.

As clinical trials are increasingly being conducted outside of the United States, the Food and Drug Administration (FDA) has concerns, rightfully so, regarding the safety and administration of foreign clinical trials in areas where the agency has no oversight or inspection capability, and where trials are completed and data compiled prior to

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95 Meadows, supra at 8.
97 Glickman et. al., supra at 816.
FDA engagement. It has been observed that, since 2002, “the number of active FDA-regulated investigations based outside the United States has grown by 15 percent annually, whereas the number of U.S.-based investigators has declined by 5.5 percent.” Governmental concerns regarding safety of clinical trials conducted in a non-domestic setting should provide additional incentive to work with stakeholders to ensure that the domestic clinical trial process is user-friendly, for patients, developers, physicians and payers alike.

The Institute of Medicine points out that the trend toward conducting clinical trials outside the United States is “an important consideration in discussing ways to improve the efficiency of trials. The number of patients enrolled in clinical trials is decreasing in the United States and increasing abroad, [and] when development programs are conducted entirely outside the United States, the FDA questions the extent to which the results can be translated to U.S. clinical practice.” It remains possible that the clinical trial model used in Europe may more appropriately combine interests in safety and efficacy with interest in expedited trial processes, so it is worth looking at as a potential model for reforms in the United States.

Restoring the number of American physicians who participate in clinical trials also would help improve knowledge about innovative therapies within the medical community. Over the past decade, the number of physicians participating in clinical research has continued to decrease in the U.S. and Europe, while participation increased at double-digit rates in Asia, Latin America and Central/Eastern Europe.

Enrollment Hurdles: Criteria, Physician Referrals and Geography

A large hurdle related to the design of clinical trials is that they tend to be inherently biased against “older” patients, based on underlying co-morbidities. Exclusion of older patients from clinical research, under-recruitment of elderly patients to clinical trials, is widespread. This problem has stark consequences for patients; according to an expert committee of the European Medicines Agency (EMA), the result is inadequate evaluation of medications indicated for use in elderly patients. The problem will continue to worsen as the population as a whole continues to age. Projections show that by 2030, nearly one in five U.S. residents will be age 65 and older, and by 2050, the older adult population is expected to reach 88.5 million, more than double that in 2010. The overall rate of cancer incidence will rise from 1.6 million in 2010 to 2.3 million in 2030, and the overall survivor rate will continue to rise as well. These figures highlight the promise of innovation, and also the importance of facilitating access to affordable treatments.


See Institute of Medicine, supra at 816.


See Id.
It is important to develop reimbursement policies that encourage, and incentivize, the enrollment of patients in clinical trials. According to a recent Institute of Medicine report,\textsuperscript{106} practitioners face a number of challenges to their involvement in clinical research. Busy patient practices and the associated billing and reporting requirements leave them with limited time for research. A further barrier is the lack of a supportive clinical research infrastructure, especially in the form of administrative and financial support. For practitioners who become engaged in running a clinical trial and recruiting patients, their financial reimbursement per patient can, in some cases, be less than they would receive from regular practice. In addition, there is a financial disincentive for physicians to refer their patients to clinical trials. Physicians who do so must often refer those patients away from their care; thus, each patient referred represents a lost revenue stream.\textsuperscript{106} In order to encourage physician participation in clinical trials, the procedures required as part of a trial protocol should be easily incorporated into physician practices.\textsuperscript{107}

The location of clinical trials remains a hurdle as well, in terms of the limitations it places on patients, particularly those in rural settings, who wish to participate. The Institute of Medicine has found that “sites for clinical trials are frequently selected on the basis of where the investigators are located, as opposed to where the patients are, creating difficulties in patient recruitment. When patient recruitment is impeded, the trial is delayed, sometimes by years, until the number of patients required by the study protocol can be enrolled. Once a trial protocol has been activated, the recruitment of patients requires a significant amount of time and money.”\textsuperscript{108} The financial burdens faced by patients can be even greater. Many lower-income patients cannot afford the cost of travel to and from a clinical trial site, meaning they are unable to participate, even when the trial could be potentially lifesaving.

\textbf{Improving the Clinical Trial Approval Process}

Clinical trials are necessary in the development process, but measures can be taken to ensure that they are completed at a rate that allows breakthrough treatments to become available to patients as quickly as possible. The reliability of data from clinical trials requires sufficient size, but regulators and sponsors should also be mindful that smaller populations and faster approval processes will benefit patients if these products are proven effective and brought to market. Delays imposed upon a safe and effective breakthrough treatment results in countless patients who are unable to benefit from what could have been a life-saving innovation, and the importance of these breakthroughs to those who need them most needs to be considered as we determine how best to structure the clinical trial and approval processes.

In an effort to expedite the approval process and move safe and effective cancer treatments to market, the use of smaller, more targeted clinical trials should be encouraged in some instances, because the trials are often “smarter,” meaning they may be better able to treat a specific subset of patients. One potential solution to the

\textsuperscript{106} Institute of Medicine, \textit{Supra.}
\textsuperscript{107} See \textit{Id.}
\textsuperscript{108} \textit{Id.}
problem of slow approvals may be increased usage of adaptive protocols,\(^\text{109}\) sometimes referred to as adaptable statistics, which provide an opportunity to expedite the clinical trial process, since sponsors are able to gain an early lead on results. By using smaller, more targeted trial populations, a sponsor can more easily determine if a particular trial is not working on a particular type of tumor or disease, and is able to modify or end the trial, allowing their time and resources to be directed toward studies that are showing promising results.

Testing for tumor biomarkers or molecular mutation contributes to this expedited process, but issues remain with respect to how such tests are administered in the clinical setting. For example, can trial centers be easily equipped to administer such a test if they are not already equipped to do so? Who will pay for these upgrades, and also for the genetic testing - is it the responsibility of the insurer, the patient, or the sponsor of the trial? If the test is integral to the trial (i.e., targeted therapy being evaluated and genetic test is needed to find candidates) does it or does it not raise a different policy question than the question of who is responsible for other routine health care costs in connection with a trial? If it does not, then perhaps the answer should be consistent with coverage of other related care. If there exists agreement among stakeholders that a truncated trial process would expedite the development of and access to lifesaving medications and therapies, we must work together to answer these questions.

Novartis’ Simplified Institutional Review Board (IRB) Process (Signaturetrial.com) is an example of one possible solution. Patients who test positive for a specific type of mutation may enter the trial and receive medication regardless of their tumor type. The program conducts smaller trials to test products against tumor type, regardless of site of tumor, in order to quickly determine which course of treatment is most effective for patients. Using a 1-800 telephone number, patients are enrolled quickly and easily, and the rapid trial execution model should be able to produce results within one year.

**Increasing Patient Access to Clinical Trials**

One method through which patients can access innovative treatments is through enrollment in clinical trials.\(^\text{110}\) Patients with life-threatening diseases need expanded access to clinical trials in order to provide them with a broad scope of treatment options, particularly when standard of care treatment has been ineffective against a particular diagnosis. Many patients are seeking access to new medications and treatments as quickly as

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possible, but also wish to be assured that the benefits of these treatments will outweigh the risk.

How do we balance patient access and public demand for affordable medications with the need for sponsors to recoup their initial investments for future research and development? Mitigating risk and improving the speed of development is important, as is discussing who actually develops the drugs. Sponsors are expected to bear these costs, not NIH, and this should be a part of the discussion, and the national consciousness, when making policy decisions related to cost and reimbursement structures. With respect to payers’ willingness to reimburse for non-standard of care treatments such as clinical trials and products resulting from progressive innovation, utilization review techniques such as step therapy and fail-first restrictions inhibit patients’ ability to access needed medications and create barriers to innovation. In short, efforts to develop a more favorable climate through which financial investments can be made, supplemented by a more certain reimbursement structure, would provide additional economic certainty to encourage new and greater investment, and lead to growth in the area of cancer innovation.

**Increased Funding for Clinical Trials**

Congressional appropriations to the National Cancer Institute and other government agencies for basic scientific research in the biological pathways of cancer and for the conduct of clinical trials toward proof of concept can greatly expedite innovation in the treatment of cancer. Payers also arguably have some responsibility with respect to the funding of services provided as part of clinical trials. Sponsors have a stake in, and can benefit from, payer funding of trials. To the extent that trials result in approvals that lower out-year health care costs and consequently provide savings in the long term, there should be a vested interest within the payer community to cover health care services provided within the context of clinical research.

The National Institutes of Health (NIH) note that “the United States lacks a clear prioritization of the gaps in medical evidence and an allocation of clinical research resources to efficiently and effectively fill these evidence gaps.” The federal government, industry, academic institutions, patient advocacy organizations, voluntary health organizations, and health insurance issuers each have incentives to develop research questions that suit their unique interests. NIH further acknowledges that a substantial amount of time, energy, and money are spent on bringing resources together for each trial, and inconsistencies in trial execution across sites is not an abnormal occurrence.

**The Role of Electronic Health Records in Simplifying Trial Costs and Marketing**

Electronic Health Records (EHRs) can play a role in analyzing and evaluating patient data instantly to determine eligibility for a clinical trial without ever compromising an individual’s privacy. By examining clinical indicators for potential participation in research, providers will be able to easily identify, as well as provide information on,


113 See Id.
relevant trials that may be beneficial to an individual’s care. EHRs can enable clinical decision support functionality when a patient exhibits certain diagnostic factors that match pre-trial eligibility requirements for relevant clinical trial opportunities. Patients and doctors could then decide whether participation in a trial makes sense for them.

Determining eligibility for clinical trials as an essential function of EHRs will increase awareness of clinical trial opportunities for all Americans; expand access to patient populations with historically low participation in clinical trials such as racial and ethnic minorities, women, and the elderly; automate pretrial screening and the identification of potential trial participants; and reduce the cost of the drug development process.

CMS can leverage existing government infrastructure for clinical trials such as ClinicalTrials.gov in combination with the Meaningful Use Program using internationally accepted data standards. By requiring that clinical trial opportunities posted on ClinicalTrials.gov include pretrial screening information using standardized technical vocabularies, EHR systems will be able to compare relevant trial requirements to a patient’s clinical and claims data without exposing the patient’s private information. As a function of the EHR, clinical decision support rules would be developed to indicate when a patient exhibiting specific conditions might benefit from participation in a clinical trial. The decision support would identify clinical trials based on diagnosis or procedure code specific to the patient and could be tailored geographically by zip code. The physician and patient would then decide whether or not to seek participation in the trial. Currently, MU program rules require CDS for at least one rule, but not for clinical trial matching.

This process will stimulate the development of cures by identifying good candidate matches for clinical trials through existing health IT infrastructure. Because 85 percent of hospitals and more than 60 percent of physicians are already registered for the Meaningful Use program, adoption of this change would dramatically expand provider and patient awareness of clinical trials while lowering costs of clinical trials by better recruiting patients who are more likely to remain in a trial effort.

**Empowering Patients to Seek Innovative Treatment Opportunities**

Lack of information for patients, particularly with respect to information on available clinical trials, places limitations on patient engagement and empowerment. Many patients are simply “unaware of the possibility of enrolling in a clinical trial. If they are aware of this opportunity, it is often difficult for them to locate a trial. Patients may reside far from study centers; even the largest multicenter trials can pose geographic challenges for those wishing to

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**Patient Story: Clinical Trials and Innovative Treatments Save Lives**

An example of successful treatment of a cancer patient through expedited enrollment in a clinical study involved a 35-year old single mother from Virginia who was diagnosed with adrenocortical carcinoma. Local doctors informed her that there were no medical measures available to stop her disease. Patient Advocate Foundation became involved in the case and negotiated her enrollment into a clinical trial that had just opened at the National Institutes of Health. She began treatment the next day. Novel therapeutic intervention was initiated one month later, and within four months the patient’s tumors had shrunk, and it was decided she would not require stem cell transplantation. Nine years later, she is cancer free.
participate,” the IOM has noted. “Moreover, depending on the number of clinic visits required by the study protocol, significant travel and time costs may be associated with participation.

Patients generally do not become aware of certain treatments and advances unless their treating physician happens to provide them with this information, and do not realize that they may be subject to substantial bills for certain costs incurred as part of the trial. An oncologist who is unaware of a particular trial that would be beneficial for the patient is unlikely to offer it as a potential course of treatment, and certainly will not have the information needed to discuss cost. More must be done to educate both patients and providers. Patients’ access to a clinical trial should not be contingent on their ability to learn about it on their own, or their capacity to afford it.

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Conclusion

Developing a cure for cancer is a daunting task that can be achieved only through substantive and cooperative investment from all health care stakeholders, including the biopharmaceutical industry, academia, health care payers, providers, nonprofit patient organizations and the public sector to the process of innovation.

As an example of such progress, the treatment of lung cancer has seen significant advancement due to innovative developments in detection and treatment options. Recent advancements have included targeted therapies through personalized medicine, therapeutic vaccines, new chemotherapies, maintenance therapies, chemoprevention, and increased efforts at early detection. These developments have helped reduce the prevalence of lung cancer in men and women, as well as mortality among men and women.

Today, thanks to biomedical innovation, most patients diagnosed with CML during the chronic period can look forward to a life expectancy similar to a non-CML patient. However, it took about 30 years of basic scientific research and progressive treatments to reach this point. The first breakthrough in CML research came with the discovery of the Philadelphia chromosome in that city in 1960. Still, until the 1980s patients with a CML diagnosis had no cause to be optimistic: the median survival rate from diagnosis was about five years. Through the next two decades, advances in interferon treatment and stem cell research led to better outcomes for patients, and clinical trials in the early 1990s revolutionized CML therapy. Development of tyrosine kinase inhibitors followed. Today, the 7- and 10-year survival rates for CML are now over 80 percent.

Between 1988 and 2000, life expectancy for cancer patients increased by approximately four years, meaning the gains in cancer survival during this period created 23 million additional life-years and roughly $1.9 trillion of additional social value. New cancer drugs introduced over the past 30 years have increased the life expectancy of cancer patients by almost one year, and since some products introduced in more recent years have shown better tolerability than traditional chemotherapy, the quality-adjusted life benefit could be even greater.

To maintain these trends, innovation must be protected and incentivized. Only by streamlining scientific research and regulatory barriers, enhancing research funding and increasing collaboration among all stakeholders can we expedite innovation of new cancer treatments, as the childhood leukemia, CML, HIV and polio stories clearly illustrate. Recognizing the value of progressive advances as well as breakthrough technologies will propel medical science forward and ultimately lead to a cure for cancer.

The Cancer Innovation Coalition invites you to join us in this call to action to promote and expedite the research and discovery process through innovation for the benefit of humankind and public health now and in the future. The CIC recommends that to alleviate these obstacles federal and state entities invest in more research, perhaps in tandem or in collaboration with pharmaceutical manufacturers and other public and private entities while developing incentives for innovative research. The CIC further recommends the federal government develop incentives to urge collaborative efforts among pharmaceutical companies and other researchers, and to ensure patient access to clinical trials. While the CIC commends the FDA for recent advances in approvals, continued work on expedited regulatory pathways remains imperative. This necessity must be funded.

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