THE CANCER LETTER

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Obama Announces Moonshot to Cure Cancer

By Matthew Bin Han Ong

President Barack Obama announced a moonshot aimed at curing cancer, a project to be led by Vice President Joe Biden.

The United States can do "so much more," Obama said in his seventh and final State of the Union address Jan. 12. "Last year, Vice President Biden said that with a new moonshot, America can cure cancer. Last month, he worked with this Congress to give scientists at the National Institutes of Health the strongest resources they've had over a decade.

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When Moonshots Collide

By Matthew Bin Han Ong

Did Patrick Soon-Shiong attempt to scoop President Barack Obama's State of the Union address?

Several days before Obama announced the federal government's moonshot to cure cancer, Soon-Shiong put out a draft press release, claiming that the White House, NIH, FDA and pharmaceutical companies have united in "Cancer MoonShot 2020," an immunotherapy clinical trials program he devised.

Soon-Shiong, founder and CEO of NantWorks and the Chan Soon-Shiong Institute of Molecular Medicine, ultimately announced his moonshot on Jan. 11, a day before Obama announced his.

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Conversation with The Cancer Letter

Soon-Shiong Says FDA & NCI are Onboard For His Moonshot; Feds Deny Involvement

Government agencies said the biotechnology billionaire Patrick Soon-Shiong had overstated the extent of their involvement in "Cancer MoonShot 2020," the immunotherapy clinical trials program he put together.

In an in-depth conversation with Matthew Bin Han Ong, a reporter with The Cancer Letter, Soon-Shiong said that while his program doesn't seek federal funds, it has the support of NCI and FDA officials.

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Obama Announces Moonshot In State of the Union Address

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"Tonight, I'm announcing a new national effort to get it done. And because he's gone to the mat for all of us, on so many issues over the past 40 years, I'm putting Joe in charge of mission control. For the loved ones we've all lost, for the family we can still save—let's make America the country that cures cancer once and for all."

America now has three separate cancer moonshots:

• **A Moonshot to Cure Cancer:** Obama and Biden plan to "increase resources—both private and public—to fight cancer," as well as "break down silos and bring all the cancer fighters together—to work together, share information, and end cancer as we know it," Biden wrote this week.

• **The Cancer MoonShot 2020 Program:** Patrick Soon-Shiong, billionaire founder and CEO of NantWorks and the Chan Soon-Shiong Institute of Molecular Medicine, launched his own moonshot Jan. 11, the day before Obama's State of the Union address. Soon-Shiong's National Immunotherapy Coalition, which he claims is an "inclusive" effort involving many public-private partnerships, aims to "get to the path of the cure as fast as we can."

• **MD Anderson Cancer Center's Moon Shots Program:** Launched in 2012, MD Anderson President Ronald DePinho aimed to "end cancer" and pledged to "kick cancer's butt" by rapidly advancing progress in six cancer types.

"MD Anderson President Ronald DePinho has talked with Biden during the past year about cancer research, prevention and treatment, and the way forward to accelerate progress," MD Anderson said in a statement to The Cancer Letter. "Our Moon Shots Program leaders also had an opportunity to meet with him, so the vice president is aware of our established program. Along with others in the field, we look forward to continuing to confer with Vice President Biden about the scope and potential of a national initiative.

"We certainly agree the time is ripe for concerted efforts to reduce deaths from cancer. Because Vice President Biden is so well respected, he's an ideal leader to spark new collaborative efforts against these diseases."

The metaphors of war and space travel aren't new. Vows of this sort were a prominent part of political buildup that produced the National Cancer Act of 1971, and have resurfaced regularly since. However, America's top-ranking scientists are saying that this era is different.

This time, two of the moonshots appear to have collided: one from the White House, and the other from Soon-Shiong, who declared that Biden and several key government officials support his program. NCI and FDA officials said the agencies aren't involved.

Details of this cosmic collision emerge on page 1.

Biden's moonshot comes seven months after his son, Beau Biden, the attorney general of Delaware, died of brain cancer in May 2015 at the age of 46. In December of last year, the vice president helped secure a $264 million funding boost for NCI, the largest increase in over a decade of flat budgets and decreasing purchasing power.

Biden's initiative arrives at an especially promising time in cancer research, NIH Director Francis Collins said during a press call Jan. 14.

"There's strong interest in the vice president's office, and I think rightly so, in bringing together academia, industry, philanthropy, and others and figuring out new kinds of partnerships and particularly assisting in data sharing, something that doesn't always happen when it should, and building new partnerships between the public and private sector," Collins said. "So the goal then, I think, now that Vice President Biden has accepted the charge to lead, is to pull those kinds of opportunities together—to get all the kinds of input that are needed. And he's already met with more than 200 scientists; he's on a very steep learning curve…

"Again, I think we need to be careful not to overpromise, but I think everyone who studies this issue would agree that we are at an especially promising time, and to have the vice president, given the charge by the president—this is moment to really pull out the stops. It is very energizing and exciting to all of us who have

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dreams about where this could go."

The details of funding for Biden's moonshot have not been disclosed, and it's not publicly known how much money will be distributed.

"As you know, when the president makes the State of the Union address, and a couple weeks after that, that's followed with the president's budget," Collins said. "In the meantime, it's not possible to disclose what kind of content might be in there. But on Tuesday, Feb. 9, the president's budget will be revealed, and I think that will be the point at which you would want to look closely and see what kind of resources are attached to this clearly very strong presidential and vice presidential priority."

NCI Acting Director Doug Lowy cautioned that the moonshot should not be expected to cure cancer.

"The notion of a moonshot, which the vice president articulated, I think, should be seen as one of being aspirational, not business as usual, which would translate into much faster progress," Lowy said during the press call. "The faster progress is not going to take care of the cancer problem in the next month, the next year, or even the next couple of years.

"But the opportunities that Francis mentions are enormous at this time and we really applaud the vice president for his commitment what we know will be equally as important, his follow through."

The Obama-Biden Moonshot

The American Association for Cancer Research has put together a group of 15 of its members, led by AACR President José Baselga, several board members and other leaders from 10 of the top medical institutions in the U.S.

According to AACR, the group met with Biden's senior staff Jan. 8 to discuss the state of cancer research.

"We have indeed reached an inflection point, where the number of discoveries that are being made at such an accelerated pace are saving lives and bringing enormous hope for cancer patients, even those with advanced disease," said Baselga in a statement, who is also physician-in-chief and chief medical officer at Memorial Sloan Kettering Cancer Center.

"Now is the time for a major new initiative in cancer science that supports and builds upon our basic science foundation while translating these exciting scientific discoveries into improved treatments for cancer patients, such as in the areas of genomics, precision medicine, and immuno-oncology."

The moonshot will reduce cancer-related human suffering and loss of life, the American Society of Clinical Oncology said.

"We must recommit to vastly speeding the discovery of new cancer treatments and enabling the possibility of precision medicine for every individual with cancer," said ASCO Chief Medical Officer Richard Schilsky. "With the effective application of Big Data initiatives, such as ASCO's CancerLinQ rapid-learning system, insights that have taken years to discern could happen much more quickly, helping us to better understand which treatments work best for each patient and the high impact areas where additional research is critically needed."

The moonshot will help provide the funding needed to accelerate progress, said Ellen Sigal, chair and founder of Friends of Cancer Research.

"We have made incredible progress in fighting cancer already. Making many forms of it curable today," Sigal said to The Cancer Letter. "What we need from a moonshot like this is the ability to truly have a collaborative ecosystem where the best minds, best tools and yes, very significant funding increases will be needed to make truly curing cancer a reality.

"While this is at the end of a Presidency, with the Vice President taking the lead, it can be the beginning of something great for future administrations. Cancer won't be cured in the next 8 months but it can be cured in the years ahead. It is important to note other fantastic initiatives already underway at the NIH, and important very complimentary programs initiated by congress in 21st Century Cures. Many programs outlined in that bill, and its Senate counterpart, can really help springboard this initiative."

The White House program to cure cancer should also include public health measures, said Otis Brawley, chief medical officer at the American Cancer Society.

"I'm very pro-moonshot. It is my belief that a concerted effort that looked at both improvement and research aimed at developing new therapies, research on how to prevent cancer, and how to disseminate research findings, and then programs to apply research findings is what is absolutely necessary," Brawley said to The Cancer Letter. "One of the things that I've been pointing out this week, when we're talking about dissemination of information and dissemination of research findings, we have a 40 percent decrease in breast cancer death rate in the U.S., despite the fact that 14 states have little or no decrease in death rate. The point is, a large proportion of women in those 14 states have not enjoyed the benefits of the research that has already been done.

"I think the 'cure' is a good aspirational goal. I have no problem with that language. I think what is more likely to happen is, with a concerted effort, some

cancers will be cured and more will be treated in the more chronic disease model like diabetes or AIDS where people can live in peaceful coexistence with their disease for years."

Biden's moonshot will be of "tremendous help," said Samuel Silver, chair of the board of directors of the National Comprehensive Cancer Network.

"As a treating physician of patients with cancer, personally as a cancer patient, and as a son whose parents both died of cancer, this is something I confront and think about every day," said Silver, assistant dean for research and professor of internal medicine at the University of Michigan Comprehensive Cancer Center. "I applaud Vice President Biden's moonshot initiative to cure cancer. His vision of breaking down silos, funding cancer researchers, enhancing the care provided by community physicians, stimulating discovery by the pharmaceutical industry, and bringing the cancer community together will be of tremendous help.

"However, we must realize that even though we are on the cusp of many important breakthroughs, cancer is a difficult disease caused by many different mechanisms, and it will require funding of everything from basic research to translational research, to important and well-thought-out trials, to the delivery of cancer care to our patients in order to make this initiative successful."

The moonshot will foster greater collaboration among stakeholders, said Wendy Selig, president of the National Coalition for Cancer Research.

"NCCR commends and embraces the administration's bold plan to significantly accelerate the pace of cancer research and foster greater collaboration among those involved in this effort," Selig said. "We are living in an era of unprecedented advances in our understanding of cancer, which have led to the development of treatments that were unthinkable a short time ago such as cancer immunotherapy and targeted cancer therapeutics.

"We must harness the expertise, commitment and energy of the scientific and patient communities throughout the world to continue and build on that knowledge," said Selig.

"Passage of the bipartisan 21st Century Cures Act by the House of Representatives signaled an important step forward in this conquest. NCCR looks forward to working closely with the administration, bipartisan congressional leaders and our colleagues in the cancer community to ensure the resources and strategies are in place to make the laudable and obtainable goal of a decade worth of advances in five years a reality."

When Moonshots Collide

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Sources said government officials asked Soon-Shiong to remove federal agencies from the press release.

In the final version of the release, Soon-Shiong stated that "the ambitious goals of the Cancer MoonShot 2020 Program were presented at a meeting hosted by Vice President Joseph Biden at his Naval Observatory residence in Washington, D.C. on Dec. 1, 2015, where members of the coalition presented their shared vision for translating the promise of precision medicine through the delivery of combination immunotherapy to routine clinical cancer care, as well as their shared commitment to accelerate the development of immunotherapy and vaccine therapy as the next generation evolution of cancer care," the statement reads.

In other documents, Soon-Shiong lists the names of the "members of the coalition"—staff members from the White House, NCI and FDA. Three hours before Obama's address, Soon-Shiong and other coalition members reiterated that the program has the backing from government personnel, during a public webcast at the JP Morgan Healthcare Conference Jan. 12. He repeated this claim in a conversation with this reporter Jan. 14. The conversation appears on page 1.

Soon-Shiong said his moonshot and Biden's moonshot have "common goals."

"By November, after [Biden] made the decision not to run [in the 2016 presidential race], he came quietly to Los Angeles and spent three hours on our campus," Soon Shiong said. And then I gave him the second white paper, and I said to him, 'Look, we can convene all the right people to make it happen,' because he said to me, 'Patrick, what can do to make this move faster?' So the vice president and I have common goals here to accelerate the development of drugs so we can actually get to the path of the cure as fast as we can."

White House insiders said that while Biden and his staff have participated in dozens of listening sessions, the vice president and other federal agencies are not involved in Soon-Shiong's moonshots program.

NCI and FDA have been supportive of the Cancer MoonShot 2020 program, Soon-Shiong said to The Cancer Letter.

"I've got to tell you, what was incredibly exciting is there wasn't any roadblock from the regulatory agencies," Soon-Shiong said. "I mean, the FDA was amazingly supportive—Bob Califf [who has been nominated to serve as the next FDA commissioner] and Janet Woodcock [director of the FDA Center for

Drug Evaluation and Research] and Peter Mark [deputy director for the FDA Center for Biologics Evaluation and Research] were incredibly supportive.

"This weekend is going to be very exciting, where I will be working with investigators at the NCI in drafting the clinical trial design."

NCI and FDA are not involved with Soon-Shiong as formal partners and the two moonshots are unrelated, said NIH Director Francis Collins.

"I think there has been some confusion and some misunderstanding, and maybe the word moonshot has been utilized by lots of people over time, and in this case, is somewhat a source of confusion," Collins said during a press call Jan. 14. "Dr. Soon-Shiong obviously is a highly-regarded entrepreneur, very interested in this space, but the program that he talked about at JP Morgan earlier was not one that involves the NCI or the FDA in a partnership way.

"We're obviously interested in all groups that have something to contribute here, but make no mistake, what the vice president announced in terms of his blog and what the president was talking about in the State of the Union is not the same thing as what was discussed at JP Morgan."

Soon-Shiong said that two NCI investigators, Jeffrey Schlom, chief of the NCI Laboratory of Tumor Immunology and Biology and James Gulley, chief of the NCI Genitourinary Malignancies Branch, and director of the NCI Medical Oncology Service at the Office of the Clinical Director, are intimately involved in clinical trial designs for the coalition.

"Well, I can only tell you the fact that Jeffrey Schlom is actually spending three days with me right now designing clinical trials this weekend. You're welcome to call him and James Gulley," Soon-Shiong said. "I just got off the phone 10 minutes ago saying how he's flying to Miami this weekend to talk to one pharma company, and I told him I spoke to the CEO of another pharma company and we're talking about an anti-PDL1 that he's designed. So all I can tell you is what we are doing."

In response to an inquiry about NCI staff meeting with Soon-Shiong, officials said that Schlom and Gulley's participation should not be construed as an endorsement from the institute.

"NCI intramural researchers are participating in a scientific meeting in Santa Cruz, Calif., hosted by Patrick Soon-Shiong. Their involvement and support, however, are limited to the activities under the terms of their CRADA with Etubics," NCI Acting Director Doug Lowy said to The Cancer Letter. "Often CRADAs provide for participation in scientific meetings, as is the case in this situation. The participation by NCI investigators should not be taken in any way as endorsement of the coalition that Dr. Soon-Shiong has recently announced.

Soon-Shiong said Collins isn't involved in his Cancer MoonShot 2020 initiative.

"I think it is unfair, frankly, to ask a member of the government who wasn't at the meeting [with Soon-Shiong, Biden and other alleged members of the coalition]," Soon-Shiong said. "This is very complex.

"Dr. Francis Collins is not FDA nor NCI, right? He's the head of the NIH, and I don't know if you want to mix this up with the Precision Medicine Initiative. If Dr. Francis Collins said he had nothing to do with it, I think he's correct.

"But I think, look, what happened was, you know, Robert Califf was at the meeting."

FDA officials said that the agency isn't involved in Soon-Shiong's program either.

"The FDA is not participating in the National Immunotherapy Coalition announcement," FDA officials said in a statement to The Cancer Letter. "We look forward to joining our HHS colleagues and working with the Vice President to determine how the FDA might enhance efforts to further new and innovative ways to fight cancer."

Draft Press Release Quotes Government Officials

Soon-Shiong's draft press release—which was not published, but was obtained by The Cancer Letter—included laudatory comments by NCI and FDA officials. In a conversation with this reporter, Soon-Shiong said the quotes were sent to him by the individuals to whom they were attributed.

"Let me just set the facts straight. Every member of the FDA or NCI—they actually sent me their quotes," Soon-Shiong said. "So I'm not sure how could they... okay?"

The text of the draft press release follows:

HISTORIC CANCER ALLIANCE FORMED: THE NATIONAL IMMUNOTHERAPY COALITION TO LAUNCH THE CANCER MOON SHOT 2020 PROGRAM

Nation's Most Comprehensive Cancer Collaborative Initiative Comprising Large Pharma, Biotech, FDA, NCI, DOD, Community Oncologists, NCI Designated Academic Cancer Centers, National Insurance Carriers and Fortune

500 Self-Insured and Technology Companies Seeking to Accelerate the Development of Next Generation Immunotherapy Combination Cancer Therapies Across All Tumor Types

San Francisco – January 12, 2016 – Today, leaders from major pharmaceutical and biotech companies, NCI-designated academic cancer centers, community oncologists, national and regional insurance carriers, together with Fortune 500 self-insured and technology companies announced the launch of **The National Immunotherapy Coalition (NIC)**, a historic coalition formed with the support of the U.S. Food and Drug Administration (FDA) and the National Cancer Institute (NCI) Immunology and Medical Oncology NCI branches , having a singular focus on accelerating the development of paradigm changing combination immunotherapies as the next generation standard of care in patients with cancer.

Integrating a diverse group of stakeholders from the private sector and government, the National Immunotherapy Coalition, is a unique translational medical platform, guided by the Chiefs of the Immunology and Medical Oncology branches of the NCI in collaboration with FDA, pharma and payors, with a goal to validate the potential of next generation panomic molecular analysis and novel combination immunotherapies. The Coalition will design, initiate and complete randomized clinical trials in cancer patients at all stages of disease in up to 20 tumor types in as many as 20,000 patients by the year 2020: **The Cancer Moon Shot 2020 Program** (http://www.cancerMoonShot2020.org). A nationwide clinical trial and master protocol will be designed under the guidance of the Branch Chiefs of Tumor Immunology and Biology Laboratory and Medical Oncology at the NCI, in collaboration with the FDA, together with academic cancer centers, pharma and biotech partners participating in the National Immunotherapy Coalition.

This master protocol, entitled the **QUILT Trial (Quantitative Integrative Lifelong Trial),** is designed to harness and orchestrate all the elements of the immune system (including dendritic cell, T cell and NK cell therapies) by testing novel combinations of vaccines, cell-based immunotherapy, metronomic chemotherapy, low dose radiotherapy and immunomodulators, in patients who have undergone next generation whole genome, transcriptome and quantitative proteomic analysis, with the goal of achieving durable, long-lasting remission for patients with cancer. These immunotherapy based, genomically and proteomically informed clinical trials will pave the way to delivering cancer therapy with the lowest toxicity and the highest quality of life.

The mission of the Cancer Moon Shot 2020 Program is to rapidly enroll and complete randomized Phase 2 clinical trials to validate the potential of pan-omic (whole genome, transcriptome and proteomic) analyses and to evaluate novel combination immunotherapies as the next generation standard of care. Utilizing a cloud -based infrastructure to integrate and enable the participation of both major academic and community oncologists at a national scale, the goal is to complete randomized clinical trials in patients with cancer at all stages of disease, across up to 20 tumor types in 20,000 patients within the next 24-36 months. By comparing standards of care to the next paradigm of less toxic immunotherapy combination therapy, the findings of these randomized QUILT trials will inform the design of Phase 3 registration trials, with the goal of bringing transformative advances in combination immunotherapies to cancer patients by 2020.

The ambitious goals of the Cancer Moon Shot 2020 Program were presented at a meeting hosted by Vice President Biden at his Naval Observatory residence in Washington DC on December 1, 2015 where members of the coalition, including leadership at FDA and branch chiefs of the NCI, presented their shared vision for translating the promise of precision medicine through the delivery of combination immunotherapy to routine clinical cancer care, and their shared commitment to accelerate the development of immunotherapy and vaccine therapy as the next generation evolution of cancer care.

Attendees of that meeting, convened and chaired by Dr. Patrick Soon-Shiong, Founder of the Chan Soon-Shiong Institute of Molecular Medicine (CSSIOMM), a non-profit medical research institute, and NantWorks, an ecosystem of healthcare companies, included leadership from the immunotherapy and medical oncology branches of the NCI, leadership from the FDA, leadership from the DOD, leadership from the pharmaceutical industry including Amgen, GlaxoSmithKline, Pfizer, leadership from national payors including Blue Cross and Bank of America and healthcare leaders from security and interoperability organizations including CEOs from Allscripts and Blackberry.

Major academic cancer centers represented at this meeting included center directors from Massachusetts General Hospital, Johns Hopkins University, University of Miami, University of Utah, Tufts Cancer Center and the Walter Reed National Military Institute as well as

representatives from the oncologists in the community.

"There are unique times in history when events and advancements in technology converge to elicit a quantum leap in medical care. This is not only a unique time, but also a unique inflection point in the history of cancer," said **Patrick Soon-Shiong, M.D.**, **Founder and Chief Executive Officer of NantWorks and the Chan Soon-Shiong Institute of Molecular Medicine.** "Our knowledge in the science of genomics, proteomics, immunology and immunotherapy has advanced and converged at an unprecedented speed, making now the time for the rapid deployment and orchestration of immunotherapy for the benefit of millions of cancer patients. The Cancer Moon Shot 2020 Program, the National Immunotherapy Coalition and the QUILT Trial are designed to do just that—to bring together a diverse group of visionary leaders and stakeholders to pool resources working together to develop and bring to patients, a dramatic improvement in cancer care."

"Every day, Independence Blue Cross is reimagining health care in hopes to help our members succeed on their path to improved health and high quality care," said **Daniel J. Hilferty, Independence Blue Cross, President and Chief Executive Officer and Chairman of the Blue Cross and Blue Shield Association Board of Directors (BCBSA)**, comprised of 36 Independent Blue Cross Blue Shield companies that together cover nearly 105 million individuals – 1 in 3 Americans. The National Immunotherapy Coalition is allowing Independence to further enhance our contribution to the future of health care through our active involvement in this one-of-a-kind collaboration." Hilferty stated. "We look forward to continuing our work with this incredible team to develop the most innovative cancer fighting strategy in our lifetime. Through our Blue Cross Blue Shield Association we cover 1 in 3 Americans and insure individuals across every zip code in the country – this initiative is important to all Americans".

At the meeting with the Vice President and Dr. Soon-Shiong, the **Deputy Commissioner for Medical Products and Tobacco of U.S. Food and Drug Administration (FDA), Robert M. Califf, M.D.**, stated, "There come moments in time where we ask why drugs tested do not work? I can remember that moment in cardiology 30 years ago where we took out things that did not work and kept things that did. We may be at that moment in time with cancer. The FDA is here to act as the 'cattle prod' to accelerate this moment when we are beginning to figure out how cancer works. The FDA is ready to be part of this historic coalition where we can provide clinical trial guidance for the combination of novel innovative agents brought into the coalition from the Cancer Moon Shot Program 2020 members," he said. "By adopting innovative and adaptive clinical trials, the QUILT coalition will shepherd in a period accelerated by innovation in cancer treatment."

Janet Woodcock, M.D., U.S. Food and Drug Administration (FDA), Director of the Center for Drug Evaluation and Research (CDER) has led many of FDA's drug initiatives. She introduced the concept of risk management in 2000 as a new approach to drug safety. Since 2002, she has led the "Pharmaceutical Quality for the 21st Century Initiative," FDA's highly successful effort to modernize drug manufacturing and its regulation. In 2004, she introduced FDA's "Critical Path" Initiative, which is designed to move medical discoveries from the laboratory to consumers more efficiently. At the National Immunotherapy Coalition meeting hosted by Vice President Biden, Dr. Janet Woodcock made insightful comments and voiced the sentiments of many participants present at the meeting in support of the goals of the National Immunotherapy Coalition and the Cancer Moon Shot 2020 program, stating, "Current clinical trials are organized around disease rather than immunology approaches. This traditional approach takes too long. You can only answer one question at a time and it may take you four years to answer one question about one drug and one tumor. It takes too long and doesn't give you enough information. We need a large network to enroll a large number of patients. Almost all cancer patients are never enrolled in clinical trials. Almost all of them are never treated with investigational therapies. With regard to combinations, FDA issued guidance a number of years ago about investigational combinations. Development of Hepatitis C drugs is an example."

"My personal opinion: I believe we are approaching the science wrong," she added, "We have great basic science, we learn all this information but we don't test it in the clinic in an efficient manner. Anything we learn from a mouse or human pathology is an hypotheses, and often the hypothesis is wrong when we test it in people. In complexities such as we face today in cancer where we have next generation sequencing, when we wish to combine chemotherapy with immunotherapy, when we wish to explore multiple combinations, you're often asking five questions, not one. We need an engine that can turn around this knowledge rapidly and a process to help answer these questions effectively."

"An important issue is our need to involve the community, that's where the patients are," Dr. Woodcock

continued. "To date, trials have been centered around major medical centers and these trials are slow to accrue, since patients do not want to leave their community and doctors do not want to send them to remote centers. We need to build an infrastructure that will support these community doctors and allow them to participate and have their patients participate in these clinical trials without losing their patients and without having their patients to go away. "

Peter W. Marks, M.D., Ph.D., U.S. Food and Drug Administration (FDA), Deputy Director for the Center for Biologics Evaluation and Research (CBER) echoed the FDA's support of accelerating clinical trials through the QUILT process, stating, "Our goal is to enable important pathways for progress to get important therapies to patients. We will not be the impediment on the critical path at the FDA. Evidence generation needs to happen and we will work to streamline the process for vaccine development."

Dr. Jeffrey Schlom, Chief of the Laboratory of Tumor Immunology and Biology at the National Cancer Institute (NCI), directs a translational research program in cancer immunotherapy. He has pioneered the use of novel immunotherapeutics, both as monotherapy and in combination therapies, for a range of human cancers. His studies involve the translation of hypothesis-driven preclinical studies to science-driven clinical trials. Dr. Schlom's studies involve the design and development of novel therapeutic cancer vaccines, immunocytokines, and checkpoint inhibitor monoclonal antibodies.

His longstanding experience in the biology of cancer and the efforts needed to rapidly address immunotherapy drug development through a collaborative national immunotherapy coalition were shared with the Vice President on December 1st stating, "This coalition that has been brought together by Dr. Soon-Shiong to address cancer is one of the most comprehensive sets of talents, and is quite unique. I've seen it all but I've never seen anything quite like this in my years. I've been there at the original war on cancer during the Nixon Administration and saw the bureaucracy on how it played out."

"The issue we face today in cancer care is a paradox. It is clear that immunotherapy can work in managing cancer but this approach is only working in about 20% of patients now. The problem we face today is that there are about 2 dozen drugs in immunotherapy in the clinic right now and they are all being developed in silos by each individual company. This National Immunotherapy Coalition is designed to solve this problem. I'm extremely proud to be a part of this and have never seen a situation like this where we can get these trials done so rapidly. I've been revitalized, regenerated through this whole process." he said at the historic DC meeting.

Dr. James L. Gulley who has served at the National Cancer Institute (NCI) for 18 years also participated in the meeting with the Vice President and Dr. Soon-Shiong. In his role as **Branch Chief, Dr. James L. Gulley, Director of the Medical Oncology Service, Office of the Clinical Director**. Dr. Gulley echoed the sentiments of Dr. Schlom stating, "We are seeing a sea change on how we are treating patients with cancer with immunotherapy. We are seeing deep durable and rapid responses to these new immunotherapy agents that unfortunately are only in the minority of patients and in some cancers there's no responses at all. The best way to address these failures is to explore combination therapies and this National Immunotherapy Coalition is the vehicle to break down these silos and test these combinations rapidly. That's the way we are going to make progress. I am proud to be part of this important and historic immunotherapy initiative."

Col. Craig Shriver, MD, FACS (DOD) at the John P Murtha Cancer Center at Walter Reed Military Hospital and United States Army where he served for 30 years, provided his viewpoints as it related to the Military DOD Health Care and cancer care stating, "There are five requirements for the QUILT coalition that we are proud to be part of and they're directly related with what we do at our cancer center. We validate big science through our clinical trials network. There are 1.2 million active duty military members, 9.3 million beneficiaries that receive military DOD health care. That's a huge network. Just in our active duty force, we get a thousand active duty members a year that come down with cancer. If a thousand active duty members were still getting injured in Afghanistan or Iraq, we would not accept this. So it's the same thing with how militaries respond to infectious diseases… things that affect the readiness of our active force. So cancer is that threat. Care coordination is what we can provide since we have five other sites in our health system. We have a synergistic relationship with the NCI and that will broaden the access for these patients to these clinical trials. I am honored to serve and be part of this remarkable Coalition that Dr. Soon-Shiong has convened. "

"The DOD and Col. Shriver have established a long standing partnership with the Windber Research

Institute and through our collaborative efforts the DOD and Windber were responsible for providing over 90% of the breast cancer tissue genetically analyzed by the NIH Cancer Genome Atlas study. This human tissue repository is the nation's foremost Platinum-rated, CLIA CAP certified bio-repository for cancer tissue housing over 90,000 tissue specimens, and will be utilized as the tissue repository resource to support the QUILT Trial," said **Tom Kurtz, CEO of Windber Medical Center and Windber Research Institute.** "We are proud to be involved in this historic national initiative and to expand the efforts to elucidate the biologic mysteries of cancer and build on our work that has been so strongly supported by the DOD and NIH over the last decade" he said.

Attendees of the historic gathering hosted by Vice President Biden at his Naval Observatory residents included representatives from all elements of the health care ecosystem:

Government

Robert M. Califf, M.D., U.S. Food and Drug Administration (FDA), Deputy Commissioner for Medical Products and Tobacco

Janet Woodcock, M.D., U.S. Food and Drug Administration (FDA), Director of the Center for Drug Evaluation and Research (CDER)

Peter W. Marks, M.D., Ph.D., U.S. Food and Drug Administration (FDA), Deputy Director for the Center for Biologics Evaluation and Research (CBER):

Jeffrey Schlom, Ph.D., Center for Cancer Research, National Cancer Institute (NCI), National Institutes of Health (NIH) Chief - Laboratory of Tumor Immunology and Biology

James L. Gulley M.D., Ph.D., F.A.C.P., Center for Cancer Research, National Cancer Institute (NCI), National Institutes of Health (NIH) Head - lmmunotherapy Section, Chief - Genitourinary Malignancies Branch, Director - Medical Oncology Service

Col. Craig Shriver, MD, FACS (DOD), Walter Reed National Military Medical Center Colonel, Medical Corps, United States Army Director, John P. Murtha Cancer

Academia/Medical Institutions/Community Oncology

Ralph H. Hruban, M.D., Johns Hopkins University, Professor of Pathology & Oncology at Johns Hopkins University School of Medicine Director of The Sol Goldman Pancreatic Cancer Research Center

Vivian S. Lee, M.D., Ph.D., M.B.A., University of Utah Senior Vice President for Health Sciences Dean of the School of Medicine, CEO, University of Utah Health Care

Stephen D. Nimer, M.D., University of Miami Health System, Director, Sylvester Comprehensive Cancer Center Professor of Medicine, Biochemistry & Molecular Biology and Professor of Medicine

Mark C. Poznansky, M.D., Ph.D., Harvard Medical School, Director - Vaccine & lmmunotherapy Center, Harvard Medical School, Physician at Massachusetts General Hospital

Azra Raza, M.D., Columbia University, Director of the MOS Center, Professor of Medicine

Andrew M. Evens, DO, MSc, FACP, Tufts University School of Medicine, Professor & Chief, Division of Hematology/Oncology, Director of Tufts Cancer Center

Tom Kurtz, CEO of Windber Medical Center and Windber Research Institute

Private Foundation and Health Care Industry

Patrick Soon-Shiong, M.D., FRCS(C), FACS, NantWorks & Chan Soon-Shiong Institute of Molecular Medicine (CSSIOMM) Founder & Chairman

Paul M. Black, Allscripts, Chief Executive Officer & Director

Pharmaceutical and Biotech Companies

Paul Seligman, M.D., MPH, Amgen, Chief of R&D Policy

Patrick Vallance, GlaxoSmithKline, President, Pharmaceuticals Research & Development

Mikael Dolsten, M.D., PhD, Pfizer, President, Worldwide Research & Development

Frank Jones, M.D., PhD, Etubics Corporation, Chairman, President, Chief Executive Officer, Chief Scientific Officer

Payors and Technology

Daniel J. Hilferty, Independence Blue Cross, President & Chief Executive Officer, Chairman of the Blue Cross and Blue Shield Association (BCBSA) Board of Directors

Jim Huffman, Bank of America, Senior Vice President, Head of U.S. Health and Wellness Benefits

John Chen, Blackberry, Executive Chairman & Chief Executive Officer

Shared Vision and Support for the National Immunotherapy Coalition and Cancer Moon Shot 2020:

There was a universal agreement amongst all who attended this historic meeting that the time for

this important initiative was at hand and that with the close collaboration of this comprehensive group of stakeholders, meaningful advances could be made rapidly in cancer.

Statements in support of the Cancer Moon Shot Program are below:

Government

Robert M. Califf, M.D., U.S. Food and Drug Administration, Deputy Commissioner for Medical Products and Tobacco: At the meeting with the Vice President and Dr. Soon-Shiong, Robert M. Califf, M.D., Deputy Commissioner for Medical Products and Tobacco of U.S. Food and Drug Administration, stated, "There come moments in time where we ask why drugs tested do not work? I can remember that moment in cardiology 30 years ago where we took out things that did not work and kept things that did. We may be at that moment in time with cancer. The FDA is here to act as the 'cattle prod' to accelerate this moment when we are beginning to figure out how cancer works. The FDA is ready to be part of this historic coalition where we can provide clinical trial guidance for the combination of novel innovative agents brought into the coalition from the Cancer Moon Shot Program 2020 members," he said. "By adopting innovative and adaptive clinical trials, the QUILT coalition will shepherd in a period accelerated by innovation in cancer treatment."

Janet Woodcock, M.D., U.S. Food and Drug Administration (FDA), Director of the Center for Drug Evaluation and Research (CDER) has led many of FDA's drug initiatives. She introduced the concept of risk management in 2000 as a new approach to drug safety. Since 2002, she has led the "Pharmaceutical Quality for the 21st Century Initiative," FDA's highly successful effort to modernize drug manufacturing and its regulation. In 2004, she introduced FDA's "Critical Path" Initiative, which is designed to move medical discoveries from the laboratory to consumers more efficiently. At the National Immunotherapy Coalition meeting hosted by Vice President Biden, Dr. Janet Woodcock made insightful comments and voiced the sentiments of many participants present at the meeting in support of the goals of the National Immunotherapy Coalition and the Cancer Moon Shot 2020 program, stating, "Current clinical trials are organized around disease rather than immunology approaches. This traditional approach takes too long. You can only answer one question at a time and it may take you four years to answer one question about one drug and one tumor. It takes too long and doesn't give you enough information. We need a large network to enroll a large number of patients. Almost all cancer patients are never enrolled in clinical trials. Almost all of them are never treated with investigational therapies. With regard to combinations, FDA issued guidance a number of years ago about investigational combinations. Development of Hepatitis C drugs is an example."

"My personal opinion: I believe we are approaching the science wrong," she added, "We have great basic science, we learn all this information but we don't test it in the clinic in an efficient manner. Anything we learn from a mouse or human pathology is an hypotheses, and often the hypothesis is wrong when we test it in people. In complexities such as we face today in cancer where we have next generation sequencing, when we wish to combine chemotherapy with immunotherapy, when we wish to explore multiple combinations, you're often asking five questions, not one. We need an engine that can turn around this knowledge rapidly and a process to help answer these questions effectively."

"An important issue is our need to involve the community, that's where the patients are," Dr. Woodcock continued. "To date, trials have been centered around major medical centers and these trials are slow to accrue, since patients do not want to leave their community and doctors do not want to send them to remote centers. We need to build an infrastructure that will support these community doctors and allow them to participate and have their patients participate in these clinical trials without losing their patients and without having their patients to go away. "

Peter W. Marks, M.D., Ph.D., U.S. Food and Drug Administration, Deputy Director for the Center for Biologics Evaluation and Research (CBER): "Our goal is to enable important pathways for progress to get important therapies to patients. We will not be the impediment on the critical path at the FDA. Evidence generation needs to happen and we will work to streamline the process for vaccine development."†

Dr. Jeffrey Schlom, Chief of the Laboratory of Tumor Immunology and Biology at the National Cancer Institute (NCI), directs a translational research program in cancer immunotherapy. He has pioneered the use of novel immunotherapeutics, both as monotherapy and in combination therapies, for a range of human cancers. His studies involve the translation of hypothesis-driven preclinical studies to science-driven clinical trials. Dr. Schlom's studies involve the design and development of novel therapeutic cancer vaccines, immunocytokines, and checkpoint inhibitor

monoclonal antibodies.

His longstanding experience in the biology of cancer and the efforts needed to rapidly address immunotherapy drug development through a collaborative national immunotherapy coalition were shared with the Vice President on December 1st, stating "This coalition that has been brought together by Dr Soon-Shiong to address cancer is one of the most comprehensive sets of talents , and is quite unique. I've seen it all but I've never seen anything quite like this in my years. I've been there at the original war on cancer during the Nixon Administration and saw the bureaucracy on how it played out."

"The issue we face today in cancer care is a paradox. It is clear that immunotherapy can work in managing cancer but this approach is only working in about 20% of patients now. The problem we face today is that there are about 2 dozen drugs in immunotherapy in the clinic right now and they are all being developed in silos by each individual company. This National Immunotherapy Coalition is designed to solve this problem. I'm extremely proud to be a part of this and have never seen a situation like this where we can get these trials done so rapidly. I've been revitalized, regenerated through this whole process." he said at the historic DC meeting.

Dr. James L. Gulley who has served at the National Cancer Institute (NCI) for 18 years also participated in the meeting with the Vice President and Dr. Soon-Shiong. In his role as **Branch Chief, Dr. James L. Gulley, Director of the Medical Oncology Service, Office of the Clinical Director**. Dr. Gulley echoed the sentiments of Dr. Schlom stating, "We are seeing a sea change on how we are treating patients with cancer with immunotherapy. We are seeing deep durable and rapid responses to these new immunotherapy agents that unfortunately are only in the minority of patients and in some cancers there's no responses at all. The best way to address these failures is to explore combination therapies and this National Immunotherapy Coalition is the vehicle to break down these silos and test these combinations rapidly. That's the way we are going to make progress. I am proud to be part of this important and historic immunotherapy initiative."

Col. Craig Shriver, MD, FACS (DOD), Walter Reed National Military Medical Center Colonel, Medical Corps, United States Army Director, John P. Murtha Cancer: He provided his viewpoints as it related to the military DOD health care and cancer stating, "There are five requirements for the QUILT coalition that we are proud to be part of and they're directly related with what we do at our cancer center. We validate big science through our clinical trials network. There are 1.2 million active duty military members, 9.3 million beneficiaries that receive military DOD health care. That's a huge network. Just in our active duty force, we get a thousand active duty members a year that come down with cancer. If a thousand active duty members were still getting injured in Afghanistan or Iraq, we would not accept this. So it's the same thing with how militaries respond to infectious diseases… things that affect the readiness of our active force. So cancer is that threat. Care coordination is what we can provide since we have five other sites in our health system. We have a synergistic relationship with the NCI and that will broaden the access for these patients to these clinical trials. I am honored to serve and be part of this remarkable Coalition that Dr. Soon-Shiong has convened."

Academia/ Medical Institutions/Community Oncology

Ralph H. Hruban, M.D., Professor of Pathology & Oncology at Johns Hopkins University School of Medicine and Director of The Sol Goldman Pancreatic Cancer Research Center:"We are at a crossroads, a time of discovery that's transforming the ways we manage cancer. Johns Hopkins researchers and clinicians are working tirelessly to understand cancer better and to move treatments from bench to bedside so that patients can have a better shot at beating the disease. It is my hope that the National Immunotherapy Coalition, and others like it, will advance the understanding of cancer, not by small steps, but instead by leaps and bounds."

Vivian S. Lee, M.D., Ph.D., M.B.A., University of Utah Senior Vice President for Health Sciences, Dean of the School of Medicine, CEO, University of Utah Health Care: "There really is a no more fascinating or promising time to be in medicine. The National Immunotherapy Coalition is an amazing opportunity to discuss obstacles that may impede the successful moon shot for cancer and reach the goal of establishing an effective vaccine for this disease in 5 years instead of 20. The University of Utah is deeply committed to solving these dilemmas and I, for one, am heartened that we will help lead the way."

Stephen D. Nimer, M.D., Director of the Sylvester Comprehensive Cancer Center and a Professor of Medicine, Biochemistry & Molecular Biology at the University of Miami's Miller School of Medicine: "Every day, the physicians and scientists

within Sylvester Comprehensive Cancer Center's site disease groups and multidisciplinary research programs, are working to make exciting breakthroughs that can transform the way cancer patients are diagnosed and treated. We look forward to working for the National Immunotherapy Coalition and developing the most innovative strategies to fight the most deadly forms of cancer."

Mark C. Poznansky, M.D., Ph. D. Director – Vaccine & Immunotherapy Center, Physician - Massachusetts General Hospital, Associate Professor, Harvard Medical School: "The time is now to create an accelerated path, and advance medical science forward to save lives and improve health worldwide. The National Immunotherapy Coalition clearly unites and leverages the resources and expertise of a diverse network of medical and business professionals to safely and rigorously accelerate the pace of discovery, development and actualization of cancer treatment. By accelerating the development of new safe and cost effective therapies combating cancer, we can bring them to those that are most in need faster and more cost effectively than current approaches."

Manuel Hidalgo, M.D., Ph. D. Chief, Division of Hematology/Oncology and Clinical Cancer Center Director, Beth Israel Deaconess Medical Center, Harvard Medical School: "We are now glimpsing the potential of modulating the immune system to treat cancer in an effective way. Integrating multiple treatment strategies in innovative clinical trials protocols is the path to make a real impact in cancer care. At BIDMC we struggle on a daily basis to discover and implement new treatments for our patients. We are very excited to work with The National Immunotherapy Coalition and join efforts to advanced cancer medicine."

Azra Raza, M.D., Columbia University, Director of MDS Center, Professor of Medicine: "We are very pleased to have the opportunity to work with the National Immunotherapy Coalition and collaborate with a world-class team who share a commitment to reduce cancer incidence and to improve the quality of life of those affected by cancer. Being able to pool resources and agents, we will be able to make a significant leap in developing new immunotherapeutic and combinations that will most benefit patients with various cancer types and stages."

Andrew M. Evens, DO, MSc, FACP, Professor & Chief, Division of Hematology/Oncology, and Director of the Tufts Cancer Center at Tufts Medical Center: "Tufts Cancer Center is honored to join a group of world-renowned expert physicians, scientists and researchers who have a shared passion for fighting cancer. We understand that cancer can affect every aspect of a person's life — and the lives of their loved ones. That's why we are dedicated to research focused on helping bring new and innovative treatments to patients in less time."

Tom Kurtz, CEO of Windber Medical Center and Windber Research Institute: "The DOD and Col. Shriver have established a long standing partnership with the Windber Research Institute and through our collaborative efforts the DOD and Windber were responsible for providing over 90% of the breast cancer tissue genetically analyzed by the NIH Cancer Genome Atlas study. This human tissue repository is the nation's foremost Platinum-rated, CLIA CAP certified bio-repository for cancer tissue housing over 90,000 tissue specimens, and will be utilized as the tissue repository resource to support the QUILT Trial," said Tom Kurtz, CEO of Windber Medical Center and Windber Research Institute. "We are proud to be involved in this historic national initiative and to expand the efforts to elucidate the biologic mysteries of cancer and build on our work that has been so strongly supported by the DOD and NIH over the last decade" he said.

Private Foundation and Healthcare Industry

Patrick Soon-Shiong, M.D., FRCS(C), FACS, NantWorks & Chan Soon-Shiong Institute of Molecular Medicine (CSSIOMM) Founder & Chairman: "There are unique times in history when events and advancements in technology converge to elicit a quantum leap in medical care. This is not only a unique time, but also a unique inflection point in the history of cancer," said Patrick Soon-Shiong, M.D., Founder and Chief Executive Officer of NantWorks and the Chan Soon-Shiong Institute of Molecular Medicine. "Our knowledge in the science of genomics, proteomics, immunology and immunotherapy has advanced and converged at an unprecedented speed, making now the time for the rapid deployment and orchestration of immunotherapy for the benefit of millions of cancer patients. The Cancer Moon Shot 2020 Program, the National Immunotherapy Coalition and the QUILT Trial are designed to do just that—to bring together a diverse group of stakeholders to pool resources working together to develop and bring to patients, a dramatic improvement in cancer care."

Paul M. Black, Allscripts, Chief Executive Officer & Director: "The National Immunotherapy

Coalition is an exciting step towards a more efficient future in cancer treatment, partnering research and health information technology in an entirely new way. As a leader in healthcare information technology solutions, we are making a commitment to this initiative to ensure that our clients can combine the cutting edge research being done by Nant with the power of their Allscripts clinical information solutions to better harness the enormous volume of newly available data. When new research and genomic analysis is presented in the clinicians' workflow in a way that feels natural to them, it allows them to focus first and foremost on the well-being of all those dealing with cancer."

Pharmaceutical and Biotech Companies

Stefan Oschmann, Vice Chairman and Deputy CEO at Merck KGaA: In 2014, Merck KGaA and Pfizer formed a global strategic alliance to jointly develop immuno-oncology compounds. Both Pfizer and Merck KGaA have now joined the National Immunotherapy Coalition to accelerate their development of their Immuno-Oncology portfolio such as their PD-L1 Antibody in clinical trials. **"**Forging strong partnership between academia and healthcare industry is of strategic importance to share knowledge and more effectively address existing challenges in cancer care. We are very pleased to be part of this initiative that will help combine forces to improve patient outcomes through combining highly innovative novel- novel combinations in the field of Immune-Oncology."

Mikael Dolsten, M.D., Ph.D., President, Worldwide Research & Development, Pfizer Inc. Dr. Mikael Dolsten attended the meeting in Washington DC in support of the goals of the National Immunotherapy Coalition and the Cancer Moon Shot 2020 program. He stated, "The challenge of cancer is far too great for any of us to tackle alone. Pfizer welcomes the collaboration of NCI and FDA in the creation of clinical master protocols that allow for the effective testing of novel oncology drug combinations from several companies or institutions. It is our hope that the joining together of the health innovation ecosystem under the National Immunotherapy Coalition will further accelerate the development of game changing, combination immunotherapies for the benefit of cancer patients."

Patrick Vallance, GlaxoSmithKline, President, Pharmaceuticals Research & Development who went from leading an academic department at University College London to his current role heading up our R&D operations at GlaxoSmithKline (GSK). He has refocused GlaxoSmithKline's oncology efforts to immunotherapy stated at the Washington DC meeting, "Is it possible to change cancer care to what we are doing with Malaria research? Everyone believes combinations are essential. No one should believe that a single molecule would do the trick. The problem is not about making the medicine; we have a lot of medicines, its knowing how to combine them and knowing what works in patients. Those combinations aren't going to work out sequentially by individual companies. It's going to take 30-40 years going at it alone. Its imperative we collect the data from patients already getting these today. We need to work electronically to gather these data points and assemble this data together in this National Immunotherapy Coalition."

Robert J. Hugin, Chairman and Chief Executive Officer, Celgene Corporation: Celgene has made a significant commitment to the field of immunotherapies and is a leader in the field of IMiDs, a novel class of immunomodulators, treating diseases such as multiple myeloma and Myelodysplastic syndrome. At a Pancreatic Cancer advocacy event, Robert J. Hugin, Chairman and CEO of Celgene Corporation stated, "We, too, are fully committed to your Vision of Progress goal to double survival for pancreatic cancer by 2020. When we took on pancreatic cancer and the development of the Abraxane® compound that eventually won FDA approval, we knew it was a bold and ambitious challenge. Conducting a trial that would show significant survival benefit in advanced pancreatic cancer had only been accomplished three times in the last 23 years. But we live and breathe a culture of innovation and determination. We don't back down in the face of tough odds. We know that research and the development of more effective treatments for patients call for novel approaches, bold science and a strong vision. We need more feet in the fight. We need more companies to step in and join." Celgene has made a commitment to combine their library of important molecules, both approved and in the development pipeline to participate in the QUILT Trials which will play an important role in the rapid advancement of immuno-oncology for patients with life-threatening cancer.

Paul Seligman, M.D., MPH, Amgen, Chief of R&D Policy: At the gathering in Washington DC on December 1, 2015, Paul Seligman stated, "At AMGEN, we've been intimately involved to translate genetics into biology and then biology into therapies. This is precisely the type of initiative we need to

have. We can't go it alone anymore. Science needs to work together. I worked at the FDA before working at AMGEN. This is not just a regulatory question, but how we work together as a community in developing products and developing a stable generation of research. It requires a network that's sustainable over a long period of time. This is a perfect example of public private partnerships in creating sustaining networks. I can certainly speak for AMGEN, finding the right ways to share our materials and use them in the best way possible is something we are profoundly dedicated in doing."

Frank R. Jones, PhD, Chairman, President, and Chief Executive Officer of Etubics Corporation: "At Etubics, we specialize in developing innovative immunotherapies and vaccines for a wide-range of resilient diseases including cancer, so it goes without saying that we are extremely excited about this new initiative. We recognize the value in an immune stimulation treatment approach and look forward to volunteering our agents for combination clinical trials that we anticipate will produce groundbreaking results.

Payor and Technology Sectors

Daniel J. Hilferty, Independence Blue Cross, President and Chief Executive Officer: "Every day, Independence Blue Cross is reimagining health care in hopes to help our members succeed on their path to improved health and high quality care," said Daniel J. Hilferty, Independence Blue Cross, President and Chief Executive Officer and Chairman of the Blue Cross and Blue Shield Association Board of Directors (BCBSA), comprised of 36 Independent Blue Cross Blue Shield companies that together cover nearly 105 million individuals – 1 in 3 Americans. The National Immunotherapy Coalition is allowing Independence to further enhance our contribution to the future of health care through our active involvement in this one-of-a-kind collaboration." Hilferty stated. "We look forward to continuing our work with this incredible team to develop the most innovative cancer fighting strategy in our lifetime. Through our Blue Cross Blue Shield Association we cover 1 in 3 Americans and insure individuals across every zip code in the country – this initiative is important to all Americans."

John Chen, Blackberry CEO: "At Blackberry, we understand the value that lies at the intersection of healthcare and technology, which is why we are constantly making advancements to reflect the ever-changing healthcare landscape. As we already power many of the tools that clinicians rely on heavily, we are confident that our involvement in the National Immunotherapy Coalition will be an asset to the future of Cancer treatment. This unique collaboration is pioneering extraordinary solutions to cancer care and we are truly honored to be a part of it."

Jim Huffman, Senior Vice President, Head of US Health and Wellness Benefits, Bank of America said, "We provide coverage for about 500k employees and their families. IT gives us a unique perspective of what we are paying for, which is $2 billion dollars. It's about are we getting the best product to our employees and their families?"

Conversation with The Cancer Letter

Soon-Shiong Says FDA, NCI Onboard with His Moonshot; Feds Deny Involvement

(Continued from page 1)

Soon-Shiong said he and Vice President Joe Biden met to discuss their interlocking missions and are now pursuing them.

"I made it very clear upfront—what we will do and I will do is I will be the cattle prod from the philanthropy perspective, and I will be the cattle prod from the private sector in getting the pharma and biotech to work together," Soon-Shiong said. "What the vice president can do from his perspective is look at the inefficiencies inside the government from any of the agencies, but I've got to tell you, what was incredibly exciting is there wasn't any roadblock from the regulatory agencies. I mean, the FDA was amazingly supportive—Bob Califf [nominated to be the next FDA commissioner] and Janet Woodcock [director of the FDA Center for Drug Evaluation and Research] and Peter Mark [deputy director for the FDA Center for Biologics Evaluation and Research] were incredibly supportive."

Soon-Shiong said his interaction with NCI draws on the institute's expertise through a Cooperative Research and Development Agreement. NCI officials say the existence of the CRADA cannot be construed as an endorsement of Soon-Shiong's Cancer MoonShot 2020 program.

Matthew Ong: *What led you to convene this coalition and put together your "Cancer MoonShot 2020" program?*

Patrick Soon-Shiong: This is an evolution, and it started, obviously, way back, as I started developing

Abraxane. And by 2005, as I got the drug approved, I began to recognize that the immune system is what we needed to protect.

And so I was pushing on the concept of low-dose chemotherapy as early as 2005. I trained under Donald Morton [founder of the John Wayne Cancer Clinic] at UCLA as an oncologist-surgeon, and he was one of the forefathers of immunotherapies, because he was working with melanoma tissue and injecting it with cancer vaccines.

So when CancerVax [the company Morton founded] unfortunately failed, Don turned to me, and I continue to help support it, because I really believed in it. It failed only because Don unfortunately had designed a trial incorrectly—he had BCG as a control arm, which is another immunotherapy, and without realizing it, he was comparing immunotherapy to immunotherapy.

Nonetheless, it was very clear to me that we need to protect the immune system and walk away from maximum tolerated dose chemotherapy, and as you know, writing through The Cancer Letter, our protocols haven't changed at all, frankly, all these years in chemotherapy. That was my level of frustration.

Because I had the power of the pulpit as a CEO of a company that had a drug called Abraxane, I used that, and in 2008 or 2009, raised $1 billion to run maybe 50 to 60 multiple phase I/II trials simultaneously, or with this molecule, but with combination chemotherapies. But to figure out which are the combinations that are best to get the maximum activity but low toxicity and highest immune activity—in 1990, I wrote an obscure paper in which we demonstrated a cell called a natural killer cell required cell-to-cell contact and could kill tumor cells.

So I've been obsessed with the idea that, since our own immune system protects our cells against cancer, then why are we actually depleting that? So I began to write papers and file patents on metronomic dosing. Still, even to this day, it's not had very much traction, but the concept here was to use metronomic chemotherapy to immuno-modulate and activate the immune system rather than maximum tolerated dose chemotherapy, which may get a rapid reduction in tumor: you win the battle, but you lose the war, because you wiped out the immune system.

When I sold the two companies, I decided that I'm just going to take this on myself, and when I sold that, I thought, "Oh my gosh, the obstacles!" So you sort of see the obstacles—the first thing is that the pharmaceutical companies are building all these different drugs in silos, and you need these coordinated combinations. I needed to find a way to get these pharmaceutical companies to work together.

And, fortunately, because I was the CEO of a pharmaceutical company, I knew all the CEOs at the CEO-to-CEO level, and as some of the even younger members became more senior and ultimately because CEOs, our relationships grew through the 15 years of just experience of knowing each other. And I took it upon myself to get the CEOs together many times and kept on harping, "We've got to work together and collaborate."

Then I turned around and found that, "Oh my gosh, most of the trials are done in the community, as opposed to the academic centers." Yet the academic centers set the protocol, but the trials are accrued in the community. So if we wanted to change the clinical trial development where changing more than 5 percent of trials being accrued, we need to actually involve the community.

But we need the support of the academic centers and organizations like the NCI to give the guidance. So now I need to bring another two sort of parties who don't normally work together—the community oncologists and the academic oncologists. We were able to bring that together.

I then realized with the heterogeneity, we need to actually go to whole genome sequencing, but more than that, all the way down to the proteome level, so that we can actually understand what targeted therapy is and also understand the immunome. So we need to build that, and I built the CLIA-certified whole-genome sequencing. The trouble is, the community oncologists and academic oncologists would not use this, because nobody's paying for it.

So we subsidized it for many years. And just this last week, Independence Blue Cross said, "We will step up and we will pay for it." So all the dominoes fell together around 2014. And then, in May of 2015, I came into contact with Vice President Joe Biden, because of his son Beau Biden, and then in October 2015, Vice President Biden asked me to come see him, and the White House and I wrote this two-page white paper on the moonshot.

He was intimately and personally involved in the care of his son, and he saw firsthand the struggle of what the patient goes through with cancer. By November, after he made the decision not to run [in the 2016 presidential race], he came quietly to Los Angeles and spent three hours on our campus. And then I gave him the second white paper, and I said to him,

"Look, we can convene all the right people to make it happen," because he said to me, "Patrick, what can do to make this move faster?"

So the vice president and I have common goals here to accelerate the development of drugs so we can actually get to the path of the cure as fast as we can. I said, "Well, the issues are, we should get all these disparate parties in I call the 'complex workflow of cancer.'" On one hand, we can get pharma together to work with biotech and bring them to the table, but we need pharma and biotech all to combine the drugs and get comfort from the FDA that it's okay to do novel combinations and get them to the table.

Then we need to state to the academics and to the community that you really should define what you're going to do to the patients through quantifiable genomic and proteomic sequencing that they're going to say who's going to be paying for it. So I brought Bank of America and Independence Blue Cross to the table.

And then I brought all the clinical leadership of academic centers and the directors of the branches at NCI—Jeffrey Schlom [chief of the NCI Laboratory of Tumor Immunology and Biology] and James Gulley [chief of the NCI Genitourinary Malignancies Branch, and director of the NCI Medical Oncology Service at the Office of the Clinical Director]—who direct work in this field of immunotherapy, and then Bob Califf and Janet Woodcock.

The goal was, at the end of that meeting, to find a common consensus that we should all work together and actually bring to the table our strengths of areas of responsibility that we could be making impact on and lead for the common goal of enhancing and making more efficient clinical trials by doing combination therapy, and by validating it with genomic sequencing and also validating with Big Data.

So that's the plan, and we called that Cancer MoonShot 2020.

MO: *There is also a lot of interest in Vice President Biden's moonshot. You mentioned working with the vice president—what is the connection between your program and Biden's moonshot?*

PSS: I think Vice President Biden likely is doing his due diligence. The moonshot that we proposed to him was the moonshot that I proposed to him on Oct. 6 and again on Nov. 16 and Dec. 1. I think he's written very clearly what his views are, and his views are aligned with our views to accelerate the efforts of the path to the cure.

I think it's a good thing for the country that President Barack Obama put him in charge of what he called "mission control" of the moonshot, because he is exactly the right person, I've always said, because he can make things happen across the aisle, and he's completely committed and passionate and is a fantastic human being. I got to know him from May to December in a very difficult time, and he is one of the most sincere people who really cared.

MO: *Do you expect a partnership between your program and Biden's moonshot or for the two initiatives to work together in the coming year?*

PSS: The program that we are working is really for the clinical acceleration of drugs. I think he's working through all the elements. As far as I understand, his statement is to accelerate the efforts to improve progress and to unleash breakthrough and do immunotherapy, genomics and combination therapies, because these are stuck in silos, and to improve clinical trials in the community.

That's the program that's needed, right, and if you look at the program Dec. 1, we are completely aligned in that concept.

MO: *So this is the part where I'm completely new to this story, so you have to forgive my ignorance with the next few questions about your partnerships. When I read your official press release—*

PSS: When you said partnerships, before you go any further, one thing we did make clear to the vice president, is we are not asking for any funding. We are truly—and I made it very clear upfront—what we will do and I will do is I will be the cattle prod from the philanthropy perspective, and I will be the cattle prod from the private sector in getting the pharma and biotech to work together.

What the vice president can do from his perspective is look at the inefficiencies inside the government from any of the agencies, but I've got to tell you, what was incredibly exciting is there wasn't any roadblock from the regulatory agencies. I mean, the FDA was amazingly supportive—Bob Califf and Janet Woodcock and Peter Mark were incredibly supportive.

You should feel free to call directly Jeffrey Schlom and James Gulley, and maybe you should call Janet Woodcock. She made some very passionate statements that we need to increase the efficiency of clinical trials.

So I was very pleased that what the vice president

can do is really convene a call to action. I can't speak for the vice president what his plans are, all I can tell you is he's the perfect person for this initiative—or whatever moonshot you want to call it, there's nothing to the name—but more importantly, we're both aligned in improving the chance of getting a race to the cure going.

MO: *When I read your official press release, your moonshot looked like something that NIH, FDA and other federal entities would be highly interested in.*

PSS: Just be careful, because it's not an NCI initiative, nor is it an FDA initiative. It's a public-private initiative, but in enlisting the insight of the NCI of the science through the CRADA (Cooperative Research and Development Agreement) or whatever participation, and in enlisting the support of the FDA so that it's clear that it's within the new guidance of combinations.

So if you called the NCI and said, "Is this an NCI initiative?" they would say no, and they would be correct, because it's non-NCI. This is not an NCI-driven initiative. Having said that, the directors at the immunotherapy division holistically and enthusiastically—I want them to have a role in the first-in-human trials of combination, so that there's completely no bias. It should be scientifically driven.

MO: *Right, so I did make some phone calls, and to my surprise, I was told by Dr. Francis Collins, as well as insiders at FDA and the White House that they are not involved in your program, in contrast to what was apparently stated in the initial draft of your press release.*

PSS: Well, I can only tell you the fact that Jeffrey Schlom is actually spending three days with me right now designing clinical trials this weekend. You're welcome to call him and James Gulley. I just got off the phone 10 minutes ago saying how he's flying to Miami this weekend to talk to one pharma company, and I told him I spoke to the CEO of another pharma company and we're talking about an anti-PDL1 that he's designed. So all I can tell you is what we are doing.

Dr. Francis Collins is not FDA nor NCI, right? He's the head of the NIH, and I don't know if you want to mix this up with the Precision Medicine Initiative. If Dr. Francis Collins said he had nothing to do with it, I think he's correct.

But I think, look, what happened was, you know, Robert Califf was at the meeting. Maybe what you should do is call some of these doctors, like Dr. Azra Raza [director of the MDS Center at Columbia University], Mark Poznansky [a laboratory director of the Vaccine & Immunotherapy Center at Massachusetts General Hospital], Stephen Nimer [director of the Sylvester Comprehensive Cancer Center], Ralph Hruban [director of the The Sol Goldman Pancreatic Cancer Research Center], Andrew Evens [director of the Tufts Cancer Center at Tufts Medical Center], and ask them—coming from me—what did Robert Califf say, what did Janet Woodcock say, what did Peter Marks say, what did Jeffrey Schlom say, what did James Gulley say.

I mean, you kind of have to speak with the people at the meeting, right? I think it is unfair, frankly, to ask a member of the government who wasn't at the meeting. This is very complex.

MO: *Since NCI is a part of NIH, I was going through the regular channel of press offices to obtain statements for the public side of your program's public-private partnership. Do you think there was a misunderstanding initially? It appears as if many parties were not aware that you have included them in your announcement of the coalition.*

PSS: No, wait, let me just set the facts straight. Every member of the FDA or NCI—they actually sent me their quotes. So I'm not sure how could they… okay?

But I mean, look, we should get beyond that, the issue really is, this is an important program. What's more important is, rather than any of these—I don't know what these issues are, politics, whatever you may want to call it—is, what is important for the patient, right?

And I think in the collaboration, we should have an aligned mission, it's not this program or that program. We all are aligned in the mission to look for the cure, that's all I can say, and whoever can get there, I'll be on their bandwagon, and because I'm there to support whoever can actually bring us to the cure.

This is one of the most inclusive collaborations. The whole idea here is about collaboration.

MO: *It appears that you've received positive responses from pharma, but I've read that Pfizer, Merck and GlaxoSmithKline have—much as the federal agencies have done—pulled out from formal association with your coalition. Are there any other pharmaceutical companies who have done the same?*

And what do you make of their withdrawal?

PSS: Let me tell you that this is the launch. So Amgen, Celgene are absolutely on board. Glaxo initially said they were on board, and so did Pfizer, and when they got together, their legal team said they need to find more information from the CRADA, they need to get more data from the NCI and they didn't have time. I said that's fine, and then Merck is part of the Pfizer joint venture, and Merck said they have to do what Pfizer had to say, and so there wasn't time for me to meet the CEO of Glaxo and Pfizer, so that's fine.

Since the announcement, I've met the CEO of Merck and she said I can use her name and she said she's enthusiastic—I just had a conversation about the antibody, it was Pfizer's antibody and the NCI's meeting them in a couple of weeks.

So these are just processes in motion, we've had four other biotech companies join even while we were here. The number of molecules has now gone from 60 to maybe 70, so I think it's an exciting process, where we bring molecules and make drugs accessible to patients.

MO: *What is the budget for the program, and what are your next steps? What will the milestones be and when will you expect results?*

PSS: The budget of this will be billions, I anticipate. Let me give you the design—this is very important, this is The Cancer Letter, they need to understand the design and the people who read this are oncologists.

The importance of this is there's going to be what we call a Discovery Phase, where patients who have active cancer and are clinical patients will undergo, for the first time, whole genome sequencing, RNA-seq proteomic analyses and quantitative proteomics. So we've evolved the platform from genome to the proteome. That's point one.

Point two, we've taken all comers and that allows us to then create a separate protocol called a stratification protocol, where we will stratify the patients to the anatomical tumor types. We've arbitrarily said 22 metages, obviously we can as many—brain, breast, lung, bladder, melanoma, liquid tumor cells, etc.—so that the oncologists who are trained to the anatomical tumor types could then see patients along those paths.

We then stratify that across all the stages, whether it be new adjuvants, adjuvant first line, second line. Once we've done the stratification phase, we then drop them into randomized phase II buckets. The randomized phase II buckets, on the one hand, are standard of care, and the other one is standard of care plus and minus the immunotherapy and novel combinations.

What that does for us is it allows the standard of care to be paid for by the insurance companies, and it allows the treatment arm to be where the pharma companies provide their drug. Now for the first time, you'll have data across thousands of patients, across multiple phase II trials, but randomized, for which the data of the genomic sequencing will be available, so that when we measure the outcomes, we now have data that is useful and can be then driven into either randomized phase III trials or expanded phase II trials for registration.

So nobody owns any proprietary advantage. What everybody has gained is efficiency in trial design; doctors have gained access to drugs for their patients who are from a complex GPS test; community oncologists now for the first time don't really have to lose their patients, and we accelerate, I believe, immunotherapy.

The budget for that may cost billions, because you may have 10, 50 or 100 or 200 randomized phase II trials over the course of the next four or five years, but the cost of that will be borne either by the insurance company, pharmaceutical company, the drugs themselves—and yes, if the government wants to support through grants and clinical trials—and most importantly, also philanthropy.

The entire financial method of payment hasn't changed other than we've created an efficient framework.

MO: *Do you have any further comments? Did I miss anything?*

PSS: I'm excited that we've moved, and that we're already designing three or four or five [clinical trials] and this weekend is going to be very exciting where I will be working with investigators at the NCI in drafting the clinical trial design.

Step one in the moonshot will be establishing a global advisory group, which we are doing in real time, and we go into January. Step two is to publicly announce it, which we have just done. Step three was to establish a common web-based secure portal that Independence Blue Cross has provided us through NaviNet. Step four is to amend currently approved vaccine-based trials, which we are doing as we speak this weekend.

So we are very much on the path, very much a methodical approach, there's no mystery to it I think.

USPSTF Recommends Biennial Mammography Screening For Women Ages 50-74

The U.S. Preventive Services Task Force published its final recommendation statement on screening for breast cancer, delivering a B rating for mammography screening every two years in women between ages 50 and 74.

The task force also recommended selectively offering mammography to women below age 50, saying that the decision to begin that screening should be an individual one. The USPSTF gave this age group a C recommendation.

"Women who place a higher value on the potential benefit than the potential harms may choose to begin biennial screening between the ages of 40 and 49 years," the task force wrote in its recommendations.

"For women who are at average risk for breast cancer, most of the benefit of mammography results from biennial screening during ages 50 to 74 years. Of all of the age groups, women aged 60 to 69 years are most likely to avoid breast cancer death through mammography screening.

"While screening mammography in women aged 40 to 49 years may reduce the risk for breast cancer death, the number of deaths averted is smaller than that in older women and the number of false-positive results and unnecessary biopsies is larger. The balance of benefits and harms is likely to improve as women move from their early to late 40s."

The task force also issued three "I statements," saying that evidence was insufficient to recommend screening mammography in women over the age of 75; to assess digital breast tomosynthesis as a primary screening method for breast cancer; and to assess the balance of benefits and harms of adjunctive screening for breast cancer using breast ultrasonography, magnetic resonance imaging, DBT, or other methods in women identified to have dense breasts on an otherwise negative screening mammogram.

The B and C recommendations apply to "asymptomatic women aged 40 years or older who do not have preexisting breast cancer or a previously diagnosed high-risk breast lesion and who are not at high risk for breast cancer because of a known underlying genetic mutation (such as a BRCA1 or BRCA2 gene mutation or other familial breast cancer syndrome) or a history of chest radiation at a young age," the task force wrote.

The Patient Protection and Affordable Care Act mandates that private health insurers cover clinical preventative care procedures that receive A and B recommendations from the USPSTF, where the task force has found moderate or substantial net benefits. C recommendations, with a small net benefit, are neither included or excluded from insurance coverage.

"In the linkage to coverage established by the Patient Protection and Affordable Care Act, the USPSTF's role is limited to evaluating the science to determine the net benefit of a clinical preventive service," wrote representatives of the task force in an accompanying editorial. "Our review of the scientific evidence may be only one of the inputs to determining insurance coverage; often it is the floor to determining minimal coverage, not the ceiling. Coverage decisions are the domain of payers, regulators, and legislators.

"Whatever we may believe about the importance of coverage in shared decision making about mammography, we cannot exaggerate our interpretation of the science to ensure coverage for a service. This would lead to confusion regarding the state of science versus the politics of coverage."

The USPSTF's final recommendation statement and editorial were published online in the Annals of Internal Medicine.

The American College of Radiology and the Society of Breast Imaging said that women should continue to begin annual mammography screening at age 40, and that, if followed, the task force's recommendations would lead to "thousands of unnecessary deaths each year and thousands more women enduring extensive and expensive treatment than if their cancer had been found early by an annual mammogram," the groups said in a statement.

"A recent study in the British Medical Journal confirms that early detection of breast cancer via mammography is critical for improving breast cancer survival, regardless of therapy advances. Moving away from yearly screening in women

40-and-older endangers women, would cause needless death and disfigurement of women, and would simply not be good breast cancer screening policy," said Debra Monticciolo, chair of the American College of Radiology Breast Imaging Commission.

Congress, in its appropriations bill passed last month, included provisions to delay the implementation of USPSTF's breast cancer screening recommendations for at least two years, a move that was previously part of the Protecting Access to Lifesaving Screenings Act.

"Women should have the opportunity to make informed screening choices and have insurance coverage for those decisions. The USPSTF's recommendations may still jeopardize women's access to these lifesaving exams. We are grateful for Congress's foresight to include legislative language from the PALS Act in the Consolidated Appropriations Act. Clearly, they agree that women ages 40-49, 50-74, and 75-and-older who want annual mammograms, should be covered for, and have access to, these lifesaving exams. Women and their families should continue to make sure that their lawmakers safeguard that access," said Elizabeth Morris, president of the Society of Breast Imaging.

The American Cancer Society said that its breast screening guideline and the USPSTF statement include similar recommendations, but there are a few areas of important differences, including the age by which all women should have started screening, the frequency of screening mammography, and at what age screening should stop.

"The updated USPSTF recommendation has once again confirmed the value of screening mammography, concluding that the benefit of mammography outweighs the harms of screening in all age groups from age 40 through ages 74. It emphasizes that women and clinicians providing primary care to women both need to understand the benefits and harms of screening," the society said in a statement.

"Compared to the initial draft recommendation, the USPSTF now places greater emphasis on the importance of making a personal, informed decision about when to start screening. The new language adds greater clarity regarding the higher risk of developing breast cancer in the late 40's compared to the early 40's and endorses a woman starting to screen any time in that decade if she believes screening is right for her."

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Campaign for Tobacco-Free Kids Demands Recall of Gold Standard Accreditation of U.S. Chamber of Commerce

The Campaign for Tobacco-Free Kids said the U.S. Chamber of Commerce is undeserving of its Gold Standard accreditation by the CEO Roundtable on Cancer, saying it should be rescinded because of the trade group's lobbying efforts against tobacco regulations.

"It is incomprehensible that the U.S. Chamber of Commerce has been recognized by the CEO Roundtable on Cancer for its efforts against cancer when the Chamber has helped the tobacco industry fight life-saving and cancer-preventing tobacco control policies around the world, as recently revealed by a multi-part investigation by The New York Times," said Matthew Myers, president of the Campaign for Tobacco-Free Kids. "Far from helping to reduce cancer, the Chamber's pro-tobacco activities are undermining global efforts to fight cancer."

According to reports from the Times last year, the U.S. Chamber of Commerce has been working internationally against antismoking laws, such as labeling requirements for cigarette packaging and taxes on tobacco products.

CVS Health, which chose to forego selling tobacco products at all its pharmacies and retail stores in 2014, decided to leave the Chamber of Commerce in July 2015, following the Times report.

"CVS Health's purpose is to help people on their path to better health, and we fundamentally believe tobacco use is in direct conflict with this purpose," said a CVS spokesperson at the time.

The CEO Roundtable on Cancer is a nonprofit organization that administers the Gold Standard workplace accreditation program, which encourages and recognizes the commitments of employers to reduce the risk of cancer in their employees.

The chamber was recognized with the accreditation in December 2015. To earn Gold Standard accreditation, an employer must establish programs to reduce cancer risk in five pillars: "establishing policies and programs to reduce cancer risk by prohibiting tobacco use and supporting tobacco cessation efforts; promoting physical activity, healthy nutrition and weight management; providing health insurance options that include detecting cancer at its earliest stages, access to quality care and participation in cancer

clinical trials; promoting employee awareness of these initiatives; and supporting the needs of cancer survivors in the workplace," the CEO Roundtable said in a press release announcing the chamber's certification.

Nearly 200 private, nonprofit and government employers in a wide range of occupational categories have earned the Gold Standard accreditation, according to the CEO Roundtable, including the NCI and a number of NCI-designated cancer centers.

John Dornan, executive director of the CEO Roundtable, said that the group did not have plans to re-examine the chamber's accreditation. "The Gold Standard is based on employers' efforts to improve the health and wellness of their employees," Dornan said, adding that the roundtable's goal is to affect public health by recognizing these efforts and encouraging others to do the same.

In Brief

Leonard Zon Receives NCI Knudson Genetics Award

LEONARD ZON received the 20th annual Alfred G. Knudson Award in Cancer Genetics from the **NCI.**

Zon is director of the Stem Cell Program at Boston Children's Hospital and the Grousbeck Professor of Pediatrics at Harvard Medical School,

The award, named for the physician and geneticist who helped revolutionize the understanding of the genetic basis of cancer, is presented annually to a scientist who has made outstanding research contributions to the field of cancer genetics. Zon delivered his award lecture, "Modulating Cell Fate for Cancer Therapeutics: Insights from Embryonic Development," at the annual NCI Intramural Scientific Investigators Retreat in Maryland.

In addition to his positions at Boston Children's Hospital and Harvard Medical School, Zon is a Howard Hughes Medical Institute investigator, professor of stem cell and regenerative biology at Harvard University, and an associate member of the Broad Institute of Harvard and MIT.

He pioneered the use of the zebrafish for the study of human blood formation and disease, including many types of cancer. Zon's translational zebrafish research led to the discovery and development of two novel therapeutics that are now being evaluated in clinical trials of patients with leukemia and melanoma. His research is largely focused on better understanding the genetics of blood diseases, melanoma, and other cancers.

Zon founded and is past president of the International Society of Stem Cell Research and former chairman and a member of the executive committee of the Harvard Stem Cell Institute. He is also a member of the Institute of Medicine of the National Academies and the American Academy of Arts and Sciences, and former president of the American Society for Clinical Investigation.

Zon has previously received the E. Donnall Thomas Prize from the American Society of Hematology (2010) and the International Society for Experimental Hematology Donald Metcalf Lecture Award (2013).

AMY MCKEE was appointed acting deputy office director in the **FDA Office of Hematology Oncology Products.**

As deputy office director, McKee will continue to develop and support OHOP strategic policy initiatives and research efforts. She assumed this position effective Dec. 27, 2015.

McKee began working at FDA in 2008 and has served as a medical officer and clinical team leader for breast and gynecologic products within the Division of Oncology Products 1. Her work has included outreach to the ovarian cancer community, including a public workshop with stakeholders to advance drug development in this disease.

Her interest in early-phase drug development and improved dose-finding has led to cross-disciplinary initiatives within FDA to develop new methodologies for early dose-finding trials and dose exploration throughout drug development as well as discussion with external stakeholders to identify the best practices within industry.

McKee completed her pediatric hematology/oncology fellowship at the combined Johns Hopkins/NCI program, where she received the 2008 NCI Director's Innovation Award for her basic research in neuroblastoma.

PAUL KLUETZ was appointed associate director of clinical science in the **FDA Office of Hematology Oncology Products**, effective Jan. 24.

As associate director, Kluetz will continue to develop and support OHOP strategic policy initiatives and serve as the lead for patient-focused drug development and clinical outcome assessments.

Kluetz began working at FDA in 2010, and

has served as a medical officer in the genitourinary oncology team within the Division of Oncology Products-1.

In addition to extensive outreach to the GU oncology community as a scientific liaison, he has been involved in multiple scientific and regulatory policy initiatives associated with FDA expedited programs, oncology efficacy endpoints as well as cross-center initiatives to identify ways to encourage and improve patient-reported outcomes and other clinical outcome assessments in cancer trials. Kluetz completed his medical oncology fellowship at NCI, where he was active in clinical research in metastatic prostate cancer.

RAVI SALGIA joined **City of Hope** as a professor and chair of the Department of Medical Oncology & Therapeutics Research.

In addition to his role as the Arthur & Rosalie Kaplan Endowed Chair in Medical Oncology, he will also serve as associate director for clinical sciences in the institution's comprehensive cancer center. He will play a leadership role in the expansion of clinical programs at City of Hope's Duarte campus and its clinics throughout Southern California.

Previously, Salgia served at the University of Chicago Medical Center and Pritzker School of Medicine, where he was a professor of medicine, pathology and dermatology, director of the Thoracic Oncology Program, vice chair of medicine, and associate director of translational sciences for the comprehensive cancer center.

During his 12 years with the University of Chicago, Salgia identified several novel targets in oncology and led a strong clinical and research group. His laboratory conducted research on how the receptor tyrosine kinases. Salgia's most recent work has focused on the development of biomarkers for early diagnosis, as well as prognosis and therapeutic monitoring of thoracic cancers, which occur in the lung and chest area.

DEAN TSARWHAS was named medical director of cancer services for Northwestern Medicine's Lake Forest Hospital and Grayslake Outpatient Center. Previously, Tsarwhas treated oncology patients in private practice. He has also been on staff at Lake Forest Hospital.

"Dr. Tsarwhas is an outstanding oncologist who shares our commitment to utilizing a strong multidisciplinary team approach to provide the best cancer care possible," said Leonidas Platanias, director of the Robert H. Lurie Comprehensive Cancer Center of Northwestern University.

"I look forward to working closely with him to find new ways of providing patients in the northern suburbs with more streamlined access to novel clinical trials and therapies through the Lurie Cancer Center and the new Northwestern Medicine Developmental Therapeutics Institute clinic at Lake Forest Hospital."

RAFAT ABONOUR was appointed medical liaison for the **International Myeloma Foundation**, to support the group's European program expansion.

Abonour established and currently directs the multiple myeloma and plasma cell program at Indiana University Simon Cancer Center. His selection follows the foundation's recent appointment of French myeloma researcher Jean-Luc Harousseau.

THE FEDERATION OF AMERICAN SOCIETIES FOR EXPERIMENTAL BIOLOGY published a set of recommendations aimed to promote the reproducibility and transparency of biomedical and biological research.

The recommendations address general factors that impede the ability to reproduce experimental results as well as factors that specifically affect the use of two key tools critical to basic research: mouse models and antibodies. The report suggests actions for stakeholders across the research enterprise, including scientists, institutions, professional societies, journals, and federal agencies.

"What began as a thought-provoking discussion of some very real challenges facing our community resulted in practical recommendations to help scientists begin to move the needle in their own labs and institutions," said FASEB President, Parker Antin. "We cannot take for granted the public's trust in science. It is time to enact policies and procedures that emphasize the tradition of rigor in research."

THE COMMUNITY ONCOLOGY ALLIANCE elected members to its board of directors and officers to its executive committee.

"We anticipate significant progress on payment reform initiatives such as the Oncology Medical Home and ongoing examinations of the 340B drug program and more," said Bruce Gould, COA president and practicing oncologist at Northwest Georgia Oncology Centers in Marietta, Ga. "COA is strengthened by the very active involvement of its volunteer board and executive team. We welcome the new and continuing board members. Their participation ensures that COA

will continue in its strivings for the betterment of community cancer care."

COA's executive committee reports to the board, which is comprised of volunteer representatives from community oncology, who direct the management of COA by its executive director.

All director positions are three-year terms, ending Dec. 31, 2018. Four individuals were elected to serve a first-time term on the COA Board of Directors: **Bruce Burns; Jose DeVilla; Deborah Patt;** and **Jeffrey Patton.**

The following were re-elected to serve on the board: **Henry "Mac" Barnes; Marsha DeVita; Michael Diaz; David Eagle; Robert Green; Dinesh Kapur; Joseph Lynch; Barbara McAneny; Carol Murtaugh;** and **Mark Thompson.**

Two current board members whose terms expire in 2015—**Abraham Mittleman** and **Hamidreza Sanatinia**—will rotate off of the board.

The following individuals were elected to serve one-year terms as officers of COA and on the executive committee: COA President **Bruce Gould;** Vice President **Jeff Vacirca;** Secretary **Mike Diaz;** Ex Officio Treasurer **Ricky Newton;** Immediate Past President **Mark Thompson;** Past President **Dave Eagle;** Past President **Henry "Mac" Barnes;** Officer-at-Large **Miriam Atkins;** and Ex Officio Executive Director **Ted Okon.**

All officer position terms end Dec. 31. As full-time employees of COA, Okon and Newton serve as non-voting members on the executive committee. The complete list of Officers and Board members can be viewed on the COA website.

Drugs and Targets

Blincyto Receives Conditional Approval from Health Canada

Health Canada granted conditional approval of Blincyto (blinatumomab) for the treatment of adults with Philadelphia chromosome-negative relapsed or refractory B precursor acute lymphoblastic leukemia.

With this approval, Blincyto becomes the first Health Canada-approved bispecific CD19-directed CD3 T-cell engager antibody construct product, and the first single-agent immunotherapy to be approved for the treatment of patients with Ph- relapsed or refractory B precursor ALL.

The conditional approval is received under Health Canada's Notice of Compliance with conditions policy which allows for earlier marketing of promising drugs for serious conditions before the drugs have definitively demonstrated clinical efficacy. Amgen Canada Inc., the application's sponsor, has made commitments to complete confirmatory trials to convert the approval to a full notice of compliance.

Bispecific T cell engager, or BiTE, antibody constructs are being investigated for helping the body's immune system detect and target malignant cells. The modified antibodies are designed to engage two different targets simultaneously, thereby juxtaposing T cells to cancer cells.

FDA granted Priority Review to venetoclax for the treatment of chronic lymphocytic leukemia in adults who have received at least one prior therapy, including patients with 17p deletion.

With priority review, the FDA's goals include a faster timeline for review of six months, compared to 10 months for the standard review period.

Additionally, the European Medicines Agency validated venetoclax Marketing Authorization Application for the treatment of patients with chronic lymphocytic leukemia with 17p deletion or TP53 mutation.

Venetoclax, sponsored by AbbVie, is an inhibitor of the B-cell lymphoma-2 protein being developed in partnership with Genentech and Roche to treat CLL. Venetoclax is believed to lead some cells, including some cells with CLL, to undergo cell death.

The FDA granted venetoclax a Breakthrough Therapy designation in April 2015 for the treatment of CLL in previously treated patients with the 17p deletion genetic mutation.

The two applications are supported by pivotal data from a phase II, open-label study of venetoclax in patients with relapsed/refractory CLL with 17p deletion. In August 2015, AbbVie announced the phase II study met its primary endpoint of achieving an overall response rate, according to an assessment by an independent review committee. The safety profile was similar to other venetoclax studies and no unexpected safety signals were reported.

Sorrento Therapeutics Inc. formed an exclusive partnership with the Karolinska Institutet in Stockholm, Sweden, to perform immuno-oncology research and to develop new natural killer cell-based therapies.

Under the agreement, Sorrento will sponsor preclinical and clinical research & development programs focused on NK biology as well as adoptive NK cell therapies and, in return, obtain full rights to

the resulting discoveries and developments.

The research will be performed at KI, but there will also be an active research exchange with Sorrento R&D in San Diego. A joint steering committee with members from both Sorrento and KI will guide the program activities.

"Given the fact that NK cells were discovered at Karolinska Institutet, it is of course now exciting to take part in the ongoing developments involving these cells in settings of adoptive immunotherapies targeting human malignancies," said Professor Hans-Gustaf Ljunggren, dean of research at Karolinska Institutet.

Eisai submitted a Marketing Authorization Application to the European Medicines Agency for the use of lenvatinib in combination with everolimus, to treat people with unresectable advanced or metastatic renal cell carcinoma who have received one prior vascular endothelial growth factor-targeted therapy.

A similar application has already been submitted to FDA. Lenvatinib was granted an accelerated assessment in Europe by the EMA in October 2015.

The application is based on a phase II trial of lenvatinib, which when used in combination with everolimus showed progression-free survival was significantly extended in people with metastatic renal cell carcinoma versus everolimus alone.

People treated with the combination regimen experienced a median progression-free survival of 14.6 months compared with 5.5 months for those who received everolimus alone (HR 0.40; 95% CI: 0.24-0.68; $p<0.001$). These data were presented at the American Society of Clinical Oncology in June 2015 and more recently published in Lancet Oncology.

Lenvatinib has been approved for the treatment of refractory thyroid cancer in the U.S., Europe, Japan, Switzerland and South Korea, and has been submitted for regulatory approval in Canada, Singapore, Russia, Australia and Brazil.

Lenvatinib was granted Orphan Drug Designation in the U.S. for treatment of follicular, medullary, anaplastic, and metastatic or locally advanced papillary thyroid cancer and in Europe and Switzerland for follicular and papillary thyroid cancer.

MD Anderson Cancer Center and DelMar Pharmaceuticals Inc. entered into a collaboration to accelerate the clinical development of DelMar's lead anti-cancer candidate, VAL-083, for the treatment of glioblastoma multiforme.

MD Anderson will initiate a new phase II clinical study with VAL-083 in patients with GBM at first recurrence/progression, prior to Avastin (bevacizumab) exposure. Patients eligible for the study will have recurrent GBM characterized by a high expression of MGMT, the DNA repair enzyme implicated in drug-resistance and poor patient outcomes following current front-line chemotherapy. MGMT promoter methylation status will be used as a validated biomarker for enrollment and tumors must exhibit an unmethylated MGMT promoter for patients to be eligible for the trial.

DelMar recently presented interim data at the annual meeting of the Society for Neuro-Oncology from its ongoing phase II clinical trial with VAL-083 as a potential third-line therapy in GBM patients whose tumors have recurred following treatment with both temozolomide and bevacizumab.

Debiopharm International SA announced a collaboration in order to supply triptorelin pamoate 3.75 mg one-month formulation for patients suffering from salivary gland cancer participating in a clinical study sponsored by the European Organisation for Research and Treatment of Cancer.

EORTC intergroup trial 1206, "A randomized phase II study to evaluate the efficacy and safety of chemotherapy vs androgen deprivation therapy in patients with recurrent and/or metastatic, androgen receptor expressing, salivary gland cancer," is coordinated by the EORTC Head and Neck Cancer Group in collaboration with the International Rare Cancer Initiative UK Salivary Gland Cancer Group.

This study will accrue 152 patients at approximately 30 sites in 10 countries: Austria, Belgium, France, Germany, Greece, Hungary, Italy, the Netherlands, Portugal and the United Kingdom. The clinical study is expected to last until mid-2021.

The purpose of this study is to evaluate the efficacy and safety of chemotherapy versus androgen deprivation therapy in patients with recurrent and/or metastatic androgen receptors-expressing salivary gland cancers. There is currently no standard of care for treating salivary gland cancers.

Triptorelin is an agonist analogue of the natural gonadotropin-releasing hormone. Debiopharm has developed three sustained-release formulations of triptorelin pamoate. The one-, three- and six-month formulations have been registered in numerous countries in several indications, mainly for advanced prostate cancer and endometriosis, and are available under different brand names: Trelstar, Decapeptyl, and Pamorelin.