



## Six Perspectives on the Cancer “Moonshot” Goal of Making Ten Years of Progress in Five Years: What does that mean and how can we get there?

President Obama has issued a challenge to galvanize the cancer research and patient communities in the effort against cancer through the “Moonshot” initiative announced in his 2016 State of the Union Address.

Much success in our understanding and ability to combat cancer has been achieved through a sustained and coordinated collaboration of basic, epidemiologic, clinical, behavioral and translational research. This commitment to science has revolutionized our ability to prevent and detect many cancers, and to develop personalized forms of cancer treatment and predict their response with enhanced precision. Yet, as we all know, the fight is far from over as too many people today still suffer and die from cancer in all its forms.

The cancer “Moonshot” initiative is bringing together scientists, oncologists, patient advocates, and representatives of the biopharmaceutical industry with renewed collaborative focus and the ambitious objective of consolidating ten years of cancer research in five years. Achieving this outcome will require unprecedented cooperation by all stakeholders to prioritize and focus on the highest impact emerging and unforeseen scientific pathways of cancer research.

On April 25, 2016, the National Coalition for Cancer Research (NCCR) brought together an esteemed panel of experts to discuss this groundbreaking Initiative and provide key insights as to how we can accomplish the goal of making ten years of progress in five years. Many central themes emerged from the discussion as panelists offered their perspectives on priority areas of focus that are rich for acceleration.

*Richard L. Schilsky, M.D., FACP, FASCO, Chief Medical Officer, American Society of Clinical Oncology*

“Now is a special time in the history of cancer research and cancer care,” remarked **Dr. Richard L. Schilsky, Chief Medical Officer of the American Society of Clinical Oncology**, who moderated the session. Dr. Schilsky’s optimism about the significant progress that has been made in the fight against cancer - as a result of sustained investments in research - was evident. “The typical patient diagnosed with cancer today has better than a two-thirds chance of being alive five years from now and many will be cured,” he stated. Despite recent advances in the field, he emphasized that several challenges still remain. “Basic science research has provided enormous insight into the fundamental causes of cancer but has also revealed the tremendous heterogeneity of cancer and its incredible ability to adapt and develop resistance to treatment.” Dr. Schilsky highlighted the need for better biomarkers to identify which individuals are at highest risk of recurrence and of developing cancer. He cited that scientists now recognize hundreds of forms of cancer. More research is needed to better understand the underlying mechanisms of cancer and this research will continue to be essential as researchers identify more cancer types in the future.

Dr. Schilsky also noted that we simply don’t have enough time or money to learn everything that still needs to be learned about cancer prevention and treatment through traditional clinical trials. He highlighted the need to incorporate an untapped source of information – Electronic Medical Records. He believes that we have the ability to accelerate progress against cancer by sharing data from these records.

In order to share data, however, he stressed that “we must develop a common standard vocabulary so that we can all be describing the clinical characteristics to patients, the genomic features of their tumors, and their clinical outcomes in the same way.” This will be critical so that when information is shared “we are sharing similar pieces of data and don’t end up drawing erroneous conclusions.” He strongly underscored that one of the priorities of the “Moonshot” must be a national medical vocabulary that is “widely adopted, standardized and widely used” by the scientific and medical community.

*Linnea Olson – Lung Cancer Survivor, Participant in numerous clinical trials, Artist, Boston, Massachusetts*

As a patient who has been surviving lung cancer and participated in numerous clinical trials since April 2005, **Ms. Linnea Olson** offered a unique perspective on the clinical trials experience. “Clinical trials have become my lifeline, my next best hope,” she remarked. “When I chose to enroll in my first clinical trial, I knew three things: 1. Experimental therapy might or might not extend my life and could hasten my death. 2. The only other participant at my institution had died almost immediately in large part by side effects brought on by experimental therapy. 3. If I did not enroll, my cancer would surely kill me.” She confessed she was terrified when she entered her first clinical trial, but she trusted her oncologist and felt the trial offered a “sliver of hope”.

Ms. Olson pointed out that despite the efforts of many in the oncology community, only three to five percent of adult patients choose to participate in clinical trials. This is in large part due to the many barriers associated with participating in trials. Ms. Olson admitted that she was incredibly fortunate that the opportunity for a clinical trial was presented to her and that her oncologist was willing to explain the trial in a way that she could easily comprehend. “I didn’t have to find it myself as many patients do, and the trial was offered at my home institution so the cost and hassle of travelling a great distance was not an additional challenge.” She emphasized that if we want patients to participate in clinical trials, we need to remove some of the barriers and keep in mind why clinical trials exist in the first place. “They are not to advance medical research but rather to treat disease, prolong life, and ease suffering.”

Regarding the evolving emphasis on “big data”, Ms. Olson stated that she is excited about data sharing but cautious about anything that has “big in front of it.” Her one piece of advice for the “Moonshot” initiative is to embrace the concept of “patients as partners.” She cannot emphasize the importance of the patient voice enough. “Although my data speaks loudly, I am the expert on my own experience,” she said. Finally, she expressed the utmost importance of collaboration in advancing the cancer field. “For patients, collaboration is everything, we don’t have room for competition in our world.”

*Elizabeth Blackburn, Ph.D., Nobel Laureate, President, The Salk Institute, La Jolla, California*

**Dr. Elizabeth Blackburn, President of the Salk Institute**, acknowledged that there are limitations to the scientific knowledge that has been gained in recent years and “we must continue to invest in and understand what we still don’t know.” She cited the example of immunotherapy, a new treatment approach that uses the body’s immune system to fight cancer. Dr. Blackburn stressed that the concept of unleashing the immune system came from the basic science of understanding the immune system itself. She cautioned that while there has been huge success in this arena, immunotherapy doesn’t work all the time for all patients.

In her remarks, Dr. Blackburn highlighted three main areas of focus for the cancer “Moonshot”. First, she believes it is vital to invest in research to better understand all of the ways that we can harness the immune system. “There are still many unknowns and we can’t just be content with the approaches that are working already.” Second, in the era of “big data”, she would like to see “big data being smart data”. She thinks that data is going to be maximally made smart by sharing it from patients to clinicians, between patients, and from clinicians to scientists. “Cancers are not standing still - they are changing all the time. This sharing must be done in a dynamic way,” she stated. Finally, she advised that it is crucial to focus on preventing cancers. “We want to be able to intercept cancers before they even start.” She emphasized that at this time, too many cancers start and develop. “If we could have fewer cancers and intercept them earlier, that would make a huge difference.” Dr. Blackburn acknowledged that cancers

don't happen overnight and that they are processes that develop over long periods. She encouraged scientists to think of their favorite therapeutic mode and investigate if this approach is something that might be relevant earlier and earlier in the course of cancer.

*Michael B. Atkins, M.D., Deputy Director, Georgetown-Lombardi Comprehensive Cancer Center, Washington, D.C.*

**Dr. Michael Atkins, Deputy Director of the Georgetown-Lombardi Comprehensive Cancer Center**, focused much of his remarks on immunotherapy, an approach that he believes is “critical to making the cancer “Moonshot” a reality.” He cited the recent progress this treatment has had in melanoma. “As recently as five years ago melanoma patients had a bleak prognosis. Half of patients would not survive longer than six to nine months. Today, with latest immunotherapy, over half of such patients can expect to be cured with less than a year of outpatient treatment and can live out the rest of their lives free of cancer.” A particular class of immunotherapies, known as anti-PD1 inhibitors, have shown responses in 20 different cancers and have already received FDA approval for melanoma, lung and kidney cancers. “No single treatment target in the history of oncology has produced such broad spectrum activity,” he declared. Dr. Atkins noted that all ten anti-PD1 pathway-based Phase 3 clinical trials reported to date have shown significant survival advantages. “These antibodies are producing lasting benefits without significant toxicity making them ideal agents to be used in earlier stages of disease, in patients with comorbidities and as backbones for combination treatment.”

Dr. Atkins shared several areas of focus that he and others in the medical community have identified as critical to accelerating the “Moonshot” initiative. He touched on the importance of supporting basic science to better understand how cancers evade the immune system and the need to develop translational clinical trials to validate these concepts. He also highlighted the need to incentivize community oncologists to participate and refer patients to clinical trials and educate, train, and re-train scientists and physicians in immune oncology principles so that they can better contribute to the field.

In addition to these areas, Dr. Atkins believes that better understanding how to use immunotherapies in the adjuvant setting, which are given after cancer has been treated by surgery with the goal of preventing cancer from coming back, will have the most impact. He noted that it is critical to create pharmaceutical, government, and academic partnerships to launch these types of trials in the adjuvant setting and to design such trials in a way that would get information quickly and allow as many patients as possible to gain access to the newest therapeutic approaches “The goal is no longer to turn cancer into a chronic disease, but to make it curable disease.” He acknowledged that with immunotherapy this vision is fast becoming a reality for many patients with many cancer types. “As we develop cures for cancer, it is important to know that these advances will be the foundation for future progress. Immunotherapy has the potential to cut deaths from cancer in half,” he concluded.

*Otis W. Brawley, M.D., FACP, Chief Medical Officer, American Cancer Society, Atlanta, Georgia*

**Dr. Otis W. Brawley, Chief Medical Officer of the American Cancer Society** emphasized the need to support basic research, clinical research, and clinical trials. He also echoed the real progress that has been made in the fight against cancer, noting that since 1991, cancer mortality rates have decreased by 25 percent. Dr. Brawley attributes much of this progress to efforts aimed at prevention, smoking cessation, improvements in treatment, and wise screening. He noted that the definition of cancer of is evolving – it is now one that involves “biopsies, genomics, proteomics, and things that were not even imaginable fifty years ago.” He is thrilled about these advancements but admits it has been very heterogeneous within the United States. For example, there has been a 40 percent decline in colon cancer death rates as a whole, but there are ten states that have had less than a ten percent decline. “One of the problems is that the technologies and knowledge that we currently have are not being adequately disseminated throughout our country. Everybody is not getting equal treatment and everybody does not have equal outcomes,” he remarked. Dr. Brawley highlighted some of his own research that has shown that 20 percent of women with breast cancer get less than optimal care – a disparity that he has noticed usually results from lack of coordination and fragmentation of care. He noted that the tools we currently

have are not being maximized to save as many lives as possible. Thus, Dr. Brawley believes that one of the biggest priorities for the “Moonshot” should be better coordination of care.

*Sandra Horning, M.D., Chief Medical Officer and head of Global Product Development, Genentech, San Francisco, California*

**Dr. Sandra Horning, Chief Medical Officer and head of Global Product Development at Genentech**, emphasized that now is an exciting time in history where science and technology are converging in the fight against cancer. She warned, however, “this is not enough” and “policies are needed to help create an environment that is conducive to taking advantage of this.” Dr. Horning stressed the importance of a need for a commitment to data sharing and transparency. “We are now at a time where we have a huge amount of information available from different sources. It is important to bring new partners to the table – people who know how to aggregate and analyze these data.” She also highlighted the demand for clinical and regulatory science to keep up with the pace of basic science, specifically regarding endpoints. “There is a need for work on meaningful endpoints that lead to good decision-making in drug development, in clinical trials, and for registration and approval.” Dr. Horning underscored that traditional endpoints, such as progression-free survival used in clinical trials do not capture the value of recent treatment approaches such as immunotherapy. She believes there is an opportunity for the community to come together and think about this in a cooperative way. Additionally, Dr. Horning pointed out that as clinical science evolves, it will be more and more important to consider how to capture the experience of the patient in a real-world setting. She believes there are many opportunities in this space with advancements in technology and big data but noted, “It will require a commitment to quality, volume, and understanding of how to manage these data.”

Dr. Horning also highlighted the importance of community participation in clinical trials. She emphasized the need to involve community physicians because they are key to advancing their patients’ outcomes through clinical trials. “Patient access to clinical trials and to influencing clinical trials and the endpoints as full partners is very important.” She thinks an important area of focus is to “establish a framework for clinical trials for embedding biomarkers and adaptive designs that lead to more informative endpoints that allow us to reach decisions more quickly.” Dr. Horning strongly believes that collaboration is key to accelerating progress of the cancer “Moonshot”. “The word collaboration can’t be said enough. Collaboration is about understanding what each and every one of us can do and contribute to this effort. It is essential to effective partnership that each is respected and working toward a common goal,” she concluded.

*Throughout the panel discussion, it became apparent that with the right investments, there is a real opportunity to make ten years of progress in just five years. The key areas of focus and recommendations set forth by these experts provide guidance for how we can prioritize resources to make the biggest impact. The most fundamental theme that permeated the panelists’ remarks was collaboration. Bringing together all stakeholders in the scientific and medical community will be the key to success for the “Moonshot” initiative and ultimately in the fight against cancer.*