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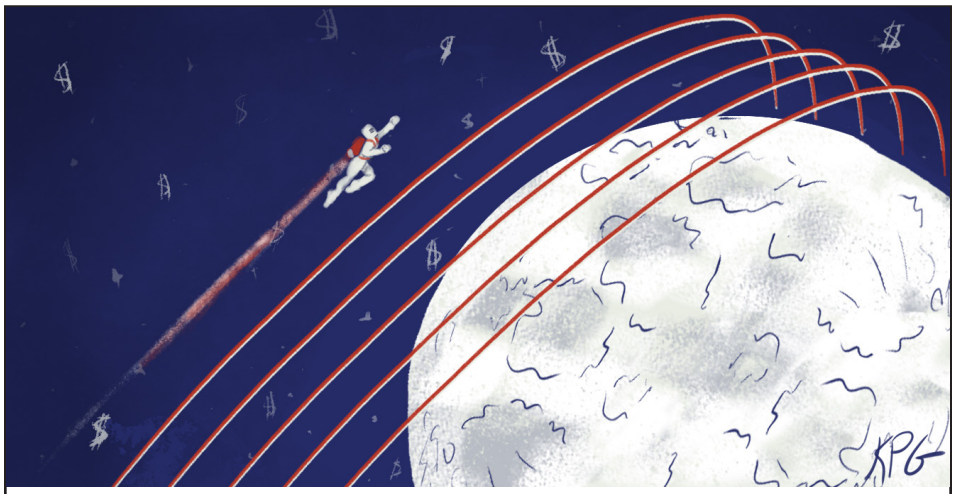

Parker Bets $250 Million on Immunotherapy

By Paul Goldberg and Matthew Bin Han Ong

A foundation established by Silicon Valley entrepreneur Sean Parker—founder of Napster and first president of Facebook—has committed $250 million to research in cancer immunotherapy.

The newly founded Parker Institute for Cancer Immunotherapy brings together immunologists from Memorial Sloan Kettering Cancer Center, Stanford University, UCLA, UCSF, MD Anderson Cancer Center and the University of Pennsylvania.

(Continued to page 2)

Conversation with The Cancer Letter

Parker Mantra: Collaborate Like Hell

The Cancer Letter invited Jedd Wolchok, associate attending physician and chief of the Melanoma and Immunotherapeutics Service at Memorial Sloan Kettering Cancer Center, to describe the workings of the just-announced Parker Institute for Cancer Immunotherapy.

(Continued to page 3)

Slamming the Door

Part X - Silencing Faculty Voice

By Paul Goldberg

In the fall of 2012, just before Al Gilman's departure, MD Anderson officials cracked down on internal critics.

On Sept. 26, 2012, Raphael Pollock, head of MD Anderson's Division of Surgery, was summoned to the office of Thomas Burke, then the executive vice president and physician-in-chief, and was relieved of his duties.

(Continued to page 5)

Parker Bets $250 Million On Immunotherapy

(Continued from page 1)

"We hope to raise additional funds, both by philanthropy and partnerships with industry and government," said Jeff Bluestone, president and CEO of the Parker Institute and the A.W. and Mary Margaret Clausen Distinguished Professor at the University of California, San Francisco.

"Also, we expect to expand the number of investigators involved in the institute," Bluestone said to The Cancer Letter.

The initial amount of funding is $10 million to $15 million per center—about $60 to $90 million in the first year—and it will grow over seven years as the program gets going.

This level of spending on research establishes the new foundation as either the second or third largest private funder of cancer research—and the single largest funder of cancer immunotherapy research.

In 2015, the American Cancer Society spent about $150 million on research, the Leukemia and Lymphoma Society spent about $67 million, Susan G. Komen spent about $54 million, and the Prostate Cancer Foundation spent about $32 million.

Parker's objective is to unify the research programs, intellectual property licensing, data collection and clinical trials across multiple centers under the umbrella of a single non-profit biomedical research organization.

"The idea is to push people to take risks and to collaborate," said Jedd Wolchok, head of the Parker-funded center at MSKCC. "The really important message that we got from the beginning was, 'Collaboration is key.' Of course, that's not new, the idea that team science is very important. Stand Up To Cancer [and] many organizations have been focusing on team science—but here, it's a mantra: 'Collaborate like hell.' It's time to come out of the siloes and to make progress together.

"The really novel part of this is the idea that some quality sites were identified, were given a very generous donation by someone who does study the field, but the researchers are set loose to form their own agenda."

A conversation with Wolchok appears on p. 1.

The institute unites over 40 laboratories, more than 300 researchers and over 30 industry partners.

The directors of the six Parker-funded centers are:

• **Jedd Wolchok**, associate attending physician and chief of the Melanoma and Immunotherapeutics Service at MSKCC.

• **Crystal Mackall**, professor of pediatrics and of medicine, associate director of the Stanford Medical Institute, co-medical director of the Stanford Laboratory for Cell and Gene Medicine and program leader in pediatric cancer immunotherapy.

• **Antoni Ribas**, professor of medicine, professor of surgery and professor of molecular and medical pharmacology at UCLA.

• **Lewis Lanier**, professor and the Microbiology and Immunology Chair at UCSF.

• **Carl June**, the Richard W. Vague Professor in Immunotherapy, director of the Center for Cellular Immunotherapies, and director of translational research at the University of Pennsylvania Abramson Cancer Center.

• **James Allison**, professor and chair of immunology at MD Anderson Cancer Center.

A roster of scientists involved in the program is available here.

Parker's initiative is part of what appears to be a growing trend by philanthropists and the federal government to fund focused forays into cancer research.

Parker's goal is to fund a multi-institutional approach to immunotherapy. At the same time, billionaires Michael Bloomberg and Sidney Kimmel, as well as a group of other donors last month, committed $125 million to establish an immunotherapy research center at Johns Hopkins.

Nike co-founder Phil Knight and his wife Penny gave $500 million to the Oregon Health & Science University to create the first large-scale program dedicated to early detection of lethal cancers. OHSU matched the Knights' gift (The Cancer Letter, June 26, 2015).

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The federal government's $1 billion cancer moonshot initiative appears to be aiming at a broad range of targets. NCI recently announced the appointment of 28 members to a blue ribbon panel charged with completing a plan by the end of the year—three weeks before inauguration of the next president.

The federal initiative gives $195 million to NIH in 2016. The fate of the remaining funds—$680 million for NIH; $75 million for FDA and $50 million for DOD—will be determined by who wins the presidential election and what Washington will look like in 2017 (The Cancer Letter, April 1; Feb. 26; Feb. 12; Jan. 22; Jan. 15).

"Sean and the Parker Institute are, I believe, setting a bold new standard for how philanthropy can really move the needle in serious ways for patients," said Ellen Sigal, chair and founder of *Friends of Cancer Research and a member of the Parker Institute for Cancer Immunotherapy Strategic Advisory Group," said Otis Brawley, chief* medical officer of the American Cancer Society.

Parker has "drafted an all-star team of immunotherapists," Brawley said. "I am very glad to see something like this happen. My only concern is when I look at the future of cancer treatment, I see immunotherapy as part of the answer, I see targeted genomic therapy as part of the answer, I see targeted metabolic therapy as part of the answer, and I hope we fund all of these things and don't slight all of them the way immunotherapy has been slighted over the past 25 years."

Conversation with The Cancer Letter

Parker Institute Mantra: Collaborate Like Hell

(Continued from page 1)

Wolchok, director of one of the Parker-funded centers, spoke with Matthew Ong, a reporter with The Cancer Letter.

Matthew Ong: *What was the genesis of this initiative? Whose idea was it and how did it happen?*

Jedd Wolchok: This was really the idea of Sean Parker. Sean, as you know, is a very successful technology entrepreneur, but he also has a very significant interest in immunology, because of some personal allergy issues that he had, and also because of his friendship with Laura Ziskin, the founder of Stand Up To Cancer.

He became very close to her during the time that she had recurrent breast cancer, and educated himself as to different treatment modalities. He became fascinated with immunotherapy, and as a result of that, he started to look at the entire cancer research process as sort of an outsider looking in—as someone who lives in a different world observing this process.

As someone who is—in his own words—fascinated with "disruptive technology," he decided to identify ways in which the process of doing cancer research can be made more efficient, and if there was any way for him to financially support the research process, most specifically in immunotherapy.

I think the emphasis on immunotherapy is probably because of his own personal interest in immunotherapy, but also because it is the sort of disruptive technology in cancer medicine. It looks at the cancer problem from the other side—it looks at it from the patient side rather than from the "What can we do to stop the tumor from proliferating?" side. It fits with his focus.

When he first became interested in immunotherapy, it was more outside the mainstream than now—it's widely considered to be an additional pillar of standard cancer therapeutic approaches. But when Sean first became focused, there were no approved checkpoint-blocking antibodies. There was very little research on CAR T-cells—Chimeric Antigen Receptor T-cells—that this could have the activity that we now know they do. I think that it was certainly more outside the mainstream.

I think that this was probably about six years ago, when he began to try and help Laura Ziskin. I first met Sean almost three years ago, and at that point, he had already made his decision that he wanted to support immunotherapy. At that moment, it wasn't clear to me what the institute would look like. He had made a sizable contribution to SU2C to support the Immunotherapy Dream Team. He also had made a donation to the Cancer Research Institute, which is an organization that's solely focused on immunotherapy. The idea to actually have his own institute really began to gel two, two-and-a-half years ago, in my experience.

MO: *How is the $250 million divvied up? Who are the recipients, and what are the amounts awarded? How is MSKCC going to be a part of it?*

JW: There are six home institutions. In addition to the six Parker Institute sites—MSKCC, MD Anderson Cancer Center, University of Pennsylvania, Stanford University, University of California, San Francisco, and University of California, Los Angeles—there are also extramural member researchers. Individual researchers whose work is thought to be synergistic, co-complementary with the other sites will be supported singularly.

Some of those folks are at Mount Sinai Hospital and Washington University, specifically Bob Schreiber [director of the Center for Human Immunology and Immunotherapy Programs at the Washington University School of Medicine], Nina Bhardwaj [director of immunotherapy and the medical director of the Vaccine and Cell Therapy Core facility at Icahn School of Medicine at Mount Sinai], and Jeff Hammerbacher [chief scientist and cofounder at Cloudera, and assistant professor of genetics and genomic sciences at Mount Sinai].

An initial funding of $10 to 15 million in the first year was given to set up the Parker Institute centers' sites. This investment will continue to grow on an annual basis via additional project grants, shared resources and central funding. That's to really put infrastructure in place and to fund projects that are germane to the goals of the Parker Institute internally. This is not contract research. The individual experiments are the experiments that the funded researchers at the individual sites want to do, but there are some overall streams of research focus. And obviously, the institute itself will have some money that they'll use and distribute to support institute-wide efforts.

There will be other funds coming from Parker Central, the headquarters in San Francisco, to support global initiatives amongst the six sites. For example, at MSKCC, we launched the first Parker coordinated clinical trial last week, which is a checkpoint blockade trial in melanoma to determine what the best therapy is at the point of resistance to PD-1 blockade. Parker Central, not the individual sites, is paying for the central coordination of the trial.

That trial is actually a very good example of how this institute can accelerate research. That trial was written by Claire Friedman, an MSKCC oncology fellow, and I, and she refined it at the American Society of Clinical Oncology and American Association for Cancer Research clinical trial workshop last summer. It was approved for provision of free drugs by Bristol-Myers Squibb in the fall—it went through all regulatory review and was released as being exempt by the FDA and opened last week.

Ordinarily, to open a trial like that, I would have to apply for grants to pay for the research, to pay for the data management, to pay for the correlative science—especially research biopsies, which are an important part of a biomarker-heavy clinical trial like this. Because I apportioned part of our yearly budget to pay for this, to have those things immediately available, we wouldn't have to go and seek out additional funding. We just hit the ground running immediately.

Now, we can do this at potentially six sites rather than one. We will get done much faster. The number of patients is the same—we've powered it specifically, but it's how quickly you can get those patients treated and get a conclusion. This is really about answering the right question as quickly as with as much depth as possible.

The other attractive aspect of the Parker Institute is that there's a lot of technology that's coming of age in terms of ability to interrogate single-cell specimens, subtle immunologic changes, multiplex analytics, and each site is now getting access to state-of-the-art equipment with which to ask those questions. We can now ask such questions in a non-overlapping way, but each site can dive into the communal specimens in a more in-depth way. I think this trial epitomizes really what the strengths of the network are.

MO: *You mentioned CAR-T and checkpoint inhibitors—could you explain the research goals in greater detail?*

JW: The three basic research streams were arrived at by the center directors and researchers, and the Parker staff. This is not someone telling us what to do, this is us coming up with essentially a research agenda that we wanted to lay out. The three areas are:

• Identifying the optimal way to adoptively transfer T-cells—CAR-T, transgenic TCR-bearing cells, etc.

• Understanding resistance—the PD-1 blockade and hopefully that will feed forward into what to add the PD-1 blockade to overcome or bypass that resistance, and

• Studying deeply the neoepitope-based vaccine concept—we're very aware now that the immune system can see the products of mutated genes that occur in cancer, either drivers or passenger mutations, and how to identify, most importantly, the qualities or features of mutations that are most interesting to the immune system.

It is really a very current area that needs significant attention, and plays to the strengths of the institute, because of the background of many of the founders of the institute in terms of bioinformatics and big data. Jeff Hammerbacher and Sean Parker, in fact, have very significant experience and knowledge in Big Data, and can help get through this large amount of valuable data from patient specimens as effectively as possible.

MO: *So basically this initiative is an evidence-driven endeavor by cancer researchers for cancer researchers.*

JW: The idea is to push people to take risks and to collaborate. The really important message that we got from the beginning was, "Collaboration is key." Of

course, that's not new, the idea that team science is very important. Stand Up To Cancer, many organizations have been focusing on team science—but here, it's a mantra: "Collaborate like hell." It's time to come out of the siloes and to make progress together.

The really novel part of this is the idea that some quality sites were identified, were given a very generous donation by someone who does study the field, but the researchers are set loose to form their own agenda. The other part is the intellectual property-sharing model. This is a bit of an experiment in what is hoped to be an evergreen foundation or institute where the IP generated by the sites and supported researchers is shared between the six home institutions and the Parker Institute.

The institute will redistribute whatever royalty through licensing agreement, etc., is generated back to the individual laboratories. It's a feed-forward evergreen model, which is an experiment. We hope it works, because this is a really generous gift. As you probably know, doing cancer research, especially when it involves clinical trials, can have a lot of zeros involved in the numbers. This is a really large amount of money, but it could get used up without an evergreen approach. That really is quite a visionary and innovative spin.

MO: *Are the funds only going to be used for basic research, or are there industry partnerships in the works?*

JW: It's both. Yes, there are plans to collaborate with pharma to both get access to agents of interest, to combine with standard available agents, and also to have dialogue about new targets that we may discover.

But I think if you look at the team, they are both basic immunologists—some of the best ones in the world—as well as translational scientists and clinicians. The idea here is to, in a quite literal way, bridge the laboratory with the clinic and have a bidirectional transfer of information. It's both at once, not in phases.

MO: *A good number of initiatives launched this year—by the White House, for instance—also focus on immunotherapy and precision medicine, and the "Moonshot" brand seems to really be in vogue in oncology. Was there a conscious decision to not call this a moonshot?*

JW: I don't think we really spent much time thinking about whether to call it a moonshot or not. I think the goal of this seems, in some ways, very aligned with the other very important initiatives, and we're really glad to see all of the interest in the field. It's very firm evidence for how immunotherapy has taken on a major role in cancer research today, and I think there are some similarities, some differences.

The good news is that we're not constrained to only work with these sites. This is one part of what we do and we have ongoing collaborations with, say, Johns Hopkins. Through other funding mechanisms, we at Memorial co-lead the Stand Up To Cancer KRAS mutated non-small cell lung cancer Dream Team, focused on innovative research and care for lung cancer, and work with some other Parker Institute sites as well as others, which currently are not. This is really a situation where more is better, and the more attention we all can pay to this, the more quickly we will make progress. I know that may sound trite, but I think that's really true.

MO: *Is the foundation planning on working with others beyond the academic cancer center realm, including federal entities like NCI and NIH?*

JW: Right now, we're focused on working with these individual sites, but nothing has been written out. The institute is focused on identifying the most important folks in the field and working with them. I think it's a very dynamic structure.

This is really focused on immunotherapy pretty singularly. I think that we recognize that immunotherapy may need to be combined with other therapeutic interventions to achieve optimal results, but I think the questions are being framed around, "What can we do with immunotherapy alone or in conjunction with other approaches?"

Slamming the Door

Part X - Gilman's Departure & Crackdown at MD Anderson

(Continued from page 1)

Pollock, who is Jewish, was fired on Yom Kippur, the Day of Atonement.

Sources told me that Pollock was told he was fired because his division was inconsistent in meeting financial targets. Indeed, MD Anderson financial data showed that surgery produced revenues that were $5 million below budgetary projections, underperforming by 33.2 percent.

This financial performance was caused in part by changes in CPT codes for surgery, which were published in November 2011, three months after MD Anderson finalized its budget, sources said.

Surgical facilities at the institution were running at full capacity—additional operating rooms would be required to add revenues.

"I'm grateful for Dr. Pollock's commitment to leading the division for the last 15 years, and I'm pleased

that he will continue making contributions as a professor with joint appointments in the departments of Surgical Oncology and Molecular and Cellular Oncology," Burke wrote in an Oct. 1 email to the MD Anderson staff.

The word of the incident spread immediately.

People I talk with often were recruiting members of MD Anderson's faculty, and Pollock was one of the biggest stars out there.

Starting immediately, many of the top academic cancer centers started putting together proposals to lure Pollock, who is also the program director and principal investigator of an $11.5 million five-year Specialized Program of Research Excellence grant for translational research in sarcoma. The grant is held by the Sarcoma Alliance for Research Through Collaboration, which meant that the money would move with him.

Indeed, after 31 years at MD Anderson, Pollock landed on his feet, becoming a professor and the director of the division of surgical oncology at The Ohio State University Wexner Medical Center College of Medicine's department of surgery. Pollock also serves as the chief of surgical services of the Ohio State University Comprehensive Cancer Center–Arthur G. James Cancer Hospital and Richard J. Solove Research Institute. He has since become the surgeon-in-chief at James Comprehensive Cancer Center and surgeon-in-chief for the Ohio State University Health System.

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A month after Pollock's firing, the volunteer publishers of Faculty Voice, the MD Anderson faculty blog, were told that anonymous bloggers would henceforth need to submit their names to the blog administrator, who would keep them on file.

The blog allowed faculty members to say exactly what they wanted without fear of retaliation. Many posts focused on the faculty's quality of life—working too hard to meet the quotas. There were also complaints about the style of new management.

The faculty used it to share news and opinions—and to blow off steam. It made for entertaining reading for insiders—and for reporters. Besides, some executives at MD Anderson regarded Faculty Voice as a great resource. The blog made it possible to keep an eye on the mood of the faculty.

When I called the press office, I was told that the administration was enforcing an existing MD Anderson policy, which required that the blog contributors identify themselves to the blog administrator, who would then be able to post the contributions anonymously. I was told that this is done to ensure safety. Imagine if someone posts a bomb threat—but also so that people who represent themselves as faculty members actually are.

By that time, Len Zwelling was taking it for granted that his career at MD Anderson was over, and with his posts on the blog, he was likely speeding up the process. Some of his zingers were published on the blog pseudonymously, i.e. as Moonshot Marvin, but increasingly Zwelling signed his own name, and he was not at all reticent to be quoted in the Houston Chronicle and The Cancer Letter. Prior to him being quoted, I administered something similar to informed consent, reminding him that this can't possibly help his career.

"Obviously, my posts are usually not anonymous, but I have sought the ambiguity of anonymity on occasions when I felt the opinions I expressed might be offensive to some folks with power over me. This is in spite of the fact that I believe the most important American right of all is the power to offend others," Zwelling wrote in a post Oct. 29, 2012. "We would be a lesser country without the Will Rogers, George Carlins, Shelly Bermans, Lenny Bruces, Richard Pryors and Sara Silvermans of the world and MD Anderson would be a lesser place without strong, opinionated people expressing their outrage at the behavior of their colleagues or their unease with wrongs being perpetrated upon them. To me, these people are what make MD Anderson and the United States great and we will all be less for the loss of a forum for their expressing their opinions, even if they are anonymous. I am not sure what the administration fears about anonymous expressions of feelings or thoughts, but clearly these are a threat when reduced to an internal blog.

"It has been fun writing these posts and especially fun hearing from you on-line or in person about what I have written. I liked those disagreeing with me the most as I learned the most from them. And those of you who have stopped me in the hall and encouraged me, I really want to thank a great deal. As a writer, I have learned that you never really know if anyone is reading what

you write, so thank you so much for speaking up, even if you disagree with a position I have taken."

"Whatever happened to academic freedom? What ever happened to freedom itself?"

Ultimately, Zwelling's seven-year contract was not renewed the following year. He said he was offered another year of employment for signing a non-disparagement agreement, but he declined.

Now, he runs a widely read blog which focuses on MD Anderson.

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"This isn't just about academic freedom or freedom of speech. It's also about freedom from fear," Warren Holleman, the founder of Faculty Voice, posted Oct. 27, 2012. "When I came to MD Anderson three years ago, my job was to assess the health and well-being of the faculty. One of my most vivid impressions of the organizational culture was the fear factor and its impact on individual morale, job satisfaction, and the relationship between faculty and administration. I wish I could say that things are better now, but that just isn't the case."

Holleman, who started the blog when he arrived at the institution three years earlier, is a professor at the Department of Behavioral Science and director of the Faculty Health & Well-Being Program.

"One of my objectives in starting the Faculty Voice was to reduce this fear and thus improve faculty health, well-being, and morale by creating a safe place for conversation about faculty concerns. Call me naïve, but I believed that as we faculty expressed concerns about particular problems and issues, our friends on the administrative side would join the conversation by acknowledging our concerns, expressing empathy, presenting their perspectives, and engaging in a solution-focused dialogue. That hasn't happened, at least here in the blog, so in that sense the Faculty Voice has failed.

"Instead, many of my friends on the administrative side view the blog as a place where a small contingent of disgruntled faculty vent, gripe, and whine. They assume that these views do not represent the 'silent majority' of our faculty. To those on the administrative side I would say this:

"'I believe you are wrong. I think the concerns expressed in this blog are representative of the faculty. Last year I interviewed 19 of our department chairs, most of whom sounded an alarm about low faculty morale. The recent morale survey by the Faculty Senate will be published soon, and it will offer additional insight. I think you are also wrong in another respect. Not only do you tend to caricature the faculty, but you also tend to caricature the blog. I have observed that, for many of you, your knowledge of the blog is based on reading only the most extreme posts and comments that you email to each other. If you logged onto the blog and read more representative samples, you'd see that we focus mostly on solutions, not problems. You'd see that we love this place as much as you do and are just as committed to its success—if not more so.'

"As moderator of the Faculty Voice, I wish I had done a better job of 'selling' the blog to the administration in general and the executive leadership in particular. This did not happen, but there are many other good ways to reduce the fear factor and to improve health, well-being, and morale.

"Let's get together, and let's get it done."

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In the fall of 2012, it was an open secret that DuBois, too, was looking for a job.

Indeed, he left MD Anderson on Dec. 31, 2012 to become the executive director of the Biodesign Institute at Arizona State University.

In a letter to the editor, which appears in this issue of The Cancer Letter, DuBois described the CPRIT controversy as a "brief, awkward blip in my career."

"Taking the long view, it's rare that anyone in academic medicine doesn't hit a rough patch, and it pales in comparison to the kinds of rough spots cancer patients deal with all the time," DuBois writes.

Later, as the CPRIT crisis continued to unfold, an effort was made to recruit DuBois to run a Texas state agency as a sideline to his job in Arizona.

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When Gilman vacated his office Oct. 12, 2012, his departure was followed with the sort of fireworks rarely observed in science.

The Nobel laureate and his friends used the departure as a teachable moment, an opportunity to demonstrate to Texas politicos that scientists can be pushed only so far.

In the process, they demonstrated what the universe beyond the breaking point looks like.

Next Week: Gilman's exit strategy plays out.

Letter to the Editor

Former MD Anderson Provost Reflects on "Brief, Painful Episode"

By Raymond DuBois

Over the past several weeks, The Cancer Letter has been running a series of articles that report on a past conflict between people at The University of Texas MD Anderson Cancer Center and Nobel Laureate Al Gilman, who led the scientific review teams of the then newly formed Cancer Prevention and Research Institute of Texas.

At the time of the controversy, I was the founding provost and executive vice president at the MD Anderson Cancer Center, a position I enjoyed greatly. While I have no desire to revisit this brief, and somewhat painful episode in my academic career, I have been written into Goldberg's Texas drama as an important bit player and therefore feel compelled to go on record and provide my view of the story.

First and foremost, I was thrilled to be involved in the early days of CPRIT when the agency was finding its sea legs in terms of funding cancer research that would have a transformational impact. Our president at the time, John Mendelsohn, wanted MD Anderson to compete well for the CPRIT funds and use those funds to advance the cause against cancer. I introduced myself to Al Gilman shortly after he was appointed and let him know that I would take the lead in organizing the CPRIT applications from MD Anderson and offered to do anything I could to help him in the process.

Over the next several months we came to know and respect each other and communicated several times each month. Dr. Gilman basically wanted CPRIT to support the highest quality science and avoid an avalanche of less-than-stellar applications that might clog the system. The scientific advisory committee he assembled was one of the finest in the nation—in fact, world-class—and the system he set up worked extremely well. CPRIT funded, and continues to fund, top-notch cancer research that makes a difference in the prevention and treatment of this insidious disease.

When Dr. Gilman decided to resign from the organization, I was still at MD Anderson and, at one point, was even approached about considering replacing him as CPRIT's chief scientific officer. However, by that time I had accepted a position as the executive director of the Biodesign Institute at Arizona State University, so I was not an appropriate candidate to lead that organization.

Fortunately for all involved, Margaret Kripke stepped in and was able to get CPRIT back on track. She was a much better choice than I would have been. CPRIT needed a strong scientist with impeccable credentials, who could "herd all the cats," negotiate through the politics, and return the focus to the intractable issues of cancer – which is exactly what Dr. Kripke did.

On the other hand, I remained involved with CPRIT and spent the past three years serving as a scientific advisor for the commercialization grant review committee. I am pleased to have helped support the funding of some truly groundbreaking commercialization grants which will have an economic impact on the state of Texas.

Which brings me back to my original point. As Paul Goldberg revealed through his tape recorded phone interview with me, the CPRIT controversy was a brief, awkward blip in my career. Taking the long view, it's rare that anyone in academic medicine doesn't hit a rough patch, and it pales in comparison to the kinds of rough spots cancer patients deal with all the time.

Plus, I was offered an outstanding opportunity to lead the Biodesign Institute at ASU, which was a unique and mind-broadening experience that allowed me to work with scientists from a wide range of disciplines—medicine, biology, chemistry, physics, botany, astronomy, medicine, evolutionary medicine, nanotechnology, bioengineering, nanocrystallography, biomimicry, vaccinology, bioinformatics—all trying to find answers to the most intractable issues in health, sustainability and biosecurity now facing our planet. I benefited scientifically and professionally from having had this experience.

Recently, I accepted a new position as Dean of Medicine at the Medical University of South Carolina and am thrilled to be overseeing the training of the next generation of doctors, clinical scientists and others in a region of the country important to me and my family, and where I believe I can genuinely make a positive impact. I am very happy with how my career has unfolded, and I have even been able to stay in touch with my friends and colleagues at MD Anderson, Vanderbilt and Hopkins.

The Cancer Letter's decision to publish a historical perspective between Al Gilman and CPRIT has given me a chance to contemplate a few things. First, I'm glad this will give me the opportunity to properly eulogize Al Gilman. He was brash, bombastic and brilliant. He also established an elegant structure for stimulating creative cancer research in Texas, which continues to propel the

field forward in unprecedented ways.

Second, I want to state that MD Anderson's clinicians and physician-scientists are among the best in the world. I still call upon them regularly for referrals when patients present with complex cancer diagnoses. To a person, they have always taken my calls and genuinely care about the overwhelming dilemmas these patients confront. I am especially thankful for their recent superb care of one of my family members.

Also, I must acknowledge the unsung heroism of Margaret Kripke. She realized CPRIT's importance in the fight against cancer, ignored all the background noise and kept the organization afloat and operating successfully. Not many people would have been able to achieve as much.

And finally, I want to express my pride in the people from my home state of Texas. They courageously voted to fund a then-amorphous cancer research organization with $3 billion of their hard earned money in the hope it might crack the cancer code and lead to better treatments. Their faith in CPRIT weathered the ups and downs of several extraneous issues that threatened to derail it. It took real guts to continue to support the initiative.

In fact, CPRIT is alive and well today because even when things got tough, people like Al Gilman, Margaret Kripke and the citizens of Texas refused to back down. In the end, it's cancer patients around the world who will benefit from their foresight, determination and resilience.

Ironically, cancer is the culprit responsible for taking Dr. Gilman's life. I have to believe that some of the research supported by CPRIT under his watch will result in more effective treatments and early detection for pancreatic cancer that will affect generations to come. Part of this future success will come from the highest scientific standards he so vigorously supported.

The author is the dean of medicine at the Medical University of South Carolina.

FDA Inspects Hospitals for Morcellation Harm; Rep. Draws VP Biden's Attention to Issue

By Matthew Bin Han Ong

FDA has conducted inspections of several hospitals—including Brigham & Women's Hospital—based on allegations that physicians and administrators did not report patient harm and deaths resulting from power morcellators.

In a March 29 letter to Rep. Mike Fitzpatrick (R-Pa.), the agency said it "takes these issues very seriously."

"In recent months, we have conducted inspections of hospitals highlighted in your letter, including Brigham & Women's Hospital, Rochester General Hospital, and the University of Rochester Medical Center," FDA officials wrote, responding to a Dec. 18, 2015, letter from Fitzpatrick to the agency's Office of Criminal Investigations.

Under Section 803 of Title 21 of the Code of Federal Regulations, manufacturers and hospitals are required to report adverse outcomes—deaths and serious injuries that a device may have caused or contributed to—to FDA within 10 to 30 days.

In November 2014, The Cancer Letter first reported on Brigham's role in upstaging a patient's leiomyosarcoma via power morcellation performed in 2012. Erica Kaitz, the patient, died on Dec. 7, 2013. In October 2013, another patient, Amy Reed received her cancer diagnosis at Brigham. Reed subsequently led a campaign against widespread use of the procedure (The Cancer Letter, Nov. 21, 2014).

In a study by Brigham physicians Michael Muto and Michael Seidman published November 2012 in PLOS ONE, the authors identify four patients—out of 1,091 patients—who showed evidence of peritoneal dissemination of leiomyosarcoma after undergoing power morcellation. Three of the four patients died, with an average post-diagnosis survival of 24.3 months.

It is not publicly known where the four patients were treated.

Johnson & Johnson subsidiary Ethicon—the largest manufacturer of the devices—said it didn't know of the dangers of power morcellators prior to December 2013, when Reed and her husband Noorchashm filed a Medical Device Report to FDA. Whistleblower Robert Lamparter, a retired pathologist from central Pennsylvania, disagreed, and produced documents from 2006 proving that he had reported to J&J a near-miss case as well as risk estimates similar

to FDA's numbers (The Cancer Letter, Nov. 20, 2015).

FDA said it did not receive any reports of adverse outcomes from manufacturers and hospitals prior to December 2013 (The Cancer Letter, Dec. 18, 2015).

FDA joins three other federal agencies in looking into potential violations of statutory requirements for hospitals and manufacturers to report adverse outcomes: the Federal Bureau of Investigation, the Government Accountability Office and the Subcommittee on Oversight and Investigations of the House Committee on Energy and Commerce.

"In order to protect the integrity of the investigative process, it is FDA's policy not to confirm or deny the existence of a criminal investigation," FDA acting associate commissioner Dayle Cristinzio wrote to Fitzpatrick. "FDA appreciates your interest in this issue and we will continue to keep you and your staff apprised of any updates."

Fitzpatrick: An Example for Moonshot FDA Reform

In a letter to Vice President Joe Biden, Fitzpatrick urged the White House to implement "meaningful" reforms as part of the cancer moonshot initiative, which aims to consolidate FDA's oncology portfolio (The Cancer Letter, Feb. 12).

Medical device reporting regulations need to be strengthened, Fitzpatrick said, and the controversy over reporting of adverse outcomes resulting from power morcellators is one situation where consolidation of cancer expertise at the agency could have life-saving impact.

"As part of the Cancer Moonshot, we have the chance to protect others from the harm caused by dangerous medical devices," Fitzpatrick wrote in the April 14 letter. "President Obama called for the development of a virtual Oncology Center of Excellence within the Food and Drug Administration.

"The purpose of the center is to 'support the continued development of companion diagnostic tests, and the use of combinations of drugs, biologics and devices to treat cancer.' While it is critically important we support the development of innovative new devices and drugs to treat cancer, it is just as important to take this opportunity to implement meaningful reforms to make medical devices safe.

"Unfortunately, for decades, a medical device known as a laparoscopic power morcellator proved tragically unsafe, spread cancer throughout the body of those it was designed to help, and led to the death of hundreds, if not thousands, of women."

The correspondence between Fitzpatrick, FDA and Biden can be downloaded here.

The moonshot program aims to provide $75 million in proposed fiscal 2017 mandatory funds would be used to "leverage the combined skills of regulatory scientists and reviewers with expertise in drugs, biologics, and devices." (The Cancer Letter, Feb. 12.)

An effort is underway to restructure FDA based primarily on therapeutic areas, de-emphasizing the silos of drugs, biologics and devices. This would be done through the creation of FDA Centers of Excellence, which advocates say would improve coordination between FDA medical product centers.

At a recent Capitol Hill briefing, panel members—representing industry, FDA, academic oncology and patient advocacy—agreed that the time has come to reassess the way medical products are regulated at FDA (The Cancer Letter, Feb. 26).

"First of all, there's nothing wrong, and FDA is doing a great job with what they have," said Ellen Sigal, chair and founder of Friends of Cancer Research, the advocacy group that convened a panel discussion to explore a way forward for the regulatory agency.

"People at Center for Devices and Radiological Health, Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research are all doing a great job. But, the question is, can it be better?" said Sigal at the Feb. 24 event. "Patients get diseases, they don't get a biologic; they don't get a device. It all works together. Is there a more efficient way that is better? Because the goal is to get patients better treatments. This is what it's about.

"It isn't about institutional structures; it's not about anyone doing a bad job. Is there an efficient, better way to serve the patient towards the goal of really integrating these centers of people in their particular fields working together?"

The text of Fitzpatrick's April 14 letter to the vice president follows:

Dear Vice President Biden,

I applaud your efforts as part of the cancer Moonshot program to make cancer a thing of the past. This program will have life-changing effects on the more than 14 million people living with cancer in the United States.

As part of the Cancer Moonshot, President Obama called for the development of a virtual Oncology Center of Excellence within the Food and Drug Administration (FDA). The purpose of the center is to "support the continued development of companion

diagnostic tests, and the use of combinations of drugs, biologics and devices to treat cancer."

While it is critically important we support the development of innovative new devices and drugs to treat cancer, it is just as important to take this opportunity to implement meaningful reforms to make medical devices safe.

Unfortunately, for decades, a medical device known as a laparoscopic power morcellator proved tragically unsafe, spread cancer throughout the body of those it was designed to help, and led to the death of hundreds, if not thousands, of women.

Power morcellators are FDA-cleared medical devices that are used to remove uterine fibroids. The blades of this device shred the uterine fibroids, which are then removed through a laparoscopic incision. For over two decades, morcellation was marketed as a safe, routine procedure. However, if a uterine fibroid is harboring an undetectable cancer, the morcellation of that cancerous tissue and its removal through the abdominal cavity can spread that cancer throughout a woman's body. This device can take a Stage 1 treatable cancer immediately to a Stage 4 terminal cancer. And tragically for too many women, this routine procedure ended with a death sentence.

Although the risk of spreading unsuspected cancers in women is as high as 1 in 352 cases, for decades the device stayed on the market, and it was only in November 2014 that the FDA put a black box warning on the device. It took a victim of morcellation and mother of 6, Dr. Amy Reed, to get the FDA take notice. It took the families of those who lost mothers, sisters, and wives, and those who continue to battle the cancer spread by morcellation, to get the FDA to take action.

As part of the Cancer Moonshot, we have the chance to protect others from the harm caused by dangerous medical devices. In 2016 there will be an estimated 595,690 cancer deaths in the United States. We must guard against unsafe medical devices from contributing to those staggering statistics. While supporting the development of new devices to target cancer, we must ensure that the FDA is able to review, monitor, and quickly take action should those devices do harm.

Unfortunately, we are too late for many women who, because of morcellation, were not given a fair chance to fight and beat their cancer. But we have a chance to protect others. We must take action.

There are simple steps we can take today to improve patient safety for tomorrow's beneficiaries of innovative new devices. One simple way we can do this is to ensure that serious injuries and deaths caused by medical devices are promptly reported to the FDA. For years there was evidence that morcellation was spreading cancer in women. But those reports never made it to the FDA. For years women were dying from the cancer spread by this device. But their deaths were never reported to the FDA by the hospitals, the device manufacturers, or the doctors who were charged with their care.

This was despite federal requirements that FDA be informed about unsafe devices. We must do more to strengthen medical device reporting regulations to ensure that dangerous devices, like the morcellator that spread cancer in women for decades, are quickly identified and removed from the market before they can do more harm.

I applaud your leadership on this important mission that will undoubtedly save lives, and look forward to continuing to work with you to help eliminate cancer as we know it.

Please do not hesitate to contact me if I may be of assistance.

Sincerely,

Mike Fitzpatrick

Member of Congress

Stand Up To Cancer Debuts Catalyst Research Program With Merck, BMS and Genentech

Stand Up To Cancer announced Catalyst, a program that will use funding and materials from the pharmaceutical, biotechnology, diagnostic and medical devices industries to accelerate research on cancer prevention, detection and treatment.

Founding collaborator Merck; and Bristol-Myers Squibb, and Genentech, a member of the Roche Group, will serve as charter supporters.

"The Catalyst program is a perfect fit with the SU2C mission of accelerating the pace of groundbreaking translational research that provides new therapies to patients quickly," said Sung Poblete, president and chief executive officer of SU2C. "This will be a nimble program that will help speed up the rate at which we discover what works."

Under the SU2C Catalyst program, companies like Merck, Bristol-Myers Squibb and Genentech will donate funds to support collaborative research studies

using products the companies will provide, such as new pharmaceutical compounds that they are developing or approved agents that can be investigated for other uses.

Through the American Association for Cancer Research, SU2C will issue a request for proposals based on each company's commitment of funding and materials such as drugs and diagnostic tests. The RFP will lay out the compounds that will be available, the research emphasis, the estimated number of projects that will be supported, and funding available.

The SU2C Catalyst program will be overseen by an executive committee chaired by Nobel laureate Phillip Sharp, chair of the SU2C Scientific Advisory Committee and institute professor at the Koch Institute for Integrated Cancer Research at MIT.

Since 2008, SU2C has launched 19 Dream Teams, two Translational Research Teams, and 26 individual Innovative Research Grants, with funds committed by philanthropic, organizational, corporate and individual donors, as well as non-profit collaborators.

In Brief

Sosman to Lead Melanoma Program at Northwestern

JEFFREY SOSMAN will join the **Robert H. Lurie Comprehensive Cancer Center of Northwestern University** as co-leader of the Translational Research in Solid Tumors Program and director of the Melanoma Program, effective May 1. He will also serve as director for faculty development at the Lurie Cancer Center.

Sosman comes from Vanderbilt-Ingram Cancer Center where he has an Ingram Chair for Cancer Research, directs the Melanoma Program and is co-leader of the Translational Research and Interventional Oncology Program. He is a recipient of the first Mary-Hendrickson Johnson American Cancer Society Melanoma Research Professorship.

The TRIST Program consists of accomplished faculty conducting translational studies centered on the themes of molecular and cell biology, early diagnosis, prognosis, risk factors, therapeutics and treatment of cancer of the aerodigestive tract, dermatologic, gastrointestinal, genitourinary and neuro-oncologic cancers.

Sosman has contributed to trials that led to the approval of at least eight new therapeutic agents for melanoma in the past five years. He continues to study new investigational agents to further increase treatment options for patients with melanoma, and is also recognized for his work with malignancies such as renal cell carcinoma.

ERIC DISHMAN was named director of the **Precision Medicine Initiative Cohort Program at NIH.**

Dishman will lead NIH's effort to build the PMI longitudinal research study of one million or more volunteers. Dishman was a member of the working group that helped develop the design for the study.

He succeeds **Josephine Briggs**, who served as interim director of the PMI Cohort Program, while also serving as director of the National Center for Complementary and Integrative Medicine.

Most recently, Dishman served as vice president and Intel Fellow of Intel Corporation's Health & Life Sciences Group, responsible for global strategy, research, platform development, and policy.

ED SAUTER was named director of the breast surgery program at the **Hartford HealthCare Cancer Institute**. He will lead the breast surgery programs at each of the institute's five cancer centers.

Sauter previously served as director of the Cancer Treatment and Prevention Center at the University of Texas Health Science Center at Tyler. Prior to working in Texas, Sauter was a professor of surgery at the University of North Dakota School of Medicine and Health Sciences. He has been practicing in the field of breast surgery for more than 25 years. Sauter will also practice at the HHCCI locations in Hartford and Avon.

MAYA MARTINEZ-DAVIS was appointed global head of the oncology franchise of **Merck KGaA.**

She will be based in Billerica, Mass., and will report to Rehan Verjee, chief marketing and strategy officer of the healthcare business sector.

Martinez-Davis was a senior executive with Pfizer for more than a decade. Prior to her tenure with Pfizer, she held senior-level and director-level positions with Aventis Pharma in oncology. At Merck, she succeeds Andrew Schiermeier.

Martinez-Davis' responsibilities will include defining integrated global oncology strategies, and delivery of therapeutic launches, starting with Avelumab, an investigational anti-PD-L1 antibody initially discovered and developed by Merck and currently managed under a strategic alliance with Pfizer. The first potential commercial launch for Avelumab is expected in 2017.

LYNN MATRISIAN was named the inaugural chief research officer for the **Pancreatic Cancer Action Network**. Matrisian was promoted from vice president of scientific and medical affairs, where she has served since 2011.

Doug Laidlaw was named vice president of scientific and medical affairs, and will be responsible for overseeing the nonprofit's research grants, patient services and clinical initiatives departments.

Matrisian will develop initiatives designed to transform pancreatic cancer outcomes. These initiatives include early detection approaches and the creation of novel biomarker-driven clinical trials.

Matrisian was the founding chair of the department of cancer biology in the School of Medicine at Vanderbilt University, president of the American Association for Cancer Research, and a special assistant to the director of the NCI.

Laidlaw most recently served as president and founder of Summit Scientific Consulting, providing clinical and medical affairs services for start-up and small-scale specialty pharmaceutical and biotechnology companies.

KARIN JOOSS was named chief scientific officer of **Gritstone Oncology**.

Jooss previously served as head of Cancer Immunotherapeutics and Immunopharmacology at Pfizer for seven years.

Mojca Skoberne also joined the company as senior director of immunology. Skoberne most recently served as program lead at Genocea Biosciences, a company that identifies targets of T cell responses.

While at Pfizer, Jooss built and led immuno-oncology teams within the Vaccine Immunotherapeutics department, was a member of the Vaccine Immunotherapeutics leadership team and served as the head of the Immunophamacology team. Her duties included overseeing the assessment of all cancer vaccine in-licensing opportunities, and launching Pfizer's first clinical cancer-vaccine program deploying a variety of vaccine platforms and immune modulators to build a multi-component vaccine-based immunotherapy regimen. Prior to joining Pfizer, Jooss served as vice president of research at Cell Genesys Inc.

THE U.S. PREVENTATIVE SERVICES TASK FORCE published a B recommendation for low-dose aspirin use for the primary prevention of colorectal cancer and cardiovascular disease in adults aged 50 to 59 years who have a 10 percent or greater 10-year CVD risk, are not at increased risk for bleeding, have a life expectancy of at least 10 years, and are willing to take low-dose aspirin daily for at least 10 years.

The USPSTF said physicians should recommend this therapy to their patients in this population.

The task force also issued a C recommendation for use in adults aged 60 to 69 years with a 10 percent or more 10-year CVD risk, saying that the decision should be an individual one. In that population, "persons who place a higher value on the potential benefits than the potential harms may choose to initiate low-dose aspirin," the task force said.

Regarding the possible harms, the task force "found adequate evidence that aspirin use in adults increases the risk for GI bleeding and hemorrhagic stroke. The USPSTF determined that the harms vary but are small in adults aged 59 years or younger and small to moderate in adults aged 60 to 69 years. The USPSTF found inadequate evidence to determine the harms of aspirin use in adults aged 70 years or older," the task force wrote.

The task force said the benefits of colorectal cancer prevention is not apparent until 10 years after aspirin therapy is began: "Patients need to take aspirin for at least 5 to 10 years to realize this potential benefit, and persons with shorter life expectancy are less likely to benefit. Thus, aspirin use is more likely to have an effect when it is started between the ages of 50 and 59 years," the task force wrote.

The USPSTF's full recommendation statement can be found here.

THE INTERNATIONAL MYELOMA FOUNDATION will fund a large-scale screening study for preventing myeloma.

iStopMM, for Iceland Screens Treats or Prevents Multiple Myeloma, will examine blood samples from approximately 140,000 adults over age 40 in Iceland for the earliest signs of myeloma. Nearly all Icelandic citizens over age 40 already have routine blood tests, according to the foundation.

Project leader Sigurdur Kristinsson of the University of Iceland and his team will screen for MGUS (monoclonal gammopathy of undetermined significance) and smoldering myeloma. Those individuals diagnosed with the precursors will then be invited to participate in a randomized clinical trial to identify the best strategy for treatment and to create a risk model for disease progression.

The study's co-principal investigator, Ola

Landgren, chief of the Myeloma Service at Memorial Sloan Kettering Cancer Center, and his team will perform the molecular characterization of MGUS cases based on DNA sequencing of abnormal plasma cells in the bone marrow. Binding Site, a U.K.-based maker of diagnostic assays, will perform the study's initial screening phase, using Freelite immunoassays and automated electrophoresis testing equipment.

"The impact of early diagnosis in a whole population is a very ambitious and challenging goal," said Kristinsson. "With more potent therapies available with fewer side effects, it is very likely that treatment of precursor states will be shown to improve survival and quality of life in smoldering and MGUS patients."

MD ANDERSON CANCER CENTER dedicated the **Sheikh Zayed Bin Sultan Al Nahyan Building for Personalized Cancer Care**.

The 12-story building, now in the second phase of construction, will house the Sheikh Khalifa Bin Zayed Al Nahyan Institute for Personalized Cancer Therapy, the Sheikh Ahmed Bin Zayed Al Nahyan Center for Pancreatic Cancer Research, and molecular diagnostics, histocompatibility and molecular pathology research laboratories.

The dedication was attended by Hamed Bin Zayed Al Nahyan, chairman of the Crown Prince Court of Abu Dhabi; Mohamed Haji Al Khoori, director general of the Khalifa Bin Zayed Al Nahyan Foundation; and H.E. Yousef Al Otaiba, the United Arab Emirates ambassador to the U.S.

The construction was funded by a $150 million grant from the Khalifa Foundation in 2011. The grant also funds three distinguished university chairs and a Faculty Scholar program.

THE UNIVERSITY OF CALIFORNIA, DAVIS and **Novogene** established a genomics sequencing center on the university's Sacramento campus.

Novogene purchased its second Illumina Hi-Seq X Ten system, which can perform whole human genome sequencing for less than $1,000, and will install five sequencers in the new facility, scheduled to open in early May.

The center will provide whole genome sequencing and analysis of human, plant and animal samples for biomedical and agricultural research.

According to the company, Novogene's goal is to establish a CLIA-certified laboratory in the UC Davis facility to enable human genome sequencing for clinical applications as well.

THE UNIVERSITY OF FLORIDA Health Proton Therapy Institute signed a contract with **Ion Beam Applications** to install a Proteus One system and to upgrade its current proton therapy equipment.

The contract also includes an operation and maintenance agreement for the Proteus One system. In total, the contract is worth approximately $30 million to IBA. The project is estimated for completion in 2018.

Drugs and Targets

Accelerated Approval Granted To Venclexta Tablets in CLL

FDA granted accelerated approval to Venclexta tablets (venetoclax) for patients diagnosed with chronic lymphocytic leukemia with 17p deletion, as detected by an FDA-approved test, who have received at least one prior therapy.

The indication was approved based on overall response rate, and continued approval may be contingent upon verification and description of clinical benefit in a confirmatory trial.

The FDA approved Venclexta as a first-in-class, oral, once-daily medicine that selectively inhibits the BCL-2 protein. The BCL-2 protein blocks apoptosis of cells, including some cancer cells, and can be overexpressed in CLL cells.

Venclexta is being developed by AbbVie and Genentech, a member of the Roche Group. It is marketed collaboratively by the companies in the U.S. and by AbbVie outside of the U.S. AbbVie expects Venclexta will become commercially available in the U.S. within a week.

FDA granted priority review for the NDA application of venetoclax as a single agent in January. The FDA also granted venetoclax three Breakthrough Therapy designations: for the treatment of CLL in previously treated patients with the 17p deletion genetic mutation; in combination with rituximab for the treatment of patients with relapsed/refractory CLL; and for patients with untreated acute myeloid leukemia who are ineligible to receive standard induction therapy.

The safety and efficacy of Venclexta was evaluated in an open-label, multicenter clinical trial of 106 previously-treated CLL patients with 17p deletion. In the study, patients with 17p deletion were identified using Vysis CLL FISH Probe Kit.

Patients received Venclexta via a weekly ramp-up schedule starting at 20 mg and ramping to 50 mg, 100 mg, 200 mg and finally 400 mg once daily. Patients continued to receive 400 mg of Venclexta once daily

until disease progression or unacceptable toxicity. The median time on treatment at the time of evaluation was 12.1 months (range: 0 to 21.5 months). Overall response rate was 80 percent. The median time to first response was 0.8 months (range: 0.1 to 8.1 months). Median duration of response has not been reached, with approximately 12 months of median follow-up. The DOR ranged from 2.9 to over 19.0 months.

The safety of single agent Venclexta is based on pooled data of 240 patients with previously treated CLL from two phase II trials and one phase I trial. The most common adverse reactions were neutropenia, diarrhea, nausea, low red blood cell count, upper respiratory tract infection, thrombocytopenia and fatigue.

Serious adverse reactions were reported in 43.8 percent of patients. The most frequent serious adverse reactions were pneumonia, low white blood cell count with fever, fever, abnormal breakdown of red blood cells resulting in low red blood cell count, low red blood cell count, and tumor lysis syndrome. Discontinuations due to adverse reactions occurred in 8.3 percent of patients, and dosage adjustments due to adverse reactions occurred in 9.6 percent of patients.

FDA approved Epi proColon, a blood-based colorectal cancer screening test developed by Epigenomics AG.

Epi proColon will be made available in the U.S. under a joint commercialization agreement with Epigenomics partner Polymedco.

Epi proColon is indicated for colorectal cancer screening in average-risk patients who choose not to undergo colorectal cancer screening by guideline-recommended methods such as colonoscopy and stool-based fecal immunochemical tests. There are no dietary restrictions or alterations in medication required for the test. Epigenomics plans to initiate a post-approval study to show the long-term benefit of blood-based colorectal cancer screening using Epi proColon.

FDA granted priority review for atezolizumab (MPDL3280A) for the treatment of people with locally advanced or metastatic non-small cell lung cancer whose disease expresses the protein PD-L1, as determined by an FDA-approved test, and who have progressed on or after platinum-containing chemotherapy.

"In a study of atezolizumab in people with previously treated advanced lung cancer, PD-L1 expression correlated with how well they responded to the medicine," said Sandra Horning, chief medical officer and head of Global Product Development at Genentech, the drug's sponsor. "The goal of PD-L1 as a biomarker is to identify people most likely to benefit from atezolizumab alone."

Atezolizumab was granted Breakthrough Therapy Designation by the FDA in February 2015 for the treatment of people whose cancer expresses PD-L1 and whose disease progressed during or after standard treatments.

The BLA submission for atezolizumab is based on results from clinical trials including the phase II BIRCH study, and the FDA will make a decision on approval by Oct. 19.

BIRCH is an open-label, multicenter, single-arm study that evaluated the safety and efficacy of atezolizumab in 667 people with locally advanced or metastatic NSCLC whose disease expressed PD-L1. PD-L1 expression was assessed for both tumor cells and tumor-infiltrating immune cells with an investigational IHC test based on the SP142 antibody. People in the study received a 1200-mg intravenous dose of atezolizumab every three weeks. The primary endpoint of the study was objective response. Secondary endpoints included duration of response, overall survival, progression-free survival and safety.