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THE CANCER LETTER
Inside information on cancer research and drug development
Vol. 43 No. 02
January 13, 2017
www.cancerletter.com

NCI DEVELOPS A "FORMULARY" TO MAKE IT EASIER FOR CANCER CENTERS TO TEST DRUG COMBINATIONS

With a simplified tech transfer agreement, six industry partners and 15 compounds (but no dedicated research funds), NCI challenges investigators to think creatively.

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Subscription $425 per year worldwide. ISSN 0096-3917. Published 46 times a year by The Cancer Letter Inc. Founded Dec. 21, 1973, by Jerry D. Boyd.

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NCI DEVELOPS A "FORMULARY" TO MAKE IT EASIER FOR CANCER CENTERS TO TEST DRUG COMBINATIONS

By Paul Goldberg

With a simplified tech transfer agreement, six industry partners and 15 compounds (but no dedicated research funds), NCI challenges investigators to think creatively.

PROBLEM

Academic investigators encounter a regulatory maze and lengthy delays when they attempt to test cancer drugs. Obtaining the compounds for testing combination therapies is especially challenging.

SOLUTION

A formulary that tames the bureaucratic procedures to allow investigators at NCI-designated cancer centers to get the drugs—and clearance—faster.

"It has been a considerable problem over many years to bring drugs together into combination treatments for agents that are derived from a variety of different companies," said James Doroshow, NCI deputy director for clinical and translational research and director of the Division of Cancer Treatment and Diagnosis.

Working under the general rubric of the National Cancer Moonshot Initiative, NCI has put together a public-private partnership, starting with six participating companies and 15 investigational agents. Pharma companies sign a uniform technology transfer agreement dubbed CRADA Lite by one insider, CRADA being the acronym for the Cooperative Research and Development Agreement. The formulary was announced Jan. 11.

Under the formulary's rules, drug companies have a right to allow—or nix—an investigator's access to their agents. However, under shortened review deadlines, they have 60 days to respond.

As the administrator of the formulary, NCI acts as a clearing house, getting the compounds to investigators. The drugs are provided at no cost.

NCI doesn't fund the research and doesn't hold the Investigational New Drug license from FDA. Under a standard CRADA, the institute holds the IND.

"This process, we hope, will facilitate the ability of investigators at our NCI-designated cancer centers to use novel drugs in combination, which we think will be very useful in trying to address the new ability—through next generation sequencing—to find mutational aberration that actually, to be best treated, require combinations of treatments," Doroshow said at a press call rolling out the formulary.

CRADA agreements and instructions for companies are posted here, and instructions for investigators are posted here.

The companies that have provided the initial fifteen compounds are:

- Bristol-Myers Squibb
- Eli Lilly and Co.
- Genentech
- Kyowa Kirin Pharmaceutical Development Co.
- Loxo Oncology
- Xcovery Holding Co. LLC

COMPOUNDS AVAILABLE THROUGH THE NCI FORMULARY – Source: NCI

Agent Name (other names)	NSC Number	Company	Agent Class	Agent Target/ Molecular Target(s)
Alectinib	794611	Genentech	ALK inhibitor, tyrosine kinase inhibitor	ALK, RET
Atezolizumab	783608	Genentech	PD-L1 blocking monoclonal antibody	PD-L1
Bevacizumab	704865	Genentech	Anti-angiogenesis inhibitor, monoclonal antibody	VEGF
Cobimetinib	781257	Genentech	MEK1/2 inhibitor	MEK1/2
Ensartinib	784729	Xcovery Holding Company LLC	ALK inhibitor	ALK, TrkA, TrkC, ROS, EphA2, c-MET
Ipilimumab	732442	Bristol-Myers Squibb	anti-CTLA-4 monoclonal anibody	CTLA-4
Larotrectinib	788607	Loxo Oncology	Tyrosine kinase inhibitor	NTRK-1, NTRK-2, NTRK-3 fusion proteins; TrkA/B/C proteins
LY3039478		Eli Lilly and Company	Notch inhibitor	Notch
Nivolumab	748726	Bristol-Myers Squibb	PD-1 blocking monoclonal antibody	PD-1
Obinutuzumab	793436	Genentech	anti-CD20 monoclonal antibody	CD20
Pertuzumab	740102	Genentech	anti-HER2 monoclonal antibody	HER2/neu
Prexasertib		Eli Lilly and Company	Checkpoint kinase 1 inhibitor	CHK1
Trastuzumab	688097	Genentech	anti-HER2 monoclonal antibody	HER2/neu
Vemurafenib	761431	Genentech	BRAF mutant v600 inhibitor	BRAF V600 mutant, CRAF, ARAF, wild-type BRAF, SRMS, ACK1, MAP4K5, FGR
Vismodegib	747691	Genentech	Hedgehog Inhibitor	Smoothened

"This program is an opportunity to remain close to the academic community and access the best ideas through more accessible, streamlined research models as we continue the never-ending fight against cancer," Christopher Slapak, vice president of early phase clinical research, and Distinguished Lilly Scholar at Lilly, said to The Cancer Letter. "Lilly's participation in the National Cancer Institute's formulary is an opportunity to work with the top cancer centers looking for novel, innovative ideas to help advance our pipeline molecules. The data generated will answer important questions from research that Lilly would not necessarily be able sponsor itself."

Investigators wouldn't receive money specifically for studies done under the formulary. However, their institution may receive funds under any one of NCI's existing grants.

They can also approach the pharmaceutical sponsors for funding or technologic support which may or may not be needed, depending on the research. An example could be support for a vendor that already has methods, needed to support future regulatory submissions by the industry collaborator, to conduct pharmacology or biomarker testing.

NCI would be fine with having companies pose research questions for potential studies, with the institute essentially acting as a matchmaker.

"It is an interesting and important opportunity that should diminish the challenges of access to new agents for investigator-initiated studies and encourage more flexibility for combination drug trials that have been difficult when they combine agents from multiple suppliers," said Stanton Gerson, president of the Association of American Cancer Institutes and director of Case Comprehensive Cancer Center and Case Western Reserve University and Seidman Cancer Center at the University Hospitals Cleveland Medical Center. "It does not provide support other than drug access, but this is an important first step."

Since the institute already has the infrastructure for distributing drugs and collecting data, the cost of the program in fiscal 2017 will be under $1 million, NCI officials said. Spending on the program would increase if investigators use it.

Research could lead to new indications

For companies, investigator-initiated trials could produce scientific leads that may translate into new indications.

The regulatory schema for getting new indications for some compounds through FDA approval appears to favor drug combinations.

Consider cancer immunotherapies that rely on the PD-1 protein and its ligands, PD-L1 and PD-L2. In what looks like a race to the market, the industry is experimenting with 20 of these drugs. A recent analysis by The Cancer Letter found 803 registered clinical trials of these agents, suggesting an unprecedented push. These studies had slots for 166,736 patients (The Cancer Letter, Oct. 7, 2016).

How does the agency plan to process the expected flood of applications for these similar drugs?

"We anticipate combinations of drugs in this field will be the future," said Gideon Blumenthal, lead medical officer at the Office of Hematology and Oncology Products, FDA Center for Drug Evaluation and Research (The Cancer Letter, Nov. 11, 2016).

"After all, in second-line lung cancer in an 'unselected' patient population, only about 20 percent of patients respond to PD-1/PD-L1 inhibitors.

"Even in PD-L1 high-expressing lung cancer, about 40 percent of patients respond. There is a huge unmet need for addressing those patients who do not benefit from these drugs, as well as those patients who do respond initially but eventually develop resistance.

"This is where data sharing and improved collaboration is critical. The community as a whole needs to be shrewd in trial designs in terms of figuring out which patients are likely to respond to monotherapy, which are unlikely to respond, and figuring out which combinations to prioritize and move forward and which combinations should fail quickly."

FDA is not involved in the formulary.

Doroshow said several companies are finalizing their participation in the formulary.

"We are launching today, because our initial goal was to start with at least five companies with at least ten drugs," Doroshow said on the press call. "We have agreements with six companies for 15 drugs, and it's our expectation that over the relatively near term we will have several more companies and many more agents, and that that will continue to grow over the course of time that it takes to conduct these negotiations. That being said, in the relatively near future, the range of compounds that are available will be substantially larger."

Takeda Pharmaceuticals is one of the companies preparing to take part in the formulary.

"Building on research with single agents or treatments that combine agents from multiple industry collaborators, the formulary will encourage speed, flexibility, and innovation, including expanded experience with new technologies, biomarkers, and therapeutic platforms designed to introduce personalized approaches to benefit patient care," said Howard Fingert, senior medical director at Takeda Pharmaceuticals.

"Through these new collaborations between academics and industry sponsors, the formulary should also enable shared learnings that promise to advance R&D productivity and quality, including experience with data standards, bioinformatics, evaluation of biospecimens & biorepositories, and data-sharing."

Idea grew out of NCI-MATCH

The task of putting together combination therapies has been daunting for years.

"Let's just say that there are two drugs, one from Company A and one from Company B," Doroshow said on a media call announcing the formulary. "The issue—especially for trials that are not under the auspices of the NCI—involved having formal negotiations with each of those separate companies, and then developing a way to convince both of them that the compounds should be combined, and that they should separately and together be able to share any rights that are developed from the clinical trial itself.

"That does involve significant negotiations with each company and then with the institution that wishes to carry out that trial, and then one has to negotiate whether or not the two companies would allow their compounds to be used together."

A standard CRADA often took a year to negotiate. After said CRADA became active, review of a letter of intent could take a year, without necessarily resulting in a nod.

"One of the things that we have negotiated with our agreements that are now in place is the commitment on the part of the companies to do a relatively rapid review—up or down—of the clinical trials ideas," Doroshow said. "That's something that can happen, apparently. It often doesn't happen. The notion that the companies will commit over a relatively short period of time to either providing agents or not providing agents is something that has ever happened before. It's not something that even with our other CRADA arrangement, with other trials that are, in fact, supported by NCI grants, we do not have that built-in negotiated rapid review as part of those

agreements, and sometimes that takes longer than we would like. And so we are hoping this whole process will be substantially faster."

The magic number for formulary review is 60 days.

"The companies will have a full review and approval process for the proposals that are coming in," said Sherry Ansher, associate chief of the Regulatory Affairs Branch of the Cancer Therapy Evaluation Program. "One of the difference between this and the [standard CRADA] program is that NCI will not be doing a scientific or clinical review of the proposal.

"It would be left to the pharmaceutical collaborator, and the 60-day window is that we are asking them to make this determination as to whether they want to provide agents for the study within 60 days of receiving that proposal. And then if they approve it, the investigator would move to writing a full protocol for the trial."

The idea for the formulary grew out of the NCI-MATCH trial, Doroshow said.

"This has been an issue for some years, but we didn't think that it might actually be possible to do this until my colleagues started a prolonged and very successful process of negotiating the drugs that became part of the formulary, if you will, for the NCI-MATCH trial, a trial that is trying to associate a particular molecular abnormality with an investigational agent," Doroshow said.

"Many companies were engaged with us in trying to develop, under one IND, this large umbrella phase II trial. The success of negotiating all of those different arrangements for the provision of drugs for that trial made it possible to think that something of the nature of this formulary to be used for many trials that would be investigator-initiated might be possible."

How it works:

- An NCI Formulary CRADA is executed between the NCI and pharmaceutical company collaborator to afford accessibility to the agent(s).
- CTEP acts as a "facilitator" of submitted proposals from NCI-Designated Cancer Center investigators. Investigators submit Letter of Intent (LOI) proposals for the NCI Formulary agent(s) to CTEP, and CTEP provides the pharmaceutical company collaborator with the proposals for review. CTEP will not provide scientific review of the Formulary LOI proposals. All scientific discussion is conducted between the pharmaceutical company collaborator and investigator.
- Pharmaceutical company collaborators are responsible for providing scientific review of the proposals and clinical trial, agent for the clinical trial, and a letter of cross-reference to their IND/DMF. A time limit of 60 days on the LOI review process will ensure that requests are processed in a reasonable time frame.
- Should a proposal be approved by the pharmaceutical company collaborator, the NCI will use its established clinical trial infrastructure to facilitate trial conduct.
- The study will be conducted under investigator-sponsored INDs by the requesting investigator/Cancer Center.
- NCI will transfer agents to the clinical trial sites via a clinical Material Transfer Agreement (MTA) executed between the NCI and the clinical trial site under which rights and responsibilities of the recipient investigator will be described.
- All serious adverse events will be submitted to the pharmaceutical company collaborators via CTEP's Serious Adverse Event Reporting System (CTEP AERS).
- Clinical trial data will be submitted via CTEP's Clinical Data Reporting System, which will be made available to pharmaceutical company collaborators.
- Intellectual Property will be handled under the terms of the CTEP IP Option to pharmaceutical company collaborator, with rights flowing down under the clinical MTA.
- Pharmaceutical company collaborators will have access to all data generated under the study and have the rights to review publications consistent with the current mechanism used in CTEP agreements.
- Neither NCI nor pharmaceutical company collaborators are required to provide per patient funding for the studies or any other study costs. Trial sites participating in this program are required to have the ability to support the full costs of the trial and demonstrate the source of this funding prior to protocol approval. However, if pharmaceutical company collaborator desires to provide funding for selected aspects of the study, they may provide funds directly to the investigators or could use the NCI CRADA mechanism to convey funding.
- Transfer of agent for preclinical work in support of clinical trials will also be facilitated under the NCI Formulary. It is anticipated by NCI that any agent provided for clinical trials would be made available for preclinical studies as well, as this may be critical to the rationale and design of the clinical trial. Agents may also be available for preclinical studies only.

MARKEY CANCER CENTER

In a region where cancer is at its worst, it takes bold action to make a difference. That's why the University of Kentucky Markey Cancer Center has set an ambitious goal: significantly reduce cancer incidence and mortality in our state, and the Appalachian region, by 2020. With the momentum we're building, we believe **MARKEY CAN** do it.

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The Albert Einstein College of Medicine and the Montefiore Health System are seeking a Director for the Albert Einstein Cancer Center at Einstein and Montefiore to lead one of the nation's most long-standing and prestigious Cancer Centers in integrating basic, clinical, translational and population sciences research with clinical care to accelerate scientific discovery and deliver the highest quality of cancer care.

As one of the first three National Cancer Institute designated academic cancer research centers, the Albert Einstein Cancer Center (AECC) has been continuously funded since its inception in 1972. The AECC coordinates all aspects of the College's science on cancer. A matrix center within the Albert Einstein College of Medicine, the AECC is supported by the scientific advances of the College. Designed to foster basic, clinical, translational and population sciences research to enhance the understanding of the origins of cancer and its effective detection, prevention, and treatment, the AECC conducts some of the region's most advanced cancer research. The merger of Montefiore and the College of Medicine brought an already collaborative relationship even closer, exemplified by development of coordinated clinical Centers of Excellence, and the appointment of a series of outstanding new clinical department chairs. Also, this new phase in the relationship between Einstein and Montefiore brought the opportunity to fully integrate the research-focused, National Cancer Institute (NCI)-designated AECC and clinical care delivered at the Montefiore Medical Center as outlined in a newly developed strategic plan. It is anticipated that the name of the Albert Einstein Cancer Center will be changed in the future to reflect the integration of Einstein and Montefiore.

Reporting to the Dean of the Einstein College of Medicine and the EVP and Chief Operating Officer of Montefiore, The Director is responsible for recruiting key faculty and staff, in partnership with Department Chairs, overseeing the development and operation of signature programs, setting overall strategic directions, and accelerating the Center's biomedical cancer research to the highest levels of excellence. He/she will also develop the annual operating and capital budgets, allocate research space to faculty, manage shared resources and other facilities within the Center, administer the sponsored award portfolio, and College and philanthropic funds, and collaborate with relevant department chairs.

The successful candidate will be an internationally recognized physician-scientist leader in cancer research who is knowledgeable in the care of patients with cancer across complex biomedical organizations. Ideally, she/he will have an impressive set of accomplishments and will have a track record that includes: a record of accomplishment in cancer research and deep understanding of basic, translational, clinical and population-based science; the necessary academic accomplishments and track-record of peer-reviewed funding to be appointed at the rank of Professor (with tenure) at Albert Einstein College of Medicine; previous successful senior leadership role in oncological science within academic medicine or industry; demonstrated excellence in attracting, recruiting, and developing world-class scientific and clinical leaders and successful experience in program development encompassing team building, strategy setting, and advancing scientific goals and programmatic funding; demonstrated managerial and business acumen; experience in philanthropy and a willingness to lead philanthropic efforts to support Cancer Center programs.

Spencer Stuart has been retained to assist with this most important recruitment. Spencer Stuart and the Search Committee respect the importance of maintaining confidentiality. Letters of application, with curriculum vitae, and letters of nominations should be submitted by email (preferred) to: dwestmore@spencerstuart.com

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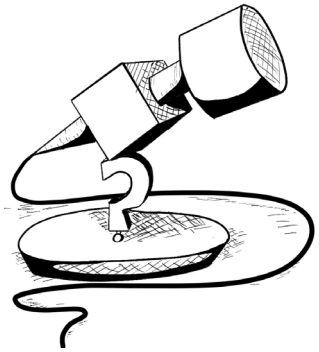


Simon: The moonshot will continue, but the electronic health record industry must step up

CONVERSATION WITH THE CANCER LETTER

Greg Simon
Executive Director, White House Cancer Moonshot Task Force

> One of the things [Biden] talked about all year is the politics of cancer as being more difficult than the politics of politics. One of his superpowers is that he is able to bring people together who otherwise might never meet to work on common problems to come up with a common solution.

The National Cancer Moonshot Initiative was created by a memorandum issued by President Barack Obama.

The wide-ranging public health initiative will cease to exist on Jan. 20—eight days short of a year after it was created—when the keys to the White House are handed over to Donald Trump.

The money remains, as does the name: the 21st Century Cures Act slates $1.8 billion over seven years for the Beau Biden Cancer Moonshot. In the absence of Trump's commitment to renew the initiative, it is unclear whether the moonshot will continue as a White House project.

"I had not spoken with anybody from the transition," Greg Simon, executive director of the White House Cancer Moonshot Task Force, said to The Cancer Letter. "There has not been any formal meeting with the moonshot team that I'm aware of. That said, obviously, there's a lot of interest in the moonshot, Republicans and Democrats."

Vice President Joe Biden will continue to be an effective convener after he leaves the White House—through the moonshot nonprofit that he plans on creating, Simon said.

"One of [Biden's] superpowers is that he is able to bring people together who otherwise might never meet to work on common problems to come up with a common solution," he said. "In whatever guise, as an individual or as the head of a nonprofit, he would continue to partner with other nonprofits, because that's his nature."

Obama earlier this week awarded Biden the Presidential Medal of Freedom, with distinction, exactly a year after Obama announced the moonshot in his seventh, and final State of the Union address.

The award is the nation's highest civilian honor.

"He suited up for our Cancer Moonshot, giving hope to millions of Americans touched by this disease," Obama said at the surprise award ceremony Jan. 12. "To know Joe Biden is to know love without pretense, service without self-regard, and to live life fully. As one of his longtime colleagues in the Senate, who happened to be a Republican, once said, 'if you can't admire Joe Biden as a person, you've got a problem. He's as good a man as God ever created.'"

Simon said he is optimistic about Congressional funding for the moonshot over the next seven years, even though the $1.8 billion needs to be funneled through NIH, before it reaches NCI to implement the scientific recommendations of the Blue Ribbon Panel (The Cancer Letter, Dec. 2, 2016).

"As you know, the NIH and the NCI have had, let's just say, tensions around the family dinner table since the Nixon War on Cancer," Simon said. "The other thing—I'm sure probably came into it—is that there's an acting director at the NCI, and there's Francis Collins at NIH. Doug Lowy is a terrific person, but I'm sure there must have been some trepidation on the Hill that he may not be the permanent director, and if he weren't, they wanted to make sure the money was run by someone who's in office.

"I think the key thing is, getting $1.8 billion and having it have a one-stop flight instead of nonstop flight to get to the NCI, is not a problem in our view. While it was not a proposal from us, the Congress … both the authorizers and the appropriators, chose to funnel the money through NIH and have the disposition to NCI occur through coordination with NIH. I don't think that's going to be a problem."

In 2016, the moonshot endorsed and highlighted numerous initiatives—including NCI's $20 million Genomic Data Commons—aimed at accelerating scientific discovery through aggressive data sharing for cancer research (The Cancer Letter, April 29, 2016). However, another piece of the bioinformatics puzzle remains largely unsolved: what else can the moonshot do to promote interoperability and common standards among electronic health record companies for aggregating patient data? (The Cancer Letter, June 3, 2016)

"We have to turn to the industry in response to the Cures Act and say, 'Okay, you're a multi-billion dollar industry now, you got $30 billion [in 2009 ARRA funding] from the government. It's time for you to step up the way the credit card industry did many years ago," Simon said. "I think this, at this point, should be an industry-led solution, because the government can only lead the industry so far if the industry doesn't want to be led. At this stage of the game, the industry needs to really take up the bid and run with it, and that's what we hope we'll see."

Simon said interoperability would continue to be at the "top of the chain of priorities" for Biden, who recently said he would also address the issue of drug prices after he leaves the White House (The Cancer Letter, Jan. 6).

"I think powerful message and passionate people don't need power of the White House to get things done," Simon said. "I think what Biden does have is authenticity, and passion, and credibility with people who have been touched by cancer, and they are willing to help him help them, not because they get to come to the White House, but because they like having his leadership."

Q & A

Simon spoke with Matthew Ong, a reporter with The Cancer Letter.

What would you say is the most important and tangible impact of the moonshot in 2016?

Greg Simon:
I think the most tangible aspect of the moonshot was creating a national movement to rethink how we approach cancer research and development.

It's not about any one project that we did, although there were over 70 that had been put together, and that continues to grow, but it's about the motivation behind what's creating these activities, and that is people responding to the vice president's passion for us to bring a bigger sense of urgency to the fight against cancer, and to change the system to be more appropriate to the tools we have at hand in the 21st century. So we see people actively rethinking what they can get done, how they can get it done.

The reason I say that's our biggest achievement is because that is going to make sure this continues beyond our time in the White House.

Would you say you are happy with what you've achieved? Do you have any regrets? Or is there anything that you could have done differently, looking back?

GS:
Certainly, I don't have any regrets. I think we couldn't get to everything that people wanted us to get to and that we wanted to get to, given the constraints of time and resources. I mean, we started in February. I joined in March. It took me most of April to put a team together, but once we had the people working on this, I think we got more done than I had anticipated and more done, than I think most people anticipated, but that's because the idea took hold and that became a multiplier. It wasn't just we do something and then we do something else. We would do something and then there would be this enormous response that took it to a different level.

In terms of if we did it over again, I just think it would have been nice to have the Cures Act passed before the very, very, very last minute so we could have done more planning for the outyears. But the Blue Ribbon Panel's roadmap for how to spend additional money for the moonshot is a very good roadmap, and I think it's one that will be followed.

How do you see your roles—yours and the vice president Biden's—evolving, going forward?

GS:
As you know, there was the story in The Post regarding his setting up a nonprofit going forward, so he is planning to make that the center of his cancer activities. I'm not in a position yet to discuss any post-administration plans of my own, but in whatever I do I will continue to be involved in the fight against cancer, as I have been for many years.

Do you know any details about the upcoming nonprofit organization? How much money would be going into it, and where would it come from? Will it be from the industry, the public, philanthropy, or all of the above?

GS:
No, is the short answer. I know that I'm not allowed to talk about the fundraising details—neither is he—until we leave the office, but it would be a 501(c)(3) nonprofit. How he raises the money and from whom is not something that I'm privy to, nor could I even talk about until we leave office.

How will the moonshot have continuity and gravitas, now that Mr. Biden and you will soon be working without the authority of the White House?

GS:
My last boss in the White House was Al Gore. Without the benefit of the White House, he was part of a Nobel Prize and an Oscar for raising awareness about climate change. I think powerful message and passionate people don't need power of the White House to get things done.

Joe Biden happened to be in the White House when this tragedy happened in his family. The world is full of examples of passionate people, from families to leaders of the free world, who take on a cause and change the future of that problem, who don't have the power of the White House behind them.

I think what Biden does have is authenticity, and passion, and credibility with people who have been touched by cancer, and they are willing to help him help them, not because they get to come to the White House, but because they like having his leadership.

As a nonprofit, will the moonshot also become an advocacy organization? Meaning, do you foresee the moonshot nonprofit joining other cancer groups in lobbying efforts?

GS:
During the moonshot, we have been cooperating and coordinating with any foundation we can find that is working in the cancer space, and many not in the cancer space, who have solutions that apply to cancer. I do not think that that's going to stop when he leaves the White House.

One of the things he's talked about all year is the politics of cancer as being more difficult than the politics of politics. One of the reasons is because there's so many cancer foundations, and they don't always play together well. One of his superpowers is that he is able to bring people together who otherwise might never meet to work on common problems to come up with a common solution.

In whatever guise, as an individual or as the head of a nonprofit, he would continue to partner with other nonprofits, because that's his nature.

Let's talk about the funding for the moonshot. Key players in oncology and pretty much the entire cancer community have stated that funding for the moonshot in the Cures bill should have gone directly to NCI. The question is, why did the language of the bill give the funding to NIH, as opposed to NCI, despite unanimous support for the institute as the leader of the National Cancer Program?

GS:
Number one, as you know, the NIH and the NCI have had, let's just say, tensions around the family dinner table since the Nixon War on Cancer. And of course, the NCI has the bypass budget, which they do directly with Congress, but NCI does have a very integrated relationship with the NIH.

While it was not a proposal from us, the Congress … and let me add one more other thing, not all cancer funding comes out of NCI. NIH does a lot of cancer funding. But the Congress, both the authorizers and the appropriators, chose to funnel the money through NIH and have the disposition to NCI occur through coordination with NIH. I don't think that's going to be a problem.

The FY17 spending, from everything I've seen, is going to follow the Blue Ribbon Panel recommendations, and there is no tension there between the NCI plan and the NIH plan.

The other thing—I'm sure probably came into it—is that there's an acting director at the NCI, and there's Francis Collins at NIH. Doug Lowy is a terrific person, but I'm sure there must have been some trepidation on the Hill that he may not be the permanent director, and if he weren't, they wanted to make sure the money was run by someone who's in office. That's just conjecture on my part.

I think the key thing is, getting $1.8 billion and having it have a one-stop flight instead of nonstop flight to get to the NCI is not a problem in our view.

Have you or Mr. Biden heard from the Trump administration on the moonshot?

GS:
I had not spoken with anybody from the transition. The vice president has met with Mr. [Mike] Pence a few times. There has not been any formal meeting with the moonshot team that I'm aware of.

Now, let me also point out, the presidential memorandum that created the moonshot expired with the administration no matter what, so whoever was president would have to start the moonshot again legally for it to continue. So technically, when the transition began inside the White House, the moonshot was not part of a transition, because we had an expiration date of Jan. 20.

Unlike other offices that continue no matter what, we have to be renewed, so to speak. That meant that we were not on the top of anybody's list who was looking at transition memos, because we were not a group that carried over. That said, obviously, there's a lot of interest in the moonshot, Republicans and Democrats. There's still a few weeks, so we don't know what could happen.

Experts have said that one of the core problems with data-sharing is the lack of incentives for the electronic health record companies to interoperate—an unforeseen consequence of the 2009 stimulus program. Has the moonshot made headway with EHR companies in 2016?

GS:
We [had a meeting Jan. 6] focused on the provisions of the Cures Act that require providers and the EHR vendors to make it possible for patients to download their records in a way that they can transfer them to other providers or repositories or researchers. We included a wide swath of people from the provider industry, the technology industry, the EHR industry.

But before that, there were many things going on over the last eight years that dovetailed with the moonshot's

interest in data-sharing. There's the so-called Sync for Science project that was about coding medical records in a standard way so that they can easily be shared. There is the medication list pilot that was just released at the recent meeting of the Office of the National Coordinator for Health IT that demoed people being able to download their full medication list and move it around as they wish.

There are a lot of things that had been going on, but the big question is how soon will it be as simple as downloading anything else on the web for people to download their medical records in a way they can use them, share them, manipulate them in terms of highlighting things and moving things around so they can share them with doctors for second opinions, they can share them with researchers, they can share them with other providers? That is the open question.

Who do you think would be in the right position to lead that effort? Who can do it, and what would it take?

GS:
The history, so far, of health IT, going back to the Bush administration, has been a stepwise iteration of technical standards and so-called Meaningful Use criteria to guide the development of medical records.

Then, with the HITECH Act at the beginning of the Obama administration, the 30 or so billion dollars was spent that supercharged the adoption of health electronic records, before all of the standards had been created for interoperability and for easy patient access, downloading, and data sharing. It is pretty clear that if we continue to do stepwise iterations of standards, it will be another decade before we reach interoperability and easy access to records. That has to change.

The fact that the thirty billion dollars did not fix that problem but just sort of spread the problem around by making electronic medical records more ubiquitous, but not more facile, means that now, we have to turn to the industry in response to the Cures Act and say, "Okay, you're a multi-billion dollar industry now, you got $30 billion from the government. It's time for you to step up the way the credit card industry did many years ago, which is to get together in a room, and within a month, pull together standards that allow you to share information between any systems, with any systems, with any user anywhere in the world, for whatever purpose the user wants, because it's their data."

Now, I'm not saying that's easy, but it is definitely not as hard as we've made it, and there are ways to do it today. There are third parties that are not in the health business who are ready and able to either introduce area in a new open-source way, or be integrators so that any system's data can be merged with any other system's data through a third-party software.

I think this, at this point, should be an industry-led solution, because the government can only lead the industry so far if the industry doesn't want to be led. At this stage of the game, the industry needs to really take up the bid and run with it, and that's what we hope we'll see.

Does the Cures Act provide a deadline for the EHR industry to make their records downloadable and transferable?

GS:
No.

It seems like the onus is on Congress to push for urgency on this issue.

GS:
Yes, that is something that has to happen. Congress has been very active in this area for years. It is something that Congress, I'm sure, will do oversight on. There's a very easy reason why Congress cares about it. They're all patients. They've all experienced the problem. If they haven't, their constituents have, and they tell them about it.

Let me clarify one thing I said about the Sync for Science. I mentioned that it was a coding, but the purpose of the coding isn't just to do coding. It's to allow patients to access their data more quickly and send it to researchers through creating an electronic environment where you can share the records electronically much more easily.

But that is a pilot. That is a pilot with a small group of companies and users. We have to get past the pilot stage and really get to the real thing the real way. That's for the last question. That has to be industry-led and, industry needs to take responsibility for it.

This is definitely one of the priorities for the moonshot going forward, anyway.

GS:
Yes. If you've followed Biden all year, data sharing has been at the top of the chain of priorities. I might point out that just today, in my news-gathering that I do every day, there were two or three articles on the impact of health industry being behind the curve in using Big Data and the problems that can be solved with a much more aggressive use of Big Data by the health industry.

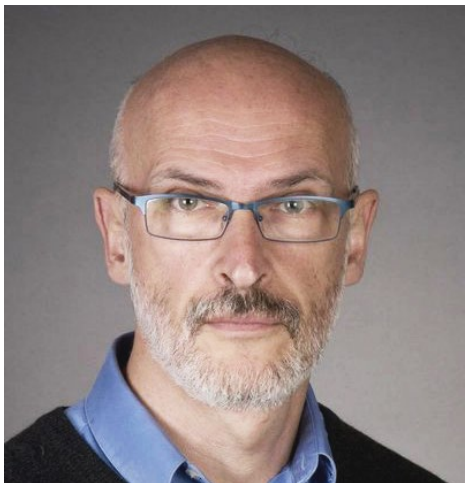
IN BRIEF

Sakaguchi, Ramsdell, Rudensky win Crafoord Prize for discoveries in immune regulation

Three immunology researchers shared the 2017 Crafoord Prize in Polyarthritis "for their discoveries relating to regulatory T cells, which counteract harmful immune reactions in arthritis and other autoimmune diseases."

Shimon Sakaguchi, of Osaka University, discovered and documented the occurrence of regulatory T cells by systematically investigating cells that develop in the thymus of young mice in a series of experiments from 1985 onwards.

Fred Ramsdell, head of research at Parker Institute for Cancer Immunotherapy, identified the faulty gene in some mice and children that are born with IPEX, a severe autoimmune disease, in 2001. This gene, FOXP3, has proven to be vital in the development of regulatory T cells.



Alexander Rudensky, of Memorial Sloan Kettering Cancer Center, knocked out the FOXP3 gene in mice in 2003, so they were unable to form regulatory T cells and thus suffered from severe autoimmune diseases. At about the same time, Sakaguchi and Ramsdell independently presented evidence that FOXP3 governs the formation of regulatory T cells.

The prize money is 6 million Swedish krona, about $1.3 million. The Crafoord Prize is awarded as a partnership between the Royal Swedish Academy of Sciences and the Crafoord Foundation in Lund. The Royal Swedish Academy of Sciences is responsible for deciding upon the Crafoord laureates.

The prize is awarded in one discipline each year, according to a set schedule for mathematics and astronomy, geosciences, and biosciences.

The prize for polyarthritis is awarded only when a special committee has demonstrated that scientific progress in this field has been such that an award is justified.

According to the prize committee:

"Even back in the 1960s, researchers were searching for suppressor cells in the immune system, but the research results were contradictory. Accordingly, over time, the consensus became that no such cells existed. Despite this, Shimon Sakaguchi persevered with the search and, after many years, he succeeded in identifying the cells that are now called regulatory T cells.

"Some years later, Fred Ramsdell approached the same area from a different direction; he isolated and identified the gene that is linked to severe autoimmune disease in a particular strain of mice. He also demonstrated that mutation in the same gene in humans, now known as FOXP3, causes a severe congenital disease called IPEX. Shortly afterwards, decisive findings were made, linking these two pieces of knowledge together. Alexander Rudensky, Shimon Sakaguchi and Fred Ramsdell each described how the FOXP3 gene is vital to a process that results in some T cells becoming security guards in the immune system. These are the regulatory

T cells, which can prevent autoimmune reactions because they detect and suppress overzealous colleagues in the immune system.

"A great number of clinical trials are now being conducted globally, with research teams testing various ways of using regulatory T cells to subdue the immune system's attacks that cause autoimmune diseases. The long-term vision is that of a breakthrough in the treatment of polyarthritis and other autoimmune syndromes, which could be treated more effectively than they are today."

The prize will be awarded in Stockholm on May 18.

Gustavo Leone named director of MUSC Hollings Cancer Center

Gustavo Leone was named director of the Medical University of South Carolina Hollings Cancer Center. His appointment begins March 1.

Leone will continue to conduct laboratory and translational research at MUSC, focusing on identifying how disruption of critical cell cycle regulatory pathways contributes to uncontrolled cell growth. Currently his laboratory group focuses on studying how genes outside the tumor cell affect the community of cells around a cancer cell, a research area that may reveal new cancer treatment strategies.

HCC includes more than 120 faculty-level cancer scientists with an annual research funding portfolio of $44 million. A primary goal for Leone will be to support and enhance the infrastructure key to the center's prestigious NCI-designated status and to build programming and recruitment efforts to attain NCI Comprehensive Cancer Center status.

Leone earned his doctoral degree from the University of Calgary and completed a postdoctoral fellowship at Duke University in 1998 before joining The Ohio State University as an assistant professor at OSU's NCI-designated James Comprehensive Cancer Center. Leone advanced to full professor in molecular genetics in 2011 and held the Klotz Chair in Cancer Research. In his leadership positions as director of the Solid Tumor Biology Program and associate director for basic research, Leone also expanded mentoring, recruitment, and collaborative research efforts as a founding member of the Pelotonia Fellowship Program in Cancer Research.

Levine Cancer Institute is first to earn top designation for patient-centered care

Levine Cancer Institute was named a "Planetree Designated Patient-Centered Organization," making it the only cancer network worldwide to earn Planetree designation, the highest achievement in patient-centered care.

In addition to being the first outpatient cancer network to receive the designation, Levine Cancer Institute is the first multi-site center to receive the designation and the first outpatient network. Because of this, the institute's role in defining criteria and benchmarks for other cancer centers and multi-site institutes seeking designation is critically important. More than 20 of the institute's sites share the designation.

"The designation signals to patients, providers and everyone with an investment in cancer care that Levine Cancer Institute is an organization where providers partner with patients and families, and where patient comfort, dignity, empowerment and well-being are prioritized with providing top-quality clinical care," said Susan Frampton, president of Planetree.

The Planetree Designation is an award that recognizes excellence in person-centeredness across the continuum of care and is based on evidence and standards within the health care industry. The criteria that an organization must satisfy to achieve designation reflect what patients, family members and health care professionals believe matters most to them during a health care experience, including quality and clinical outcomes.

There are 31 health care providers in the U.S. and 81 world-wide that have earned Planetree Designation. "Patient-centered care is about providers taking a step back and realizing that we're experts in cancer care and treatment but patients are experts in their lives and needs, and it's this recognition that is at the center of everything we do," said Derek Raghavan, president of the Levine Cancer Institute.

"By combining the leading-edge oncology treatments, nationally renowned physicians, careful cancer research and demonstrated clinical outcomes with a truly patient-centered care approach, we have built a network that allows our Institute to provide tremendous clinical care for patients. This extends far beyond the traditional definition of

care and demonstrates what is possible when all aspects of treatment are truly integrated. Carolinas HealthCare System has its own internal system of evaluating patient-centric care, but this external validation for our cancer institute program will be crucial for the welfare of our patients and families."

To achieve designation, Levine Cancer Institute has undergone rigorous site visits by a team of Planetree representatives, which included focus groups with recent Levine Cancer Institute patients, families and current staff validating that specific patient-centered policies are in place. The process also included a review of the organization's performance on patient satisfaction and quality of care measures, and how measurement of these indicators improves organizational outcomes.

Martin Edelman joins Fox Chase as hematology/ oncology chair

Martin Edelman joined Fox Chase Cancer Center as chair of the Department of Hematology/Oncology.

He will also serve as deputy cancer center director for clinical research, leading the effort to integrate discoveries from the Translational Research Initiative into a strong investigator-initiated clinical trials program.

Edelman will collaborate with clinical, scientific, and administrative leadership to grow robust therapeutic, clinical research, and translational research programs in hematology and medical oncology while leading the department in evaluating emerging national trends in the delivery of cancer care.

Edelman comes to Fox Chase from the University of Maryland Greenebaum Comprehensive Cancer Center, where he served most recently as the head of the section of solid tumor oncology and associate director of the division of hematology/oncology. In addition, he was a professor of medicine and radiation oncology at the University of Maryland School of Medicine.

Edelman developed one of the most commonly used regimens for treating advanced lung cancer and working toward the development of new agents and biomarkers to personalize lung cancer therapy. He has a particular interest in approaches that integrate surgery, radiation, and chemotherapy in the management of lung cancer patients.

He serves on the Lung Cancer Committee of the Alliance, the Board of Directors of the Alliance for Clinical Trials in Oncology, and the Thoracic Malignancies Steering Committee of the National Cancer Institute's Scientific Review Group. In addition, he is the Medical Oncology Co-chair for the Lung Cancer Committee of the Radiation Therapy Oncology Group.

He has been active within the American Society for Clinical Oncology and has chaired the lung cancer sections for the scientific and educational committees as well as a past member of the governmental affairs committee. As a member of the International Association for the Study of Lung Cancer, he has chaired the Career Development and Ethics Committees.

Diane Simeone to lead pancreatic cancer center at NYU Langone

Diane Simeone will join the NYU Langone Medical Center's Perlmutter Cancer Center March 1 to serve as associate director for translational research and to lead its newly established pancreatic cancer center. Simeone is the director of the gastrointestinal oncology program at University of Michigan Comprehensive Cancer Center.

Her laboratory was the first to identify pancreatic cancer stem cells, a discovery that might explain why current drug therapies are ineffective against the disease. She also leads a research program on pancreatic cancer prevention, early detection and therapeutics, and holds major leadership positions with organizations advancing pancreatic cancer research and advocacy worldwide.

Mario Contreraz named administrator of IU cancer center clinical trials office

Mario Contreraz was named administrator of the Indiana University Melvin and Bren Simon Cancer Center's Clinical Trials Office.

As administrator, Contreraz will provide overall direction for the strategy and vision for the Clinical Trials Office to ensure all NCI criteria and guidelines are met. He will also serve as a liaison between the administrative staff and the sponsors, collaborators, and regulatory agencies.

Contreraz had served as interim administrator for the Clinical Trials Office during the recruitment process. He initially joined the Clinical Trials Office in May 2016 as the research nurse administrator.

Prior to joining the CTO, Contreraz was a clinical research manager at the IU School of Medicine's clinical research center, a clinical research nurse at the Krannert Institute of Cardiolo-

gy, a nursing associate at Eli Lilly and Company, and an ER nurse at Eskenazi Health. He holds both a master's of nurse administration, a master's of business administration from Anderson University, and a bachelor's degree in nursing from Ball State University.

Columbia, NewYork-Presbyterian and Life Raft form GIST research partnership

Columbia University Medical Center, NewYork-Presbyterian and The Life Raft Group, a patient advocacy organization specializing in gastrointestinal stromal tumors cancer, entered into a collaborative research project to investigate the efficacy of a novel system biology approach for identifying the best treatment options for patients with advanced GIST.

The science behind the approach, developed in the Califano Lab at Columbia University, utilizes VIPER algorithm software (Virtual Inference of Protein activity by Enriched Regulon analysis) to investigate the molecular networks of GIST patients who have become resistant to approved tyrosine kinase inhibitors.

Although oncogene targets are already established in GIST, this will identify the master regulators or "tumor checkpoints" that represent the final on and off switches in the GIST cells. Personalized therapeutic agents can then be selected for patients currently lacking any effective therapeutic options. Clinical and molecular data from the study participants will be stored in the Life Raft Group's Patient Registry, a unique data management analytics tool developed by the LRG, which tracks a patient's clinical history and links it to a companion record of tissue and mutational data housed in the LRG's Tissue Bank.

The project will launch with the mapping of tissue samples donated by patients to the LRG. The LRG will also serve as the monitoring arm of the study and use their proprietary research collaboration platform, InterGR, to provide investigators a centralized repository for all data collected.

Collaboration begins with six other academic institutions, including Fox Chase Cancer Center, Oregon Health & Science University, University of California San Diego, University of Miami, Washington University and Stanford University.

NCCN publishes patient education resources for gliomas

The National Comprehensive Cancer Network has published the NCCN Guidelines for Patients and NCCN Quick Guide sheets for Brain Cancer – Gliomas—the first in a series of patient education resources focused on brain cancer.

Published by NCCN through support of the NCCN Foundation, and partly through funding from NCCN Foundation's Team Pound the Pavement for Patients, these resources inform patients about their disease and the treatment options available to them.

"Access to new, easy-to-understand treatment information for patients with brain cancer empowers these patients to make informed decisions about their care. We are excited to present the new NCCN Guidelines for Patients – Gliomas and look forward to publication of the entire Brain Cancer Series," said Marcie Reeder, executive director, NCCN Foundation.

Support from NCCN Foundation's Team Pound the Pavement for Patients was donated in honor of Melanie Moletzsky, an employee of NCCN who was diagnosed with anaplastic astrocytoma—a type of glioma—in 2015. During her time in treatment—nearly two years—Moletzsky learned a lot about living with brain cancer and the side effects of the disease and its treatment.

She shares her insights in the new NCCN Guidelines for Patients: Brain Cancer – Gliomas. These resources are available free of charge on NCCN.org/patients, as well as on the free NCCN Patient Guides for Cancer mobile app, available for Android and iOS devices. NCCN plans to publish additional patient resources focusing on other forms of Brain Cancer in the future.

NCCN Guidelines for Patients, patient-friendly translations of the NCCN Clinical Practice Guidelines in Oncology, are easy-to-understand resources based on the same clinical practice guidelines used by health care professionals around the world to determine the best way to treat a patient with cancer.

NCCN currently offers NCCN Guidelines for Patients for the following:

Brain, Breast, Colon, Esophageal, Kidney, Non-Small Cell Lung, Ovarian, Pancreatic, Prostate, and Stomach Cancers; Acute Lymphoblastic Leukemia; Adolescents and Young Adults with Cancer; Chronic Lymphocytic Leukemia; Chronic Myelogenous Leukemia; Hodgkin Lymphoma; Lung Cancer Screening; Malignant Pleural Mesothelioma; Melanoma; Multiple Myeloma; Nausea and Vomiting; Non-Hodgkin's Lymphomas; and Soft Tissue Sarcoma.

The NCCN Guidelines for Patients and NCCN Quick Guide™ sheets for Brain Cancer – Gliomas are available to download for free from NCCN.org/patients and on the NCCN Patient Guides for Cancer mobile app.

NIHCM Foundation awards $345K in investigator-initiated research grants

NIHCM Foundation awarded seven new grants totaling $345,000 to support investigator-initiated health services research.

The winning studies were selected for their potential to improve the health care system and their strong research design.

"The studies we're supporting are developing practical evidence that is actively improving the effectiveness and efficiency of the U.S. health care system," said NIHCM CEO Nancy Chockley. "This investment in evidence is critical to achieving a healthier America."

The latest round of grants will support the following projects:

Promoting Better Pain Management Outcomes: Precision Decision Support for Opioid Prescription

This study will investigate the feasibility and impact of using personalized decision support to assist physicians with prescribing decisions by predicting a patient's risk of developing opioid dependence. Findings have the potential to improve prescribing practices and reduce the prevalence of addiction.

Researchers:
- Ritu Agarwal, University of Maryland
- Margret Bjarnadottir, University of Maryland
- Kislaya Prasad, University of Maryland
- Kenyon Crowley, University of Maryland

Selected Market Failures in Health Care: Analyzing the Scope, Causes, and Potential Solutions

This two-part study will examine health care market failures affecting consumers: 1) the frequency and causes of surprise bills for out-of-network services, and 2) the significance of various barriers to shopping around for services, including travel distance, referral practices, vertical integration and transparency. Results have the potential to inform policy responses.

Researchers:
- Zack Cooper, Yale University
- Fiona Scott Morton, Yale University
- Michael Chernew, Harvard University

Evaluating and Improving Post-Hospitalization Mental Health Follow-Up Care

This study will identify the characteristics associated with receipt of timely follow-up mental health care after a hospitalization or emergency department visit related to mental illness, and it will examine whether follow-up care affects outcomes or spending. Findings have the potential to improve care for this vulnerable and potentially expensive patient population.

Researcher:
Kimberley Geissler, University of Massachusetts Amherst

The Clinical and Economic Impact of Washington State's Oral Anticancer Treatment Access Law

This study will examine how Washington state's oral chemotherapy parity law has affected access, spending, out-of-pocket costs and health outcomes. Findings are expected to be broadly applicable to the 42 other states with similar laws and to inform debate over federal legislation.

Researchers:
- Caroline Bennette, University of Washington
- Scott Ramsey, University of Washington
- Zachary Marcum, University of Washington

Patterns and Determinants of Inappropriate Diagnostic Imaging

This study will describe the magnitude and costs of inappropriate diagnostic imaging and estimate the influence of patient, physician and practice characteristics and of physician self-referrals. Researchers will also examine the impact of cost containment initiatives in Massachusetts, with findings expected to inform future efforts to reduce inappropriate care.

Researchers:
- Gary Young, Northeastern University
- Stephen Flaherty, Northeastern University
- Koenraad Mortele, Beth Israel Deaconess Medical Center and Harvard Medical School

eQuality: Improving LGBT, GNC, and DSD Health Through a Comprehensive Medical School Training Program

This study will develop, evaluate and disseminate a clinical skills training manual for medical students on how to provide high quality care to patients who are LGBT, gender nonconforming or have differences of sex development. This work has the potential

to improve care standards nationwide through broader training on evidence-based practices.

Researchers:
- Susan Sawning, University of Louisville School of Medicine
- Amy Holthouser, University of Louisville School of Medicine
- Carrie Bohnert, University of Louisville School of Medicine
- Laura Weingartner, University of Louisville School of Medicine
- Jennifer Potter, Harvard Medical School

Consumer Directed Health Plan Impact on Low-Value Service Utilization and Spending

This study will examine how enrollment in a consumer directed health plan (CDHP) affects use of and spending for 26 measures of low-value outpatient care. Results should inform discussions of the extent to which the growing use of CDHPs can reduce waste in the health care system.

Researchers:
- Neeraj Sood, University of Southern California
- Rachel Reid, RAND Corp.

LUNGevity Foundation introduces mobile app to help patients manage lung cancer

LUNGevity launched a mobile application designed to make understanding and living with lung cancer less daunting and considerably more manageable.

The new Lung Cancer Navigator mobile app provides lung cancer patients with access to the latest medical and treatment information related to their specific lung cancer diagnosis, and serves as a convenient hub for organizing customized care and support networks, asking questions, describing and tracking symptoms, and managing multiple medications.

The LUNGevity Lung Cancer Navigator app provides tools and forums to help those coping with the disease (including caregivers and support network members) communicate important details in real time, while handling care management needs with efficiency, medical guidance and less stress.

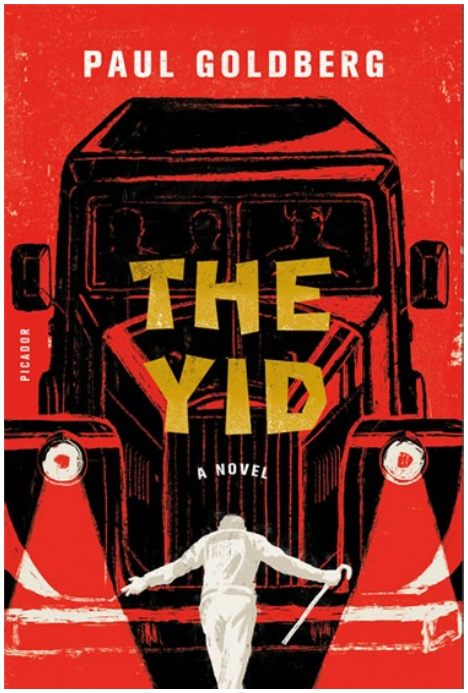
"Our goal with the LUNGevity Lung Cancer Navigator app is to empower patients and provide them with a forum for connecting to customized information and a support community that helps them navigate life with understanding and much less fear," said Andrea Ferris, president of LUNGevity Foundation.

NPR station broadcasts anniversary program on moonshot

On Jan. 12, the first anniversary of the National Cancer Moonshot Initiative, NPR's largest member station, KQED San Francisco, aired a one-hour program, which is posted here.

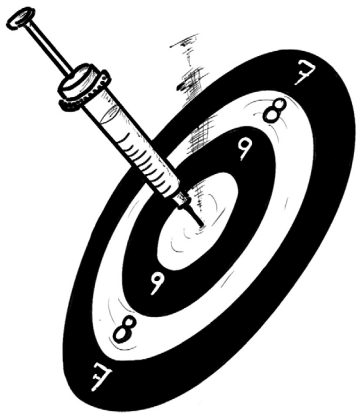
Guests included:
- Matthew Ong, reporter, The Cancer Letter
- Deborah Mayer, professor, school of nursing and director of cancer survivorship, UNC Lineberger Comprehensive Cancer Center at UNC-Chapel Hill; member, Blue Ribbon Panel for Vice President Biden's Cancer Moonshot
- Jan Liphardt, associate professor of bioengineering, Stanford University; co-founder, Cancerbase.org
- Alan Ashworth, president, UCSF Helen Diller Family Comprehensive Cancer Center; senior vice president for Cancer Services, UCSF Health Professor of Medicine

Goldberg novel is finalist in Jewish Book award



Paul Goldberg's novel, The Yid, was a finalist in the Goldberg Prize debut fiction category in the 2016 National Jewish Book Awards. The Yid is a dark comedy set in Moscow in 1953.

DRUGS & TARGETS



Merck joins ORIEN

M2Gen said Merck has joined the Oncology Research Information Exchange Network (ORIEN) Avatar Research Program.

Launched in April 2016, the ORIEN Avatar Research Program fosters collaboration among key stakeholders in cancer research, including patients themselves, with the shared goal of discovering and developing novel therapies and ultimately matching patients to the best treatment options.

ORIEN Avatar is a collaboration between leading U.S. cancer hospitals, pharmaceutical companies and M2Gen, which manages the program. Patients donate clinical and molecular data through their consent to the Total Cancer Care Protocol; that data is then utilized by the ORIEN cancer center members and pharmaceutical partners to speed discoveries and match eligible patients to cutting-edge trials.

The program represents an unprecedented, pre-competitive approach to fighting cancer, designed to accelerate the discovery and development of novel therapies for millions of patients. Merck's participation in the program builds on a history of collaboration dating back to the founding of M2Gen in 2006, to operationalize a multi-year agreement based on the Total Cancer Care Protocol.

The ORIEN Avatar Research Program links pharmaceutical companies and prominent cancer centers.

Participating cancer researchers contribute samples and disease information from patients who provide consent to be studied via the Total Cancer Care Protocol, and receive rich molecular data plus access to a rich network of potential collaborators.

Pharmaceutical companies contribute financial support and receive access to de-identified genetic and disease information that can be used to inform the discovery and clinical development of novel cancer therapeutics.

Scripps to collaborate with Pfizer to advance DNA-encoded library technology

The Scripps Research Institute announced a research collaboration and license agreement with Pfizer Inc. to pioneer new DNA-encoded library technology, including new synthetic chemistry for the creation of next-generation DELs, a potentially transformative technology for early stage drug discovery research.

Under the collaboration, Pfizer will pay a technology access fee and thereby gain access to innovative chemical synthesis technology developed at TSRI.

Members of the TSRI chemistry department—Professors Phil Baran, Dale Boger, Jin-Quan Yu, K. Barry Sharpless, and others—will work alongside Pfizer scientists to adapt these chemical methods for use in creating DELs, which require stringent processes that are tolerant of the delicate DNA backbone.

TSRI and Pfizer may choose to expand the scope of the joint research to include other technologies relevant for enabling DEL-based drug discovery. Financial terms of the agreement are not disclosed.

In contrast to conventional drug screening where a few million small molecules are evaluated in biological systems, DEL screening uses DNA-based "barcodes" to survey billions of small molecules, potentially increasing the ability of researchers to identify promising chemical leads.

While this technology was originally conceived at TSRI by Richard Lerner and Sydney Brenner in the early 1990s, the reduction to practice has taken decades and required technological advances in DNA sequencing and informatics in order to be more fully realized.

AbbVie announces four new global research collaborations

AbbVie announced four collaborations and investments with leading healthcare innovators to advance early-stage research in key therapeutic areas such as oncology and immunology.

Oncology

- **AbbVie** and **Pure MHC**, a privately-held target discovery company, will embark on a research and license agreement to discover and validate peptide targets for use with T-cell receptor therapeutics in several types of cancers.

  The collaboration between AbbVie and Pure MHC will seek to identify a library of peptide targets for further research across multiple tumor types and advance AbbVie's ongoing development of next-generation immuno-oncology therapies.

- **AbbVie** entered into an exclusive license with **Dong-A-ST**, a leading specialty healthcare company in South Korea, for MerTK inhibitors in pre-clinical development for use in conjunction with immuno-oncology therapies.

  MerTK is a protein that is believed to contribute to the promotion of immunosuppressive tumor microenvironment.

  Inhibition of this activity may help promote an inflammatory state, alerting the immune system to attack tumors and augmenting the efficacy of targeted cancer therapies such as checkpoint inhibitors (anti-PD1/PD-L1) and pro-apoptotic agents.

  The collaboration will explore the combination of MerTK inhibitors in conjunction with AbbVie's portfolio of anti-cancer agents across multiple types of solid tumors.

  The addition of this mechanism will advance AbbVie's existing research into immuno-oncology therapies and complement its oncology pipeline under development for nearly 20 cancers and tumor types.

Immunology

- **AbbVie** and **Zebra Biologics, Inc.**, a discovery stage biotechnology company, entered into a partnership to discover agonist antibody therapeutics for inflammatory diseases. Zebra will utilize its novel and patented function-based antibody discovery platform to generate antibodies that activate biological pathways associated with targets designated by AbbVie. Zebra and AbbVie will collaborate closely on the identification and pre-clinical validation of emerging candidates. The targets were not disclosed.

  Zebra will lead the discovery of candidate agonist antibodies for designated targets and will collaborate with AbbVie in pre-clinical validation of select clinical candidates. Upon advancement of clinical candidates, AbbVie would be responsible for clinical development, manufacturing, regulatory approval and world-wide commercialization.

Genomics

- **AbbVie** and **Genomics Medicine Ireland**, a life sciences startup company, partnered to conduct population genomics research in Ireland. The alliance will sequence the genomes of 45,000 volunteers across Ireland. The data to be included will originate from people with several types of immune-mediated diseases, neurological disorders and cancer, as well as people unaffected by these diseases. By incorporating the genotypic and phenotypic data across a wide sample population, the partnership aims to better understand human biology and disease etiology to discover new therapeutic targets and identify biomarkers.

  AbbVie will utilize the research to select targets for drug development, as well as potential development of companion diagnostics for selected conditions.

  GMI is backed by investors ARCH Venture Partners, Polaris Partners, GV (formerly Google Ventures) and the Ireland Strategic Investment Fund.

Lilly and Merck expand immuno-oncology collaboration

Eli Lilly and Co. announced the expansion of an existing immuno-oncology collaboration with to add a new study of Lilly's Lartruvo (olaratumab) with Keytruda (pembrolizumab) in patients with previously treated advanced or metastatic soft tissue sarcoma.

FDA recently granted accelerated approval for Lartruvo (olaratumab injection, 10 mg/mL), in combination with doxorubicin, for the treatment of adults with STS with a histologic subtype for which an anthracycline-containing regimen is appropriate and which is not amenable to curative treatment with radiotherapy or surgery.

Lartruvo (olaratumab injection, 10 mg/mL), in combination with doxorubicin, also recently received conditional marketing authorization from the European Medicines Agency for the treatment of adults with advanced STS not amenable to curative treatment with surgery or radiotherapy and who have not been previously treated with doxorubicin.

Lilly is the sponsor of the phase 1 study and enrollment is expected to begin mid-2017. Other ongoing trials between Lilly and Merck, through a subsidiary, include:

- Studies of pemetrexed (plus carboplatin) and pembrolizumab in

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first-line nonsquamous non-small cell lung cancer, including a phase III study that is currently enrolling patients;

- A phase I study examining the combination of ramucirumab with pembrolizumab in NSCLC, gastric cancer and bladder cancer;
- A phase I study examining the combination of necitumumab with pembrolizumab in NSCLC; and
- A phase I study examining the combination of abemaciclib, a CDK 4 and 6 inhibitor, with pembrolizumab. Based on the phase I trial results, the collaboration has the potential to progress top Phase II trials in patients who have been diagnosed with either metastatic breast cancer or NSCLC.

Amgen and Immatics collaborate to develop cancer immunotherapies

Amgen and Immatics Biotechnologies GmbH announced a research collaboration and exclusive license agreement to develop next-generation, T-cell engaging bispecific immunotherapies targeting multiple cancers.

The collaboration will combine Immatics' XPRESIDENT target discovery and T-cell receptor capabilities with Amgen's validated Bispecific T-cell Engager technology, with the aim of creating novel oncology drugs. Amgen will be responsible for clinical development, manufacturing, and commercialization worldwide.

Under the agreement, Immatics will receive an upfront fee of $30 million and is eligible to receive over $500 million in development, regulatory and commercial milestone payments for each program and tiered royalties up to a double-digit percentage of net sales.

Philips and Illumina to co-develop integrated genomics solutions for oncology

Royal Philips and Illumin Inc. announced a strategic collaboration that aims to integrate Illumina's sequencing systems for large-scale analysis of genetic variation and function and Philips' IntelliSpace Genomics clinical informatics platform, and to coordinate marketing and sales of the resulting solutions. Philips and Illumina will also seek to engage in clinical research collaborations with health systems in the U.S. that want to develop precision medicine programs in oncology.

Profiling tumors using genomic information is critical for complex cancer cases, and next-generation DNA sequencing—the process of rapidly profiling large sections of the genome in parallel to find mutations—is increasingly being used for this. However, challenges remain in developing ways to rapidly and accurately interpret genomic findings in the context of a patient's condition.

While cancer patients can have hundreds of gene variants in their tumors, only a small number may actually drive the individual's specific cancer or may have actionable therapeutic implications for a particular patient. The patient's history, related lab tests and cancer type are needed for a meaningful interpretation of the genomic data.

Philips and Illumina will collaborate to provide new solutions aimed at the acquisition, analysis, annotation, and interpretation of genomics data in oncology cases. The data will be acquired by Illumina's BaseSpace Sequence Hub connected to its instruments and will be processed through Philips' IntelliSpace Genomics solution for oncology. This solution will combine data from multiple sources—radiology, immunohistochemistry, digital pathology, medical records and lab tests—and will deliver a consolidated dashboard view. This system will support researchers to develop insights more efficiently and will ultimately support lowering the cost of health care delivery and improved health outcomes.

The two companies intend to collaborate on system integration, cohort analysis and health economics applications, and future research programs. Laboratories adopting the solution will be able to integrate sequencing data with information from multiple data sources (e.g., imaging, pathology and laboratory). The Illumina-Philips solution will also give them ready access to advanced analytics, deep learning technologies and available reference literature, guidelines, and evidence in a single view.

Tecentriq receives priority review for treatment of urothelial carcinoma

Genentech said FDA accepted the company's supplemental Biologics License Application and granted Priority Review for Tecentriq (atezolizumab) for the treatment of people with locally advanced or metastatic urothelial carcinoma who are ineligible for cisplatin chemotherapy, and are either previously untreated or have disease progression at least 12 months after receiving chemotherapy before surgery or after surgery.

Urothelial carcinoma accounts for 90 percent of all bladder cancers and can also be found in the renal pelvis, ureter, and urethra.

"In May 2016, Tecentriq became the first treatment approved by the FDA for people with previously treated advanced bladder cancer in more than 30 years," said Sandra Horning, chief medical officer and head of Global Product Development. "We are committed to continue working with the FDA to make Tecentriq available to more people with this type of advanced bladder cancer, specifically those who are unable to tolerate cisplatin-based chemotherapy as an initial treatment."

This sBLA submission for Tecentriq is based on results from the Phase II IMvigor210 study, and the FDA will make a decision on approval by April 30, 2017. A Priority Review designation is granted to medicines that the FDA has determined to have the potential to provide significant improvements in the safety and effectiveness of the treatment, prevention or diagnosis of a serious disease. Tecentriq is currently approved by the FDA to treat people with locally advanced or mUC who have disease progression during or following platinum-based chemotherapy or whose disease has worsened within 12 months of neoadjuvant or adjuvant platinum-based chemotherapy.

Tecentriq is approved under accelerated approval for this indication based on tumor response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. Tecentriq is also approved for the treatment of people with metastatic non-small cell lung cancer who have disease progression during or following platinum-containing chemotherapy, and have progressed on an appropriate FDA-approved targeted therapy if their tumor has EGFR or ALK gene abnormalities.

IMvigor210 is an open-label, multicenter, single-arm phase II study that evaluated the safety and efficacy of Tecentriq in people with locally advanced or mUC, regardless of PD-L1 expression. People in the study were enrolled into one of two cohorts. Cohort 1, upon which this sBLA submission is based, consisted of people who were ineligible for first-line cisplatin-based chemotherapy, and who had received no prior chemotherapies for locally advanced or mUC (i.e., first-line) or had disease progression at least 12 months after neoadjuvant or adjuvant chemotherapy.

Cohort 2, which served as the basis for the FDA's accelerated approval of Tecentriq in May 2016, included people whose disease had progressed during or following previous treatment with a platinum-based chemotherapy regimen, or who had disease progression within 12 months of treatment with a platinum-based neoadjuvant or adjuvant chemotherapy regimen. The primary endpoint of the study was objective response rate. Secondary endpoints included duration of response, overall survival, progression-free survival and safety.

EMD Serono and MD Anderson enter three-year collaboration

EMD Serono, the healthcare business of Merck KGaA, Darmstadt, Germany, and MD Anderson Cancer Center announced a three-year strategic collaboration, with the aim of more quickly advancing the development of investigational cancer therapies in four cancers--breast, colorectal, glioblastoma and leukemia.

EMD Serono will be the first company to gain access to the Adaptive Patient-Oriented Longitudinal Learning and Optimization Platform (APOLLO)—MD Anderson's research platform that standardizes the long-term collection of patients' medical history and data derived from tissue samples to better understand the biology of cancer and accelerate research-driven patient care.

The collaboration will encompass both biomarker-focused pre-clinical research and clinical trials in specific tumor types aimed at identifying biomarkers of response and resistance and developing a better understanding of the disease biology.

The collaboration will enhance the value of EMD Serono's future oncology/immuno-oncology pipeline, with a goal of multiple registrational studies in novel indications in the next two to three years. Data from APOLLO will be used to match a number of investigational compounds to select tumor types for potential development and collaboratively design biomarker-driven pre-clinical and clinical studies at MD Anderson evaluating the potential therapeutic effect of the compounds—alone or in combination.

APOLLO was developed by MD Anderson as part of its Moon Shots Program, an ambitious effort to reduce cancer deaths by more rapidly developing and implementing advances in prevention, early detection and treatment based on scientific discoveries.

MD Anderson and Deerfield Management create company to inhibit autophagy

Vescor LLC, a new company focused on discovery and development of autophagy targeted therapeutics for cancer treatment, was formed by MD Anderson Cancer Center, Deerfield Management and two autophagy experts, Eileen White, deputy director and associate director for Basic Science, Rutgers Cancer Institute of New Jer-

sey, and Alec Kimmelman, chairman, Department of Radiation Oncology at NYU Langone Medical Center and a member of the Perlmutter Cancer Center at NYU Langone.

Vescor, advised by its scientific founders White and Kimmelman, will develop small molecule inhibitors of a number of protein targets at critical nodes of the autophagy cascade, perform investigational new drug enabling studies, and move these into clinical development.

MD Anderson's Institute for Applied Cancer Science, in combination with Deerfield, will provide drug discovery and development expertise, together with translational research focused at advancing autophagy therapeutics into trials in melanoma, lung and pancreatic cancers.

Vescor's core activities will be performed at IACS, while managerial and operational expertise will be provided by both IACS and Deerfield.

Cologuard earns positive review from Blue Cross Blue Shield Association

Exact Sciences Corp. announced that the Blue Cross Blue Shield Association's Center for Clinical Effectiveness "Evidence Street" recently released a positive review of Cologuard to its members.

This continues the positive momentum for Cologuard, as coverage increased by 67 percent in 2016 and nearly 163 million Americans are now in health plans that cover the non-invasive colorectal cancer screening option.

"Nearly two thirds of the Blue Cross and Blue Shield companies already cover Cologuard," said Kevin Conroy, Exact Sciences' chairman and CEO. "This review affirms that Cologuard has a positive impact on health outcomes and provides additional support for the remaining plans to offer their members our patient-friendly, non-invasive colon cancer screening option."

The BCBSA's Center for Clinical Effectiveness is an organization that assesses the effectiveness of medical devices, procedures, and biological products through comprehensive reviews and clinical evidence. The Evidence Street assessment follows other positive reviews of Cologuard, which is now included in the recommendations of the U.S. Preventive Services Task Force, and the colorectal cancer screening guidelines of the American Cancer Society and the National Comprehensive Cancer Network.

More than 70 percent of Cologuard's addressable population is now in a health plan that covers the test, the company said. Coverage expanded by 62 million lives since June 2016, when Cologuard was included as an A-graded test in the U.S. Preventive Services Task Force's final colorectal cancer screening recommendations.

FDA accepts BLA for Mylan and Biocon's biosimilar Trastuzumab

Mylan N.V. and Biocon Ltd. said FDA has accepted Mylan's biologics license application for MYL-1401O, a proposed biosimilar trastuzumab, for filing through the 351(k) pathway.

This product is a proposed biosimilar to branded trastuzumab, which is indicated to treat certain HER2-positive breast cancers. The anticipated FDA goal date set under the Biosimilar User Fee Act (BsUFA) is Sept 3.

Mylan and Biocon's proposed biosimilar trastuzumab is also under review by the European Medicines Agency.

Mylan and Biocon are exclusive partners on a broad portfolio of biosimilar and insulin products. The proposed biosimilar trastuzumab is one of the six biologic products co-developed by Mylan and Biocon for the global marketplace.

Mylan has exclusive commercialization rights for the proposed biosimilar trastuzumab in the U.S., Canada, Japan, Australia, New Zealand and in the European Union and European Free Trade Association countries. Biocon has co-exclusive commercialization rights with Mylan for the product in the rest of the world.

FDA releases guidance on nonproprietary naming of biologics

FDA released the final guidance for industry "Nonproprietary Naming of Biological Products."

The guidance describes the agency's thinking on the need for biological products previously and newly licensed under the Public Health Service Act (PHS Act) to bear nonproprietary names that include an FDA-designated suffix.

Under this naming convention, FDA will designate a distinguishing suffix that is devoid of meaning and composed of four lowercase letters in the nonproprietary names for originator biological products, related biological products, and biosimilar products. The suffix will be attached to each product's core name with a hyphen.

FDA is continuing to consider the appropriate suffix format for interchangeable products.