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Cancer moonshot countdown

Douglas Lowy and **Dinah Singer**
Division of Cancer Biology, National Cancer Institute, Bethesda, Maryland, USA.

Ron DePinho,
Department of Cancer Biology, University of Texas MD Anderson Cancer Center, Houston, Texas, USA.

Gregory C Simon, and
White House Cancer Task Force, Washington, DC.

Patrick Soon-Shiong
NantWorks LLC, Culver City, California, USA.

Nature Biotechnology asks representatives from three different cancer ‘moonshot’ initiatives to outline their visions.

Reaching for the moon is not likely to succeed in a single attempt. That is why the United States has witnessed the launch of several different ‘moonshot’ initiatives aiming to galvanize efforts in cancer prevention, diagnosis and treatment. In January, the US government announced the National Cancer Moonshot Initiative. According to the project’s leader, Vice President Joe Biden, whose son Beau’s death from brain cancer last year prompted him to champion the effort, it “is poised to be a critical part of our nation’s anticancer strategy.” The same month, the Cancer MoonShot 2020 Program was officially launched by biotech billionaire Patrick Soon-Shiong to bring together stakeholders from industry, academia, community oncologists and government to produce vaccine-based immunotherapies against cancer. These moonshots joined Texas cancer center MD Anderson’s ambitious Moon Shots Program, which was launched in 2012. *Nature Biotechnology* approached representatives from each of the different initiatives to understand the different aims and strategies being employed.

What is the specific aim of your moonshot in cancer?

Ron DePinho: The aim of the MD Anderson Moon Shots Program is to more rapidly and systematically convert existing and emerging scientific knowledge into true clinical advancements that will alleviate pain, suffering and death from cancer. Our program brings together large, multidisciplinary teams of researchers, clinicians, policy experts, educators and staff to mount comprehensive, goal-oriented programs focused on delivering marked improvements in prevention, early detection and treatment options in 12 major cancers: acute myeloid leukemia, myelodysplastic syndrome, chronic lymphocytic leukemia, melanoma, lung cancer, prostate cancer, triple-negative breast cancer, high-grade serous ovarian cancers, B-cell lymphoma, colorectal cancer, glioblas-toma, high-risk multiple myeloma, HPV [human papillomavirus] related cancers and pancreatic cancer.

These moon shots are supported by execution-oriented implementation platforms, which include seasoned drug development professionals from industry and cancer control experts with government and policy experience. Combining such expertise with that of our academic scholars is probably the most unique aspect of our initiative. Historically, academia's strength has been its discovery of new knowledge, but it has been less well structured to systematically convert such knowledge into practical endpoints such as new drugs or legislative policy. Effective translation is not only about discovery but also application of knowledge. Translational research is also an inconsistently funded research phase that exists between scientific discovery (largely funded by federal grants) and late-stage clinical trials (financed by the private sector) where ideas often die before they have a chance to be thoroughly tested. Improving the efficiency of preclinical research and early clinical trials and cultivating the exchange of knowledge back and forth between lab and clinic are essential to successfully traversing this valley of death.

Greg Simon: The goal of the Cancer Moonshot is to make a decade of progress in preventing, diagnosing and treating cancer in five years, ultimately striving to end cancer as we know it. We are taking a critical look at the entire system of scientific research and medical care and figuring out ways for realigning the incentives of this system to promote breakthrough progress in preventing and treating cancer; creating a new paradigm of generating, sharing and integrating data to enhance patient care; and accelerating the process of bringing new prevention strategies, diagnostics and therapies to patients in communities across the world.

Patrick Soon-Shiong: Cancer MoonShot 2020 has several interrelated aims: first, to gain insight into the heterogeneous nature of the cancer cell through the most comprehensive molecular analysis, from the genome to the transcriptome to the proteome and immunome; second, with this insight, to overcome cancer's ability to avoid the immune system; third, to use combination immunotherapy to activate the entire immune biological armamentarium, from innate to adaptive immunity, against the cancer; fourth, to deliver all this in near real time for cancer patient care by building a cloud-based, interoperable clinical learning system and a global precision-medicine network of community oncologists and pediatricians; and finally, to accelerate the development of molecular imaging and diagnostic tests combining whole-genome and RNA sequencing with targeted proteomics as well as combination immunotherapy and drug development, all within a decade to five years.

Ultimately, the aim is to win the war on cancer—to get to a point in the very near future when we are managing cancer the same way we might manage any chronic disease, such as diabetes or asthma.

What are the milestones for your projects?

DePinho: Our overarching goal is to more rapidly and dramatically reduce mortality and suffering in cancer, aiming for every patient to contribute to, and potentially benefit from, our efforts. Each moon shot has individual goal-oriented milestones that are evaluated on a semiannual basis to discuss progress and explore additional points of collaboration. In the long term, collaboration among moon shots, platforms, basic scientists and clinicians will

increase understanding of the molecular details of cancer, drive new treatments and accelerate cures.

There have already been achievements. Just in immuno-oncology alone, we have launched about 125 clinical trials. By late 2015, our effort had also established a secure database containing clinical information for more than 230,000 patients treated at MD Anderson since 2012, along with full integration of genomic, proteomic and immune profiling data from those patients involved in moon shot research studies.

There is a lot of discipline in the way we structured the program, a high level of accountability and a strong focus on milestones that in the beginning are interim for impact on the cancer problem. To give a specific example: in our cancer control platform, where we focus on things like policy and K-12 education, one milestone we had was that in a given year, we were going to work with many groups to reach stakeholders and legislatures across five different states, to try to educate the public and policy makers about the impact of laws that prevent children under the age of 18 from accessing tanning beds. This effort allowed the MD Anderson Melanoma Moon Shot team and the cancer control platform to provide Texas lawmakers with science-based data that resulted in a state-wide ban on minors' use of tanning beds. Texas was the fourth state to adopt this legislation in 2013, and, today, 14 states have passed the same restriction.

Simon: President Obama established the Cancer Moonshot Task Force to coordinate expertise across 20 agencies, sub-agencies and White House offices, as well as to serve as a catalyst for stimulating further private sector advances. This group has been hard at work, with new programs, policies and initiatives being developed and implemented at an accelerated pace. Milestones will include the launch of a series of efforts focused on the spectrum of cancer research and care throughout this year and a report delivered to the President by December 31 outlining the roadmap for building upon this foundation.

Soon-Shiong: Our first milestone is to test 100,000 patients using the 'GPS Cancer' test, which was launched by my company NantHealth (Culver City, California) in January. GPS Cancer is the first CLIA-certified, comprehensive multiomic-based test that integrates sequencing of the whole genome, transcriptomics and targeted proteomics with predictive analytics, resulting in a comprehensive molecular profile of a patient's cancer, which can then inform personalized treatment options. Coverage for the GPS Cancer test has been made available to eligible members of Independence Blue Cross commercial plans since the beginning of March.

Next, 20,000 of these test patients will be matched up and enrolled with an appropriate phase 2 immunotherapy clinical trials in over 20 tumor types, including breast, lung, prostate, ovarian, brain, head and neck, and multiple myeloma. Overall, more than 60 novel immunotherapy and targeted-therapy agents have been made available by pharmaceutical and biotech partners of 2020 for these tests. The governing master trial protocol, called the QUILT Program (Quantitative Integrative Lifelong Trial), is designed to harness and orchestrate all the elements of the immune system (including dendritic cell, T-cell and natural killer (NK) cell therapies) by testing novel combinations of vaccines, cell-based

immunotherapy, metronomic chemotherapy, low-dose radiotherapy and immunomodulators and checkpoint inhibitors in molecular-typed patients, with the goal of achieving durable, long-lasting remission for patients with cancer.

A final milestone of our MoonShot is that the findings from QUILT will inform phase 3 trials that ultimately result in the creation of an effective vaccine-based immunotherapy to combat cancer by 2020.

What specific approaches and technologies are you adopting?

DePinho: Our moon shots are supported by ten ‘implementation’ platforms focused on driving actionable knowledge into clinical endpoints by assembling leading-edge expertise and technology infrastructure in four major areas: data generation (via a cancer genomics laboratory (CGL) platform, an immune checkpoint therapy group and a translational center (CCGT) for discovering and developing targeted therapeutics); product development (via expertise in the development of small molecules (IACS), biologics (ORBIT) or adoptive cell therapies); data integration analysis (via advanced analytic platforms for clinical decision-making and an institutional longitudinal patient disease registry); and finally, a cancer prevention and control effort.

As I mentioned, we now have profiles for over 230,000 MD Anderson patients that combine clinical information with tumor ‘omic’ and immune monitoring data. Over time, additional research data will be added. This ‘learning system’ will provide unprecedented, controlled access for MD Anderson researchers to generate hypotheses and make connections between tumor characteristics, as detailed from molecular analysis, test results, imaging and other sources, and the efficacy of treatment. Our big data warehouse is designed ultimately to accept and share data from external sources as well.

Simon: Under the Cancer Moonshot, we are working to apply 21st century technology to transform knowledge and data into real solutions for patients. For example, we are working to apply the nation’s most powerful computational capabilities to analyze vast and complex datasets to make sense about which treatments work for which patients. We are also focused on bringing together the numerous stakeholders to incentivize new ways of stimulating action and fostering collaborations, which will be key to the success of this effort.

Investments from the National Cancer Moonshot Initiative will support cutting-edge research opportunities. These include cancer vaccine development, technologies for the development of diagnostics for early cancer detection, immunotherapies and combination therapies for an expanded range of cancers, genomic profiling of primary tumors and their surrounding cells, technologies for facilitating data sharing, and programs for targeting pediatric cancers. An Oncology Center of Excellence [will run] under the auspices of the US Food and Drug Administration (FDA) to leverage the combined the combined skills of regulatory scientists and reviewers with expertise in drugs, biologic and devices. Finally, under the umbrella of the initiative, a Vice President’s Exceptional Opportunities in Cancer Research Fund is planned to provide financing to scientists, cancer physicians, philanthropic

organizations, and pharma and biotech companies working together on major new innovations in the understanding of, and treatment for, cancer.

Soon-Shiong: As I mentioned, there will be detailed molecular characterization of the patient's tumor and immune status via diagnostic testing. This will be synergized with all the therapeutic tools at our disposal—from standard chemotherapy, to radiotherapy, to targeted therapy, to monoclonal antibody therapy, to cell-based immune therapy, to chemokines and cytokine therapy, to approved drugs and to drugs in development.

With these tools in place, we can establish a real-world, rapid clinical learning system to understand how we could optimize this maze of combination therapy. This will require health information technology and next-generation interoperable software. NantHealth's clinical-decision support software provides evidence-based treatment support, which may include QUILT trial identification based on genomic and proteomic sequencing results. The company's provider portal technologies will also allow oncologists and other clinicians to check patient eligibility for sequencing, request health plan authorization, and monitor and view test results, while helping to coordinate the patient's care across the care continuum.

How are these efforts being funded?

DePinho: We have mobilized more than $600 million over the past couple of years for these projects, with nearly $350 million in private philanthropic commitments. These philanthropic funds are complemented by several hundred million dollars raised through grants from the US National Institutes of Health (Bethesda, MD) and the Cancer Prevention & Research Institute of Texas and contracts as well as by returns from IP [intellectual property], which are plowed back into our mission to end cancer. MD Anderson ranks number one in the nation in IP-related commercialization revenues as well as corporate alliance revenue, outpacing all universities in the country.

Simon: The administration is launching the National Cancer Moonshot with $1 billion in funding. Information can be found at https://www.whitehouse.gov/the-press-office/2016/02/01/fact-sheet-investing-national-cancer-moonshot. Funding will include $195 million in new cancer activities at the National Institutes of Health (NIH) in fiscal year 2016. In fiscal year 2017, the Administration's budget will propose to continue this initiative with $755 million in mandatory funds for new cancer-related research activities at both NIH and the FDA. The Departments of Defense and the Veterans Affairs are also increasing their investments in cancer research, including the funding of Centers of Excellence focused on specific cancers and conducting large longitudinal studies to help determine cancer risk factors and enhance treatment.

Soon-Shiong: Thus far, large biopharmaceutical companies have taken the lead to support the efforts. Specifically, Amgen (Thousand Oaks, California), Celgene (Summit, New Jersey) and my ecosystem of companies forming NantWorks have committed to this effort. NantWorks will shortly announce the funds that it has raised in support of the goals of Cancer MoonShot 2020, and it is expected that the amount will exceed a billion dollars. In

addition, Independence Blue Cross and selfinsured payers, such as Bank of America (New York), have committed to fund the GPS screening test needed.

Finally, the NantHealth Foundation and the Chan Soon-Shiong Institute of Molecular Medicine at Oxford University, UK, a 501c3 medical research organization, has been formed and will commit a billion dollars to support cancer centers around the nation and community oncologists to make available the treatments and trials of the initiative.

The funding needed to win the war on cancer should be considered in the context of that needed to place a man on the moon or the successful NASA Space shuttle program with its multiple launches. To sufficiently fund this initiative, combined support is needed from philanthropy, the private sector, government agencies and even NGOs [nongovernmental organizations].

Who are the main stakeholders?

DePinho: The Moon Shots Program has brought together nearly 2,000 faculty and staff out of 21,000 employees at MD Anderson. It's a significant effort involving basic scientists, oncologists, surgeons, radiologists, physician–scientists, experts in data management and analysis, cancer prevention and control, and other disciplines in 12 different cancers.

Using immunotherapy as an example, we've signed research collaborations with a dozen companies, including multinational pharma companies like Pfizer (New York), Bristol-Myers Squibb (Princeton, NJ), GlaxoSmithKline (London), Johnson & Johnson (New Brunswick, NJ) and MedImmune (part of London-based AstraZeneca) and biotech companies like Intrexon (Germantown, MD), Ziopharm (Boston), Kymab (Oxford, UK), Astellas Pharma (Tokyo), CytomX Therapeutics (S. San Francisco, CA), Jiangsu Hengrui Medicine (Shanghai) and AbbVie Biotherapeutics (Redwood City, CA)—not only to conduct clinical trials for their experimental drugs, but also to bring our scientists and theirs together to compare notes on research and design preclinical studies and new clinical trials to improve treatment. As always, we collaborate in clinical trials with other cancer centers testing new immunotherapies, with several thousand patients involved in more than 165 such trials now open at MD Anderson.

In cancer prevention and control, we work closely with advocacy groups, like the CATCH Foundation and Tobacco-Free Kids, and our governmental relations colleagues to educate legislators. In addition, we have extensive interactions with governmental agencies such as the CDC [US Centers for Disease Control and Prevention] (Atlanta) and have engaged in strategic public health discussions with other governments, including China, Portugal and Mexico.

Simon: The Vice President is issuing the call far and wide, recognizing that innovative solutions to tough problems often arise from unlikely sources or the combination of seemingly disparate expertise. Thus, the Cancer Moonshot aims to break down conventional silos and bring together patient advocates, health care providers, biomedical researchers, technological innovators, industry leaders and more to serve the ultimate Cancer Moonshot stakeholder: the patient.

Dinah Singer: Readers can find members of the Task Force and of the Blue Ribbon Panel at their respective websites (https://www.whitehouse.gov/the-press-office/2016/01/28/memorandum-white-house-cancer-moonshot-task-force and http://www.cancer.gov/research/key-initiatives/moonshot-cancer-initiative/blue-ribbon-panel). The 28-member Blue Ribbon Panel represents a spectrum of scientific areas, including biology, immunology, genomics, diagnostics, bioinformatics, and cancer prevention and treatment. Scientific members also include investigators with expertise in clinical trials and cancer health disparities. Importantly, the members of cancer advocacy groups and pharmaceutical and biotechnology companies will be represented on the panel and its working groups. The Panel has announced seven working groups, involving 10 to 12 leading experts per group. The working groups are tasked with helping the Panel develop two to three recommendations for major research opportunities that could lead to significant breakthroughs.

Community input will be critical to success and members of the scientific community and general public are encouraged to submit ideas for advancing progress against cancer in one or more areas on the Cancer Research Ideas platform here: https://cancerresearchideas.cancer.gov/a/pages/about.

Soon-Shiong: We have nearly 150 leaders from a diverse community across pharma, biotech, technology, academic and community oncology, the US National Cancer Institute (NCI) and not-for-profit thought leaders.

Industry partners include companies from the pharmaceutical and biotech sector and from communications technology. For the former, participants include Celgene, Amgen, NantWorks, NantKwest (San Diego), Etubics (Seattle), Altor Bioscience (Miramar, Florida) and Precision Biologics (Westlake Village, California). In communications technology, we have brought on board such leaders as Allscripts (Chicago) CEO, Paul Black and Blackberry (Waterloo, Ontario, Canada) CEO John Chen.

Major participating academic cancer centers include Johns Hopkins (Baltimore), Tufts University (Boston) and Columbia University (New York); community hospital and ambulatory settings are also contributing under the Precision Medicine Global Network, which includes nearly 1,000 oncologists in the United States.

We also have as partners leading insurance companies, such as Independence Blue Cross CEO Daniel Hilferty and Bank of America's (New York) Head of US Health and Wellness Benefits Jim Huffman, who have committed to support payment for our diagnostic tests. The NCI also is playing a direct role. The US-wide master protocol will be designed under CRADA agreements between companies and the NCI, with scientific guidance provided by the NCI's branch chiefs of Tumor Immunology and Biology Laboratory and Medical Oncology using the recently published US Food and Drug Administration guidance on 'Co-development of two or more new investigational drugs for use in combination'.

Are you concerned about confusion on the different cancer moonshots?

DePinho: In the first three years of our Moon Shots Program, we've had early successes and accomplished great strides, and there's so much more ahead of us in this golden era of

cancer research. I had the privilege of speaking several times with Vice President Biden over the past few years about our program and how we organized our efforts. The newly announced national moon shot, under the leadership of the Vice President, will only further stimulate collaboration and achievement in our nation's fight to end cancer. We also are pleased to have two of our best cancer scholars—Jim Allison and Al Yung—on the Blue Ribbon Panel to advise the NCI in its work with Vice President Biden. We all plan to work together for our patients.

Simon: The purpose of the Cancer Moonshot is to inspire the people all around the world to rise to the charge from the President. Any effort focused on accelerating preventing, diagnosing and treating this terrible disease is a win.

Douglas Lowy: The Vice President's enthusiasm is welcomed by the community of researchers, health professionals and patients who share his passion and belief that great things are possible by accelerating cancer research with leadership and resources. We are committed to breaking down silos and stimulating the groundbreaking work already underway. To be successful, we must hear a broad range of perspectives to take full advantage of the exceptional current opportunities in cancer research.

Soon-Shiong: No, not concerned at all. These moonshots are mutually reinforcing and ultimately share the same goals—to win the war on cancer. The Cancer MoonShot 2020 program has been my vision for over a decade, culminating in its 'liftoff' in January 2016. Our initiative has a laser focus on patients with active disease and to accelerate combination immunotherapy and big data integration in near real time. It is a highly focused effort that specifically looks at the potential of combination immunotherapy as the next standard of care for cancer patients, by leveraging insights from DNA and RNA sequencing with quantitative proteomics. It is led by the private sector and brings together pharma, biotech, payers, academia and community oncologists for the aspirational goal of developing a cancer vaccine by 2020.

Biographies

Ron DePinho

Greg Simon

Patrick Soon-Shiong

Dinah Singer

Douglas Lowy

US vice president Biden has been on a fact-finding tour of laboratories and institutions around the US as he seeks to lead the National Cancer Moonshot. Vice President Joe Biden speaks with Nobel laureate Paul Modrich, left, as Vickers Burdett, wife of Modrich, middle, and A. Eugene Washington, Chancellor for Health Affairs at Duke University, right, listen in a laboratory at Duke University School of Medicine in Durham, North Carolina, on Wednesday, February 10, 2016. Vice President Biden visited Duke to speak about his Cancer Moonshot initiative. (AP Photo/Ben McKeown)