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### DRUGS & TARGETS

# Biden: Trump's cuts would set back cancer research by 15 years

By Matthew Bin Han Ong

A budget proposed by President Donald Trump would set NIH and biomedical research back 15 years, said former Vice President Joe Biden, addressing members of the American Association for Cancer Research on April 3.

“

By the way, I can think of so many other things to cut that money from. Oh, God bless me. Starting with the \$1.3 trillion in tax loopholes that aren't collected every year.

”

“That's not hyperbole,” Biden said. “The chance of getting a grant would almost certainly reach a historic low, because grants are funded for multiple years, and NIH is committed to funding the existing grants already in 2018.

“Simply by proposing to end our long tradition of bipartisan support for medical research and other sciences, we could be deterring millions of bright, ambitious youngsters from high schools through graduate school from pursuing a career in science,” Biden said.

“This is tragic. We cannot let it happen. ... And by the way, I can think of so many other things to cut that money from. Oh, God bless me. Starting with the \$1.3 trillion in tax loopholes that aren't collected every year.”

Less than three months ago, when Biden left the White House, he secured

the authorization of \$1.8 billion for cancer research over seven years. The money is still slated for NCI, but after the surprise of the Trump budget blueprint that calls for slashing NIH by 18.3 percent, nobody knows what to expect (The Cancer Letter, [March 17](#)).

“There's one problem today that didn't exist when I spoke to you a year ago,” Biden said at the AACR meeting. “The president of the United States is not only not doubling down on our investment; he's proposing draconian cuts not only to biomedical research, but also the entire scientific expertise, across the board.”

Trump's blueprint proposal could cut research grants by up to 90 percent, Biden said at AACR's 2017 annual meeting in Washington, D.C.

“This is no time to undercut progress, for God's sake. This is no time to let up.

It's time to double down," Biden said. "The damage is already being done, because the message is being sent out to the world and the brilliant young folks like a lot of you who are just in graduate school now, making their final decisions what path they're going to follow, and undergraduate school, deciding what they are going to do."

Biden's remarks come one year after NCI began the process of identifying scientific opportunities for investment via the Beau Biden Cancer Moonshot, a wide-ranging public health initiative—named after Biden's late son—aimed at doubling progress in cancer research over the next five years (The Cancer Letter, [April 1, 2016](#)).

The moonshot was passed by Congress in December as part of the 21st Centu-

"I'm a lawyer, I'm not a researcher. But I know how government works and doesn't work," Biden said at the AACR meeting. "I know a little bit about collaboration. We can do more together than we can by working alone."

"Secondly, I told you last year that I would get an additional \$1 billion for the moonshot scientific priorities, and you all said, 'Ain't he a nice man. Well intended.' Well, I was wrong. I got you \$1.8 billion in additional funding."

Biden said he will soon launch an organization named the Biden Cancer Initiative to continue his work on the moonshot (The Cancer Letter, [Jan. 6](#)).

"The bad news for y'all is, I assure you I'm not going anywhere," Biden said. "The reason I'm going to stay involved



## Yoder: Republicans in Congress remain committed to NIH

At a panel discussion that followed Biden's speech, Rep. Kevin Yoder (R-KS) said Republican members of Congress will not renege on their support for NIH and biomedical research. Yoder is a member of the House Appropriations Committee and chairman of the Legislative Branch Subcommittee.

"These are different times, I understand, politically, and people may be a little nervous about where Congress is going and what Washington may do as it relates to funding," Yoder said at the meeting.

"So, I guess first and foremost, I'm here to reassure you that this continues to be a priority for this Congress, for the elected representatives in Washington to fund cancer research, to fund the NIH, to make good on our promises in the moonshot and legislation passed in the 21st Century Cures Act that was just passed a few months ago.

"Congress really hasn't changed, regardless of who operates the White House, this continues to be a heavy



I told you last year that I would get an additional \$1 billion for the moonshot scientific priorities, and you all said, 'Ain't he a nice man. Well intended.' Well, I was wrong. I got you \$1.8 billion in additional funding.



ry Cures Act, a \$6.3 billion health care reform bill designed to modernize clinical trials and accelerate progress in research (The Cancer Letter, [Dec. 9, 2016](#)).

Biden said the moonshot yielded an "unprecedented level of collaboration" in cancer research, especially in oncology bioinformatics and data sharing. Throughout 2016, the then-vice president used his charisma, the power of the White House, and his vast reserves of political capital to cajole and shame—even threaten—allies and government agencies into action.

is because, for the first time in 45 years, there's some real, significant movement and collaboration, which, in my view, is where the solution is most likely to lie to double our rate in the fight against cancer: engaging cancer centers, drug companies, insurance industries, patients, the government."

An excerpt from Biden's remarks appears on [page 7](#). The Cancer Letter's coverage of the moonshot can be accessed [here](#).

priority for us. Part of that is because many of us see this as not another line

We know that the federal government plays a key role in that.

and Hematology Products (The Cancer Letter, [July 1, 2016](#)).



**We're here to be your ally and to let you know that the support will continue to be there, regardless of who operates the White House.**



item on the budget, not some part of some budget war, but something that really is fundamental to who we are as an American people and a prioritization of what matters in this country, in this Congress. This is an issue that, of course, affects both political parties equally. The disease of cancer is not something that affects Republicans and Democrats in a disparate way.”

“So, with the moonshot and with the work all these men and women are doing and that all of you are doing, we're here to be your ally and to let you know that the support will continue to be there, regardless of who operates the White House.”

Yoder assured the audience that Rep. Tom Cole (R-OK), deputy majority whip, senior appropriator on the House Budget Committee and chairman of the House appropriations subcommittee on Labor-HHS, as well as Sen. Roy Blunt (R-MO), vice chairman of the Senate Republican Conference and member of the Senate Appropriations Committee, remain committed to investing in science.

“We're going to unify and join forces to send a message to all of you, to send a message to young researchers across the country that are thinking about, ‘Should I go into the field of cancer research or should I go in to some other field?’ to reassure them and let them know that our commitment is strong, that our commitment is unwavering,” Yoder said.

“We are unified in our quest to go to war on cancer and to cure this disease and treat this disease, and to come up with new innovative ways to solve this disease in as many ways as possible.



### **Pazdur: FDA oncology center a lasting legacy of moonshot**

One of the “longest lasting legacies” of the moonshot is the creation of the FDA Oncology Center of Excellence, said Richard Pazdur, director of the OCE and the FDA Office of Oncology

“What this is, is an attempt of the FDA to integrate the oncology programs that had existed in different centers of the FDA and bring the expertise of all the oncologists together at the FDA,” Pazdur said at the meeting.

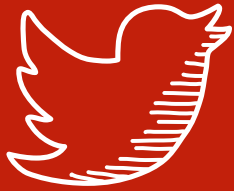
“Not only does it have an implication for getting out drugs faster, not only does it have an implication for regulatory work, but it also has implications for having the FDA be involved with other academic centers and professional groups. And, actually, developing regulatory science and the science that we will use in the regulation of drugs and drug development in general.

“So, I think the Oncology Center of Excellence is probably, for the FDA, the most important part of the moonshot project. But in addition to that, there were several other issues that we brought forth, and that included looking at how we do clinical trials, are there better ways of doing clinical trials. These include seamless trial designs where we really merge phase I, II, III testing together, taking a look at eligibility criteria, making them reflect more real-world situations, taking a look at large simple trials.

“In addition to that, I think the third prong of that for the FDA was really to take a look at, ‘Can government work better? Can government work together?’ and we've partnered with the NCI closely in developing projects that we want to work on.

“We want to work on, for example, greater disclosure of information before drugs are approved so the NCI has a realization of what is going on in the regulatory world from our perspective. So again, a lot of it is better communication with government agencies, also.”

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### **Lowy: NCI going forward with moonshot vision**

NCI is investing the first installment of the moonshot's \$1.8 billion allocation—\$300 million in FY17—in accordance with the Blue Ribbon Panel's recommendations.

"First, the NCI, in the 3.5 months since the moonshot act was passed, now has at the NCI website more than 15 different funding announcements for trying

itizing how to move forward with further development, with new awards in FY 18 and FY 19. And in the spirit of the Cancer Moonshot, this is designed to greatly accelerate the pace of cancer research and for people to work together.

"The third point I want to make is, the NCI can't do this alone. Instead, we are looking forward to partner with the private for-profit sector, with private philanthropy, with advocates, and many other groups. Vice President Biden talked about international agreements that we made, and we look forward to this being really a global effort to deal with this terrible disease."

Responding to a question from a member of the audience about Trump's proposed cuts, Lowy said that NCI requires sustained increases in appropriated dollars to fund more research ideas than it can currently afford.

"Needless to say, this is complicated. If cancer research were simple, it would've been resolved a long time ago," Lowy said. "But we are continually faced with many more outstanding, meritorious applications than we have resources for, and we continually need



**We run out of money long before we run out of perfectly great ideas to test, so the more resources we have, the more those ideas we can support.**



to implement and to start implementing different aspects of the Blue Ribbon Panel recommendations, which we look forward to being able to fund during this fiscal year," Lowy said at the meeting.

to strike a balance between the needs and the opportunities.

"We run out of money long before we run out of perfectly great ideas to test, so the more resources we have, the more those ideas we can support."

"The second part is we also have created implementation teams that are in the process of discussing and prior-

# Biden pledges to remain a player in oncopolitics, plans to launch cancer initiative

Former Vice President Joe Biden said he plans to launch the Biden Cancer Initiative, which would focus on data sharing, quality of cancer care, patient access, and increasing participation in clinical trials.



BIDEN SPOKE TO MEMBERS OF THE AMERICAN ASSOCIATION FOR CANCER RESEARCH AT THE ORGANIZATION'S 2017 ANNUAL MEETING IN WASHINGTON, D.C. ON APRIL 3. A VIDEO OF HIS SPEECH IS POSTED [HERE](#).

Biden announced his intent to remain a player in oncopolitics at the 2017 annual meeting of the American Association for Cancer Research in Washington, D.C.

“Starting very soon, we’re going to launch an organization, the Biden Cancer Initiative, with a significant board of people, including several Nobel laureates we work with, with similar goals as the Cancer Moonshot, but changing the way we do business in cancer research and development and providing cancer care, and focusing on breaking down siloes, increasing collaboration,” Biden said in a speech April 3.

“The initiative will focus on improving data standards, and giving patients some mechanism to share their data so they can help many other patients going through the same fight, so researchers can use data to find new patterns and new answers, working with

patients have all the options available; continuing to work for cultural change and improvement in our cancer research system, so we can make the best use today’s opportunities and to generate, share, and use data and knowledge from patients and researchers to help patients everywhere in the world.”

An excerpt of Biden’s speech at the 2017 AACR annual meeting follows:

The reason I’m going to stay involved is because, for the first time in 45 years, there’s some real, significant movement and collaboration, which, in my view, is where the solution is most likely to lie to double our rate in the fight against cancer: engaging cancer centers, drug companies, insurance industries, patients, the government.

But I’ll conclude by saying, there is one problem today that didn’t exist when I spoke to you a year ago.

His famous speech not only led to landing humans on the moon and bringing them back safely within a decade, but he set us on the path to be a world leader, the world leader, in science and technology, education, training, achievement.

We have enjoyed the fruits of that original inspiration and the improvements we have seen in the quality of our life.

Americans over the last 50 years have inspired the world with such achievements in space, medicine, physics, astronomy, telecommunications, computing, and energy, including the fight to combat climate change through massive improvements on how we generate and use electricity and fuel for transportation.

Parenthetically, I come from a city that was a coal city that died when the industry died in the late 1940s. It was difficult, Scranton, Pennsylvania. But, ladies and gentlemen, nothing is going to bring back the coal industry.

We have to accommodate those incredible changes those poor people are going through because it’s not only a job, it’s also a way of life. There is an ecosystem, a human ecosystem that is devastating them, but ladies and gentlemen, we created over 500,000 new jobs in alternative energy, 258,000 jobs just in solar energy. It’s now competitive with coal.

We cannot leave these people behind. But you cannot turn back the clock.

But the message sent out a few weeks ago in the president’s budget is counter to this hope and the progress we’ve made, and, now we’re standing on the cusp of delivering the promise of decades of research to develop new technology, new therapies, on the cusp of fundamentally transforming impact of cancer on our society, on the cusp of saving and extending lives of Americans.



The president of the United States is not only not doubling down on our investment; he’s proposing draconian cuts not only to biomedical research, but also the entire scientific expertise, across the board.



community care organizations help improve access to quality care so outcomes aren’t wholly dictated by the patient’s ZIP code, convening a national conversation with the pharmaceutical companies, insurers, biotech companies and others to ensure patients can actually access the treatments that become available and as are needed.

“I’m calling for greater transparency and access to clinical trials so that researchers on the next promising treatment isn’t stopped in its tracks, and

Although a year ago, there was a lot of doubt about my prediction we would have a bipartisan consensus to generate billions of dollars in additional research. But there’s a new problem.

When President [John F.] Kennedy launched the original moonshot to go to the moon, he inspired a generation of young people to enter science and technology. Maybe many of you—not so young anymore like me—are here today.



The president of the United States is not only not doubling down on our investment; he's proposing draconian cuts not only to biomedical research, but also the entire scientific expertise, across the board.

A \$5.9 billion cut from the NIH, nearly a 20 percent decrease. A cut of more than 30 percent from EPA when every one of you can tell me that the air we breathe, the water we drink has a profound impact on the very things that you are working on, a 30 percent cut.

And fundamentally changing his mission for providing a safe and healthy environment for Americans, cutting nearly \$2 billion in critical scientific infrastructure at the Department of Energy. And the NIH alone, this would set the NIH budget and biomedical research back 15 years. And that's not hyperbole.

The president of the United States is not only not doubling down on our investment; he's proposing draconian cuts not only to biomedical research, but also the entire scientific expertise, across the board.

The chance of getting a grant would almost certainly reach a historic low, because grants are funded for multiple years, and NIH is committed to funding the existing grants already in 2018.

The number of new grants for 2018 wouldn't be cut by 20 percent but, [according to] one reliable estimate, will be cut by up to 90 percent, closing labs, ending careers, delaying scientific breakthroughs.

This is no time to undercut progress, for God's sake. This is no time to let up. It's time to double down. It's time to be sure we deliver on a promise of science and technology to extend and improve lives.

And by the way, I can think of so many other things to cut that money from.

Oh, God bless me. Starting with the \$1.3 trillion in tax loopholes that aren't collected every year.

Now, look, that's the bad news. Here's the good news, I don't think there is a chance the American people, or the United States Congress—virtually the same Congress that passed the 21st Century Cures Act just several months ago—will support or pass this budget into law.

But the damage is already being done, because the message is being sent out to the world and the brilliant young folks like a lot of you who are just in graduate school now, making their final decisions what path they're going to follow, and undergraduate school, deciding what they are going to do.

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that passed the 21st Century Cures Act just several months ago—will support or pass this budget into law.

Simply by proposing to end our long tradition of bipartisan support for medical research and other sciences, we could be deterring millions of bright, ambitious youngsters from high schools through graduate school from pursuing a career in science.

And maybe moving some of your talents to other less critical challenges,

out of fear that they'll never get funded to do the kinds of things they care about, out of fear that these kinds of fields are no longer important to the country.

This is tragic. We cannot let it happen.

In his famous speech at Rice University, President Kennedy talked about the challenge of getting to the moon and back, about taking on new challenges and driving for new answers.

He said, “We set sail on this new sea because there is new knowledge to be gained, and new rights to be won, and they must be won and used for the progress of all people.”

These words inspired countless young Americans to set their sights on new horizons, and we can't back down from that purpose. It's not hyperbole; it's just a fact. We can't.

Last year, when I laid out the challenges you face in a system that needs improvement, was to let you know that I'm with you, not against you. I'm with you.

I and a lot of other people who still are in office will continue to fight for you, to give you support for the conditions you deserve to help end the scourge of cancer. And I know you will join me in doing your part in supporting this continued culture of sharing and the urgency of now.



I and a lot of other people who still are in office will continue to fight for you, to give you support for the conditions you deserve to help end the scourge of cancer. And I know you will join me in doing your part in supporting this continued culture of sharing and the urgency of now.



The moonshot is no longer an office in the White House. Although I did talk to the vice president and others about it, and I am prepared to do anything I can to work with him, if they pursue these efforts.

But our work and our endeavor, not withstanding the moonshot that's no longer in the White House, still matters a great deal. We can provide real hope for patients, hope for the progress you made and the hope for the future you know we can achieve. For I, like you, see the day when patients get the right therapy the first time for their cancers.

I see the day when prevention is more effective and where care is personalized with less harmful side effects. I see the day when those of you in this room, when you take your children and grandchildren to the physician to get to school physical, that they will be vaccinated against certain cancers like they can now be vaccinated against HPV.

I see the day when we are able to identify through markers in the blood cancers that are poised to develop into tumors and spread, but haven't developed yet, giving us the chance to treat them at the most vulnerable stage.

The one thing I can tell you is, there is hope. You can see there is not a single place I go in the country or the world—and this is not hyperbole—that the first or second thing I am asked, whether I am on the street or in a meeting with a head of state: cancer, cancer; “Tell me how it's going, Mr. Vice President. Mr. Vice President, tell me.”

I gets hundreds of hundreds and hundreds and hundreds of letters. One man just wrote to be about losing his son to opioid addiction, who was raising a child who was a child with serious disabilities, and his wife had just died from cancer—it went on for five handwritten pages.

And he ended by saying, “Mr. Vice President, I don't even know why I'm telling you all of this. I am hoping you, I think maybe you can understand.”

People are desperate, looking for hope. You all know that's the one thing that's most needed.

When Kennedy talked about going to the moon, he talked about a commitment, a commitment that he said and I think has become my sort of rallying cry for this.

He said, “We're making this commitment because, as a nation, we're unwilling to postpone this crazy adventure.”

In our fight against cancer, we have to be unwilling to postpone, even for a second, to do all we can for as long as it takes, because you know, with the incredible collective talent you possess, we can fundamentally change the prospects, and promise of life for tens of millions of people all around the world.

I can think of nothing more noble to be engaged in, than what all of you are doing, and I'm not being solicitous. I mean that from the bottom of my heart. You're an incredible, incredible national and international resource.

So, let's keep it going, folks. This is no time to stop the momentum.

Thank you all so very much.



GUEST EDITORIAL

# Oregon's Death with Dignity Act: Learning from 18 years of data on physician-aided dying

By Charles Blanke and Lee Ellis



**Charles Blanke**

*Chair of SWOG and professor of medicine at OHSU Knight Cancer Institute.*



**Lee Ellis**

*Vice chair for translational medicine at SWOG and professor of surgery at the MD Anderson Cancer Center Department of Surgical Oncology, Division of Surgery.*

Oncologists must confront end-of-life issues on a nearly daily basis. Our approach to the potential death of a patient may change over time, however, depending on the patients' diagnosis and stage, where those patients are in their treatment plan, and, of course, what the patients' wishes are. When feasible, our primary goal is to prevent death from cancer, and when we cannot achieve that, we try to delay death as long as we can. When dying seems inevitable, we do our best to make it as comfortable as possible.

For the past 20 years, Oregonians have had an option, when they have a terminal medical condition and specific

life-expectancy of less than six months, to actively seek their own death. Oregon's Death with Dignity Act became the nation's first when it went into effect in 1997, and physician-aided dying (PAD) is now also legal in California, Colorado, Montana, Vermont, Washington, and the District of Columbia. States tightly regulate eligibility and process—Oregon and those modeled upon its law also require residency and a competent patient who is acting voluntarily. One of us, Charles Blanke, offers PAD as part of his clinical practice.

The Oregon Health Authority collects mandatory data on use of the law, and SWOG recently reviewed 18 years of

state statistics. We were part of the research team that published the results in *JAMA Oncology* this week. Here's what we learned:

- From 1998 through 2015, 1,545 prescriptions for lethal medication were written, and 991 patients took the drugs and died
- Of the patients who died, most had cancer—77 percent—and lung, breast, and pancreatic tumors were the most common
- Drugs were 99.4 percent effective, and other complications were rare

- Gender was split evenly, but age ranged widely, from 25 to 102, with a median of 71
- Most patients were white (97 percent), had at least some college education (71 percent), and were under hospice care (92 percent)
- The most commonly cited reasons for using lethal medications were loss of autonomy (92 percent), inability to enjoy activities of daily living (90 percent), and loss of dignity (79 percent)
- One in four patients opted for PAD because of inadequate pain control, and, worse still, 3 percent stated the cost of medical treatment was a driver of their decision.

It's clear that patients with cancer and other terminal diseases want the option to end their lives, and when that is legal and accessible, they are increasingly using it. The reasons for choosing PAD aren't classically medical, nor are they easy to palliate. Patients want independence, control, and dignity. And when those are gone, they don't want to go on living. Sometimes terminal sedation is offered, as a "more ethical" choice.

Who gets to decide when and how a patient dies?

We think it should be the patient.

While aid-in-dying is beneficial, and increasingly popular, we have much work ahead as physicians, as researchers, and as citizens pushing for policy change. No one expects all physicians to actively participate in aid-in-dying; writing that prescription for lethal medication is hard. But in Oregon, executing PAD, a legal option, is also hard for the patient.

It sometimes takes them months just to find a prescribing physician. Many doctors not only choose not to prescribe—but also choose not to refer. This "good

luck with that" mentality doesn't serve patients well and is essentially a form of abandonment. We implore those living in states where PAD is legal to help the patients needing PAD to have access to PAD.

Also, as researchers, we must tackle important, unanswered questions. Why do so many patients—about one in three—get the lethal medications and not use them? Could changes in palliative care reduce the use of PAD? Why do some patients experience prolonged comas before dying? As a nation, we need a robust PAD research program, and it should be funded by public dollars.



The reasons for choosing PAD aren't classically medical, nor are they easy to palliate. Patients want independence, control, and dignity. And when those are gone, they don't want to go on living. Sometimes terminal sedation is offered, as a "more ethical" choice.



We must tighten the laws and regulations. In Oregon, the PAD law has been interpreted as requiring patients to request and "voluntarily self-administer" lethal medication. Most physicians have taken that to mean that a patient must be physically and mentally fit until the actual time the oral medication is consumed. It is brutally disappointing to patients who become too weak to avail themselves of PAD, and those anticipating potential mental decline might take the medicine too early.

Allowing an intravenous formulation administered by a physician would eliminate the former problem. Allowing patients to put a PAD request in an

advance directive would eliminate the latter one. though we don't soon see that happening in the United States, (we can hear the slippery-slope-based cries that we will soon be euthanizing the elderly and mentally infirm, similar to the use of the inaccurate term "Death Panels" associated with the Affordable Care Act).

Finally, we must change the marketplace. Standard aid-in-dying drugs are expensive, ranging in cost from about \$3,200 to \$7,700 in Portland, OR—and they are not always covered by insurance. New formulations are much cheaper, but they are scary. One cocktail, for example, combines two

cardiac medications at 200 times the therapeutic dose. How do we know patients will succumb before experiencing digoxin toxicity? As one can imagine, formal Phase I testing isn't done on these combinations, and no agency really regulates this type of off-label use of medications.

In our Oregon analysis, we've learned aid-in-dying is largely an issue faced by the cancer community. With an estimated 20 states considering PAD laws this year alone, more cancer caregivers and researchers will be faced with aid-in-dying. PAD is here to stay. We need to face it with more compassion, more understanding—and more evidence.

## IN BRIEF



## Michael Caligiuri starts term as AACR president

Michael Caligiuri became president of the American Association for Cancer Research for 2017-2018.

Caligiuri is the director of The Ohio State University Comprehensive Cancer Center and chief executive officer of the Arthur G. James Cancer Hospital and Richard J. Solove Research Institute, and the John L. Marakas Nationwide Insurance Enterprise Foundation chair in cancer research and a professor in The Ohio State University College of Medicine Departments of Molecular Virology, Immunology and Medical Genetics, and Internal Medicine.

Caligiuri is a physician-scientist known for his work in immunology in the treatment of leukemia, myeloma, and glioblastoma. Well over 1,500 cancer patients have been treated on clinical protocols that have emanated from the Caligiuri laboratory.

“Game-changing cancer research breakthroughs can’t come fast enough—and as an oncology commu-

nity we must continue to be an unwavering force for progress in research that benefits the many people affected by cancer,” Caligiuri said in a statement.

Caligiuri has been involved with the AACR since 1990, serving on the board of directors (2013-2016) and as chairperson of the Publications Committee since 2003. He has also served as:

- A member of the Clinical and Translational Cancer Research Committee (2001-2004, 2012-present),
- A member of the Margaret Foti Award for Leadership and Extraordinary Achievements in Cancer Research Committee (2015);
- Landon Foundation-AACR INNOVATOR Award for Cancer Prevention Research Scientific Review Committee (2011);
- Pezcoller Foundation-AACR International Award for Cancer Research Selection Committee (2010);
- Chairperson of the Annual Meeting Program Committee (2009);
- Member of the Scientific Program and Scientific Review Committees for the Translational Cancer Medicine Meeting (2008);
- Steering Committee of the Cancer Immunology Working Group (2007-2012);
- Clinical Research and Experimental Therapeutics Awards Selection Committee (2004);
- Chairperson (2002) and member (2001) of the Scientific Committee of the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics;



- Member of the editorial boards of Molecular Cancer Therapeutics (2001-2005) and Clinical Cancer Research (1996-2008);
- Associate editor of Cancer Research (2001-2003);
- Member of the Science Policy and Legislative Affairs Committee (2001-2004, 2006-2009, 2012-2015);
- The Richard and Hinda Rosenthal Foundation Award Selection Committee (1999); and
- Chairperson of the Membership Committee (1999).

He served as a member of the faculty for the Scientist Survivor Program at the AACR Annual Meeting (2003-2009) and as a member of the faculty for the educational workshop, Methods in Clinical Cancer Research (2003-2007).

Additionally, he is president of the Society of Natural Immunity, chair of the Institute of Medicine's National Cancer Policy Forum, and a member or chair of the external boards for 12 of the nation's cancer centers. He is a past president of the Association for American Cancer Institutes, as well as a former councilor and executive committee member of the American Society of Hematology, past member of the NCI Board of Scientific Advisors and the Board of Scientific Counselors, and past vice chair of the scientific advisory board of the Cure for Lymphoma Foundation.

Elizabeth Jaffee, deputy director of the Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins, and associate director of the Bloomberg-Kimmel Institute for Cancer Immunotherapy, was inducted as president-elect. Nancy E. Davidson, executive director of oncology for Fred Hutch/University of Washington Cancer Consortium and president of Seattle Cancer Care Alliance, now serves as past president.

## SU2C names researchers to \$12 Million “Dream Team” on colorectal cancer

Stand Up To Cancer announced the formation of a “Dream Team” of researchers to take on one of the toughest challenges in cancer research and treatment.

The announcement was made at a special event during the 2017 Annual Meeting of the American Association for Cancer Research, SU2C's Scientific Partner.

“We are urgently in need of new approaches to colorectal cancer,” said Phillip A. Sharp, institute professor at MIT's Koch Institute for Integrative Cancer Research, Nobel Laureate, and chairman of SU2C's Scientific Advisory Committee. “We have made great progress in prevention through widespread screening, but new methods are needed to treat colorectal cancer when it actually occurs.”

The SU2C Colorectal Cancer Dream Team, with funding of up to \$12 million from SU2C, will be led by Luis Diaz, of Memorial Sloan Kettering Cancer Center as leader, and Charles Fuchs, director of the Yale Cancer Center; Lewis Cantley, director of the Meyer Cancer Center at Weill Cornell Medicine and New York-Presbyterian Hospital; and Zhenghe Wang, of the School of Medicine at Case Western Reserve University, as co-leaders.

More than 50 researchers at six institutions are involved in the team.

“Through a combination of new avenues in immunotherapy, targeted therapeutics, metabolomics, and precision prevention, we believe we can find new ways to fight colorectal cancer and bring new hope to patients,” Diaz said.

The team's research program will also include clinical trials to investigate drugs that could attack genetic vulnerabilities in many types of colorectal cancer tumors.

“The metabolism of cancer cells, how they process nutrients, is different from normal cells,” Cantley said. “The trials are designed to take advantage of that knowledge and hopefully kill the cells.”

Serving as Dream Team Principals are Ryan Corcoran, Massachusetts General Hospital Cancer Center, and Nilofer Azad, Johns Hopkins University.

Serving as Dream Team Advocates are Anjee Davis, president, Fight Colorectal Cancer; Ivelisse Page, executive director and co-founder, Believe Big Inc.; Joanna Fuchs, patient advocate, Yale University; Martha Raymond, executive director, Michael's Mission; Thomas Herbert Marsilje; and Vanessa Whiting, president, A.E.S. Management Corp.

The colorectal team is the 20th Dream Team launched by Stand Up To Cancer since its inception in 2008, which has also launched six Translational Research Teams, 46 Innovative Research Grants, and a host of other grants and awards to encourage innovative and collaborative cancer research, with funds committed by philanthropic, organizational, corporate, and individual donors, as well as nonprofit groups working with SU2C.

## BMS gives SU2C to \$7.5 million to fund immunology grants

Stand Up To Cancer announced the award of \$7.5 million in Innovative Research Grants focused on immuno-oncology to 10 early-career scientists, in a

program funded by a grant from Bristol-Myers Squibb Co.

The awards are part of SU2C's overall IRG program, which has provided funding to 36 early-career scientists in three classes thus far (in 2009, 2011, and 2016), in an effort to support outstanding members in the rising generation of cancer researchers.

The 2017 class is unique in that funds are being provided by BMS through a grant to SU2C, and applicants were asked to focus on some aspect of immuno-oncology. The process of selecting grantees was conducted by the IRGC, with the assistance of AACR, and was independent of BMS.

From its inception in 2008, SU2C has been committed to creating opportunities for early-career researchers. Its model for the IRGs was designed specifically to support work that utilizes new ideas and approaches to solve critical problems in cancer research.

Traditionally, the projects most likely to be funded in the oncology field are those with a demonstrable expectation of success, which means that some of the research has to be done before an investigator can submit a proposal. SU2C's IRG program is one of the few opportunities for young scientists to receive funding for cancer research that does not have a "proof of concept" data requirement.

These innovative IRG-funded projects are characterized as "high-risk" because they challenge existing paradigms, and, if successful, the projects have the potential for "high-reward" in terms of saving lives. The 2017 class of immuno-oncology Innovative Research Grant recipients and their proposals are:

- **David Barrett**, Children's Hospital of Philadelphia, "Rescuing T-Cell Function for Immunotherapy of Pediatric Malignancies"
- **Gregory Beatty**, University of Pennsylvania, "Targeting the Pro-Metastatic Niche in the Liver for Cancer Immunotherapy"
- **Marie Bleakley**, Fred Hutchinson Cancer Research Center, "T-Cell Immunotherapy for Core Binding Factor Acute Myeloid Leukemia"
- **Michael Farwell**, University of Pennsylvania, "Imaging CAR T Cells with a Dual Function PET Reporter Gene"
- **Rizwan Haq**, Dana-Farber Cancer Institute, "Identifying and Targeting Mechanisms of Resistance to Immunotherapy"
- **Meenakshi Hegde**, Baylor College of Medicine, "Reworking Negative Receptor Signals for Improved Anti-Glioma T-cell Therapy"
- **Marcela Maus**, Massachusetts General Hospital Cancer Center/Harvard Medical School, "Potentiating Novel Engineered Cellular Therapies for Solid Tumors"
- **Jennifer Wargo**, MD Anderson Cancer Center, "Delineating the Role of the Microbiome in Modulating Tumor and Host Immunity"
- **John Wilson**, Vanderbilt University, "Reprogramming Tumor Immunogenicity with STING-Activating Nanoparticles"

The term of the grants begins July 1 and runs for three years. The scientists will report their progress twice yearly to SU2C and the AACR, which organized the application and review process, and will administer the grants.

## David Sabatini wins FNIH Lurie Prize in Biomedical Sciences



The Foundation for the National Institutes of Health has selected David Sabatini to receive its fifth annual Lurie Prize in Biomedical Sciences for discovery of the mTOR (mechanistic target of rapamycin) cellular pathway as a key regulator of growth and metabolism in response to nutrients.

Sabatini is a pioneer in the study of nutrient sensing and the impact of caloric restriction on health and lifespan. The Lurie Prize in Biomedical Sciences will be presented to him at the FNIH Award Ceremony hosted by CNN's Wolf Blitzer on May 17 in Washington, D.C.

Sabatini is a member of the Whitehead Institute for Biomedical Research, a Professor of Biology at the Massachusetts Institute of Technology and an Investigator of the Howard Hughes Medical Institute. His research identified the specific protein components of the mTOR pathway, including mTOR and two large complexes that contain it called mTOR Complex 1 (mTORC1) and Complex 2 (mTORC2), and documented how mTOR regulation and

dysregulation affects normal and diseased physiology.

As caloric restriction is associated with the slowing of cellular aging, Sabatini's research suggests that one day, the mTOR pathway could be manipulated to trick the body into mimicking a fasting state even under nutrient replete conditions, and thereby protect against age-related diseases, such as cancer and diabetes. The Lurie Prize in Biomedical Sciences recognizes outstanding achievement by a promising scientist aged 52 or younger.

Sabatini was selected by a jury of six distinguished biomedical researchers, chaired by Solomon Snyder, Distinguished Service Professor of Neuroscience, Pharmacology & Psychiatry, The Solomon H. Snyder Department of Neuroscience at Johns Hopkins University and Vice Chairman for Science of the FNIH.

The prize includes a \$100,000 honorarium, donated by philanthropist and FNIH Board Member Ann Lurie. Lurie is President of the Ann and Robert H. Lurie Foundation, which she founded with her late husband Robert, and the President of Lurie Holdings Inc.

Previous recipients of the Lurie Prize in Biomedical Sciences are Jeannie Lee, from Massachusetts General Hospital and Harvard Medical School (2016), Karl Deisseroth, from Stanford University (2015), Jennifer Doudna, from the University of California, Berkeley (2014) and Ruslan Medzhitov, from Yale University School of Medicine (2013).

Johnson & Johnson Innovation will sponsor the 2017 FNIH Award Ceremony.

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## Jennifer Pietenpol named chief scientific advisor for Susan G. Komen



Jennifer Pietenpol, executive vice president for research at Vanderbilt University Medical Center and director of Vanderbilt-Ingram Cancer Center, was named a chief scientific advisor for the nonprofit breast cancer organization Susan G. Komen.

She joins George Sledge Jr., professor of Medicine at Stanford University Medical Center, in the CSA role, which includes responsibility for guiding the Komen Scientific Advisory Board.

The scientific advisory board helps guide Komen's research programs and priorities. Since 2010, Pietenpol has served as a Komen Scholar, an advisory group of distinguished leaders in breast cancer research and advocacy who are chosen for their knowledge and leadership within the scientific, research and advocacy communities, and for their own contributions to breast cancer research.

Pietenpol, the Benjamin F. Byrd Jr. Professor of Oncology at Vanderbilt, is an expert in molecular genetics and triple negative breast cancer. She and her colleagues were the first to identify subtypes of TNBC and are spearheading clinical research trials to determine the best potential therapies for each subtype.

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## Mark Soberman becomes president of ACCC

Mark Soberman, was elected as the 2017-2018 President of the Association of Community Cancer Centers during its annual meeting March 31.

A thoracic surgeon and thoracic oncologist, Soberman is the medical director, Oncology Service Line and chief physician executive, Monocacy Health Partners at Frederick Regional Health System.

Soberman has been an active member of ACCC, serving on the executive committee of the board of trustees and as President-Elect (2016-2017).

He is a reviewer for the Annals of Thoracic Surgery and an active member of numerous organizations, including the Society of Thoracic Surgeons, the Southern Thoracic Surgical Association, the General Thoracic Surgical Club, the American Association for Physician Leadership, the American College of Healthcare Executives, and the American Society of Clinical Oncology.

Previously, Soberman served as vice-chair of the Department of Surgery; director, Section of Thoracic Oncology; and director, Section of Thoracic Surgery at MedStar Washington Hospital Center. Soberman has held leadership positions as Director, Division of Thoracic Surgery at Georgetown University Hospital, and Director, Section of Thoracic Surgery at Sibley Memorial Hospital.

His academic appointments include clinical associate professor of surgery at Georgetown University School of Medicine and Clinical Assistant Professor of Surgery at George Washington University School of Medicine.



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## Biocept, OHSU announce collaboration to increase clinical use of liquid biopsy

Biocept Inc. has entered into a Preferred Provider Collaboration and Services Agreement with Oregon Health & Sciences University on behalf of the OHSU Knight Cancer Institute.

The multiphase agreement grants OHSU the rights to commercially offer Biocept's Target Selector liquid biopsy testing services exclusively throughout the state of Oregon. Additionally, Biocept and OHSU plan to engage in technology transfer, whereby OHSU will have the ability to use Target Selector assays in-house, and act as a secondary laboratory for Biocept's research and testing activities.

Biocept and OHSU also plan to co-develop additional liquid biopsy assay technologies and platform capabilities including highly sensitive, multiplexed assay panels for molecular biomarker detection and assessment.

OHSU's Knight Diagnostic Laboratories selected Biocept's Target Selector platform after evaluating several commercially available and research-stage liquid biopsy technologies throughout the industry. Additional R&D and commercial pilot projects are anticipated under the agreement.

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## Sylvester, Syapse to launch precision medicine initiative

Sylvester Comprehensive Cancer Center, part of UHealth—the University of Miami Health System, and Syapse, a precision oncology company, an-

nounced that they will team up to launch a new precision medicine initiative at Sylvester.

By partnering with Syapse, Sylvester physicians will be able to more efficiently deliver personalized care that matches patients with targeted, cutting-edge therapies based on the clinical and molecular profile of the patient, leading to improved survival rates and better health outcomes.

Through the partnership with Syapse, Sylvester physicians will now have a robust platform that brings together clinical, molecular, treatment, and outcomes data to power this new era of precision medicine clinical workflows.

The Syapse platform will provide access to the largest, real-world precision oncology data sharing consortium in the world, enabling oncologists to make better decisions for their patients using real-world treatment and outcomes data on clinically and molecularly similar patients.

The Syapse platform is adopted by Intermountain Healthcare, Providence St. Joseph's Health, Stanford Healthcare, Henry Ford Health System, Catholic Health Initiatives and Dignity Health.

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## Invitae starts Patient Insights Network to enable sharing of health information

Invitae Corp. announced the launch of the Invitae Patient Insights Network, a permission-based, patient-centered network designed to make it easy for patients to share health experiences, contribute de-identified clinical data, and maintain their privacy while being connected to the latest research, treatment, and disease education opportunities.

The Invitae Patient Insights Network is a patient-directed approach to the traditional patient registry.

The Invitae PIN enables patients with specific health conditions to share information, be connected to clinical trial and research opportunities, and contribute de-identified data across a wide variety of health conditions.

The information patients share can be used by clinicians, researchers, and therapeutic developers to locate screened cohorts for possible participation in research studies. Through the Invitae PIN, patients direct and control how their de-identified information is shared and can opt in or opt out at any time.

In addition, Invitae will continue to contribute de-identified data to public research databases such as ClinVar, a freely available genetic information database from the National Center for Biotechnology Information.

Patients who enroll in the Invitae PIN are guided through a series of questions to gather their diagnostic experience, genetic information, treatment experience and other relevant health and family history information. Based on their responses, patients gain access to a dashboard that allows them to explore de-identified information contributed by others with the same diagnosis, for example viewing the treatment experiences of other breast cancer patients. Using the dashboard, patients can manage their preferences to share data or receive notifications about clinical trials and research opportunities that match their profile.

The Invitae PIN will open enrollment to people with a personal or family history of cancer or who have had genetic testing for cancer predisposition. Enrollment for patients with conditions in additional clinical areas, such as cardiology and neurology, will open throughout 2017.

## LLS Commits \$4 Million to Forty Seven Inc.

The Leukemia & Lymphoma Society made a \$4 million funding commitment in an investigational therapy being developed by Forty Seven Inc. for lymphoma patients.

LLS's investment will support Forty Seven's clinical trial using an antibody therapy (Hu5F9-G4) aimed at treating two types of NHL - diffuse large B-cell lymphoma and follicular lymphoma.

DLBCL represents approximately 30 percent of NHL patients, with 60 percent of patients surviving five years after diagnosis; however, more than one-third of patients either relapse or do not respond to therapy. Approximately 25 percent of NHL patients are diagnosed with FL, a slow-growing form of the disease.

While most patients with FL respond to initial therapy, more than 70 percent are diagnosed with advanced stage disease and are considered incurable. The novel drug will be tested in combination with the FDA-approved rituximab, already part of standard treatment for several types of NHL.

The therapy is directed against CD47, a protein that provides a "don't eat me" signal to the immune system and blocks the ability of immune cells called macrophages to devour those cancer cells. The combination Hu5F9-G4 and rituximab displayed synergy in preclinical animal models of NHL.

Forty Seven was founded in 2015 by Stanford University researchers Irv Weissman and Ravi Majeti, both of whom have been recipients of LLS grants supporting their early work targeting CD47. Forty Seven has licensed the therapy from Stanford.

## NCCN imaging appropriate use criteria published for 13 additional guidelines

The National Comprehensive Cancer Network, a Centers for Medicare & Medicaid Services-approved provider-led entity for imaging appropriate use criteria continues to build its library of AUC and has published NCCN Imaging Appropriate Use Criteria (NCCN Imaging AUC) for an additional 13 NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines).

Launched in June 2016, NCCN Imaging AUC currently are available for 48 NCCN Guidelines. The newest NCCN Imaging AUC™ include recommendations for:

- Anal Cancer
- B-Cell Lymphomas
- Chronic Lymphocytic Leukemia/ Small Lymphocytic Lymphoma
- Colon Cancer
- Hairy Cell Leukemia
- Hepatobiliary Cancers
- Primary Cutaneous B-Cell Lymphomas
- Rectal Cancer
- Soft Tissue Sarcoma
- Systemic Light Chain Amyloidosis
- Testicular Cancer
- T-Cell Lymphomas
- Waldenström's Macroglobulinemia/ Lymphoplasmacytic Lymphoma

NCCN Imaging AUC are an easy-to-use, single source for imaging recommendations pertaining to cancer screening, diagnosis, staging, treatment response assessment, follow-up, and surveillance as outlined within the library of NCCN Guidelines. NCCN Imaging AUC include all imaging procedures recommended in the NCCN Guidelines, including radiographs, computed tomography scans, magnetic resonance

imaging, functional nuclear medicine imaging, and ultrasound.

NCCN Imaging AUC are available through a web-based user interface that provides a searchable and user-customized display of approved NCCN Imaging AUC. The complete library of NCCN Imaging AUC™ is scheduled to be available in 2017. The NCCN Guidelines are the recognized standard for clinical policy in cancer care and are often the most thorough and most frequently updated clinical practice guidelines available in any area of medicine. Other NCCN Guidelines derivative products include:

- The NCCN Drugs & Biologics Compendium (NCCN Compendium) contains authoritative, scientifically derived information designed to support decision-making about the appropriate use of drugs and biologics in patients with cancer. The NCCN Compendium is recognized by public and private insurers alike, including CMS and UnitedHealthcare, as an authoritative reference for oncology coverage policy.
- The NEW NCCN Radiation Therapy Compendium includes NCCN Guidelines recommendations pertaining to indications, modalities, clinical scenario, purpose, as well as the dosing regimens used for treatment. Additional information includes the clinical notes related to the specific recommendation.
- The NCCN Biomarkers Compendium contains information designed to support decision-making around the use of biomarker testing in patients with cancer.
- The NCCN Chemotherapy Order Templates (NCCN Templates) include chemotherapy, immunotherapy, supportive care agents, doses, schedules, monitoring parameters, and safety instructions for regimens recommended in the NCCN Guidelines.

## DRUGS &amp; TARGETS



## Novartis drug combination Tafinlar + Mekinist receives EU approval for BRAF V600-positive advanced NSCLC

Novartis said the European Commission has approved Tafinlar (dabrafenib) in combination with Mekinist (trametinib) for the treatment of patients with BRAF V600-positive advanced or metastatic non-small cell lung cancer.

The approval marks the first targeted treatment approved for the patient population, who previously had few treatment options, in all 28 member states of the European Union, plus Iceland and Norway.

Every year, it is estimated, up to about 36,000 people, or about 1-3% of patients with lung cancer, are diagnosed with BRAF V600-positive NSCLC worldwide.

EU approval follows a positive opinion granted in February by the Committee for Medicinal Products for Human Use, which was based on safety and efficacy of dabrafenib in combination with trametinib in a phase II, three-co-

hort, multicenter, non-randomised and open-label study in which patients with stage IV BRAF V600E mutant NSCLC were enrolled (36 treatment-naïve [previously untreated] and 57 previously treated with chemotherapy).

For the primary endpoint of investigator-assessed overall response rate (ORR), 36 treatment-naïve patients receiving 150 mg of Tafinlar twice daily and 2 mg of Mekinist once daily demonstrated an ORR of 61.1% (95% confidence interval: 43.5%, 76.9%).

In this population, 68% of patients had not progressed after 9 months. The median duration of response (DoR) and progression free survival in the previously untreated population were not yet reached at the time of approval.

In the previously treated population receiving the same dosage, patients demonstrated an ORR of 66.7% (95% CI: 52.9%, 78.6%). The response was durable with a median DoR reaching 9.8 months (95% CI: 6.9, 16.0).

An in-depth analysis of data from the treatment-naïve cohort will be presented at an upcoming medical meeting.

The most common adverse events (incidence >20%) were pyrexia, nausea, vomiting, peripheral edema, diarrhea, dry skin, decreased appetite, asthenia, chills, cough, fatigue, rash, and dyspnea.

FDA granted Tafinlar + Mekinist Breakthrough Therapy designation for advanced or metastatic BRAF V600E-positive NSCLC patients in 2015 and Priority Review in November 2016.

Combination use of Tafinlar + Mekinist is approved in the US, Europe, Australia, Canada, and additional countries for patients with unresectable or metastatic melanoma whose tumors tested positive for the BRAF V600 mutation.

## FDA accepts for review the application of Ibrutinib for cGVHD after failure of systemic therapy

Janssen Research & Development, LLC said FDA has accepted for review a supplemental New Drug Application for ibrutinib (Imbruvica) for the treatment of patients with chronic graft-versus-host-disease after failure of one or more lines of systemic therapy.

Imbruvica is jointly developed and commercialized by Janssen Biotech Inc. and Pharmacyclics LLC, an AbbVie company.

The sNDA is supported by data from a single-arm Phase 1b/2 trial (PCYC-1129) examining the safety and efficacy of ibrutinib in patients with cGVHD who have failed first-line corticosteroid therapy and require additional therapy.

The data was accepted as a late-breaker and presented at the American Society of Hematology annual meeting in December 2016 and the Blood and Marrow Transplantation Tandem Meeting in February 2017.

Based on this data, a phase III study was initiated to evaluate ibrutinib with corticosteroid versus placebo with corticosteroid as a first-line therapy for patients with new onset moderate or severe cGVHD; the trial is currently ongoing.

FDA granted Breakthrough Therapy designation and Orphan Drug designation in June 2016 for ibrutinib as a potential treatment for cGVHD after failure of one or more lines of systemic therapy. Approximately 30-70 percent of post-allogeneic transplant patients develop cGVHD.

## Amgen seeks to expand XGEVA indications to multiple myeloma in the US, Europe

Amgen announced the submission of a supplemental Biologics License Application to FDA and the European Medicines Agency for XGEVA (denosumab).

The submissions to regulatory authorities seek to expand the currently approved XGEVA indication for the prevention of skeletal-related events in solid tumors to include patients with multiple myeloma. The applications include new data from the pivotal phase III head-to-head 482 study, the largest international multiple myeloma trial ever conducted.

XGEVA is a fully human monoclonal antibody that binds to and neutralizes RANK ligand (RANKL)—a protein essential for the formation, function and survival of osteoclasts, which break down bone—thereby inhibiting osteoclast-mediated bone destruction.

XGEVA is indicated for the prevention of SREs in patients with bone metastases from solid tumors based on results from three previous pivotal phase III head-to-head studies. In these phase III studies, XGEVA demonstrated superiority in the solid tumors studied compared to zoledronic acid.

In the U.S., XGEVA has a limitation of use noting that it is not indicated for the prevention of SREs in patients with multiple myeloma. The sBLA is based on efficacy and safety data from the pivotal phase III 482 study, which demonstrated that XGEVA is non-inferior to zoledronic acid in delaying the time to first on-study SRE in patients with multiple myeloma (HR=0.98, 95 percent CI: 0.85, 1.14; p=0.01).

The secondary endpoints of superiority in delaying time to first SRE and delaying time to first-and-subsequent SRE were not met in this study. Overall survival, another secondary endpoint, was also in favor of XGEVA over zoledronic acid (HR=0.90, 95 percent CI: 0.70, 1.16; p=0.41); however, it was not statistically significant. The hazard ratio of XGEVA versus zoledronic acid for progression-free survival was 0.82 (95 percent CI: 0.68, 0.99; descriptive p=0.036). The median PFS difference between arms was 10.7 months in favor of XGEVA.

Adverse events observed in patients treated with XGEVA were consistent with the known safety profile of XGEVA. The most common adverse events (greater than 25 percent) were diarrhea (33.5 percent XGEVA and 32.4 percent zoledronic acid) and nausea (31.5 percent XGEVA and 30.4 percent zoledronic acid).

## RedHill receives orphan drug designation for Yeliva

RedHill Biopharma Ltd., a specialty biopharmaceutical company primarily focused on the development and commercialization of late clinical-stage, proprietary, orally-administered, small molecule drugs for gastrointestinal and inflammatory diseases and cancer, said FDA has granted Yeliva (ABC294640) Orphan Drug designation for the treatment of cholangiocarcinoma.

Yeliva is a phase II, proprietary, first-in-class, orally-administered sphingosine kinase-2 (SK2) selective inhibitor with anticancer and anti-inflammatory activities, targeting multiple oncology, inflammatory and gastrointestinal indications. By inhibiting the SK2 enzyme, Yeliva blocks the synthesis of sphingosine 1-phosphate, a lipid signaling molecule that promotes cancer

growth and pathological inflammation, the company said.

Cholangiocarcinoma is a highly lethal malignancy for which there is a strong need for more effective systemic treatments. Approximately 8,000 people are diagnosed with intrahepatic and extrahepatic bile duct cancers annually in the U.S., with recent studies showing an increased incidence of cholangiocarcinoma, mainly attributed to recent advancements in diagnosis of this disease.

Surgery with complete resection remains the only curative therapy for cholangiocarcinoma, however only a minority of patients are classified as having a resectable tumor at the time of diagnosis. Additional treatment options include radiation therapy and chemotherapy; however, the efficacy of these treatments in cholangiocarcinoma patients is also limited. Despite overall advances in the ability to diagnose and treat patients with cholangiocarcinoma, the prognosis for these relapse patients who have failed initial chemotherapy remains very poor, with an overall median survival of approximately one year.

The 5-year relative survival rates of intrahepatic and extrahepatic cholangiocarcinoma patients range between 2% to 30%, depending on the tumor type and stage at diagnosis. Final results from the phase I study with YELIVA in patients with advanced solid tumors confirmed that the study, conducted at the Medical University of South Carolina Hollings Cancer Center, successfully met its primary and secondary endpoints, demonstrating that the drug is well-tolerated and can be safely administered to cancer patients at doses that provide circulating drug levels that are predicted to have therapeutic activity.

Of the three patients with cholangiocarcinoma treated in the Phase I study, all of whom had prior therapy, one subject achieved a sustained par-

tial response (Overall Survival = 20.3 months) and the other two subjects had prolonged stable disease (OS = 17.6 and 16.3 months).

RedHill said it plans to initiate a phase IIa clinical study with Yeliva in patients with advanced, unresectable, intrahepatic and extrahepatic cholangiocarcinoma in the third quarter of 2017. The single-arm study will evaluate Yeliva as a single agent in cholangiocarcinoma patients with a primary endpoint of determining the response rate of cholangiocarcinoma to this treatment.

A phase II study with Yeliva for the treatment of advanced hepatocellular carcinoma (HCC) is ongoing at MUSC Hollings Cancer Center. The study is supported by a \$1.8 million grant from the NCI, awarded to MUSC, which is intended to fund a broad range of studies on the feasibility of targeting sphingolipid metabolism for the treatment of a variety of solid tumor cancers, with additional support from RedHill.

A phase Ib/II study with Yeliva for the treatment of refractory or relapsed multiple myeloma is ongoing at Duke University Medical Center. The study is supported by a \$2 million grant from the NCI Small Business Innovation Research Program awarded to Apogee Biotechnology Corp., in conjunction with Duke University, with additional support from RedHill.

A phase I/II clinical study evaluating YELIVA in patients with refractory/relapsed diffuse large B-cell lymphoma as well as Kaposi sarcoma patients is ongoing at the Louisiana State University Health Sciences Center. The study is supported by a grant from the NCI awarded to Apogee, with additional support from RedHill. A phase Ib study to evaluate Yeliva as a radioprotectant for prevention of mucositis in head and neck cancer patients undergoing therapeutic radiotherapy is planned to be initiated in the third quarter of 2017, the company said.

## ESSA Pharma receives \$1.2 million grant payment from CPRIT

ESSA Pharma Inc., a pharmaceutical company focused on the development of novel small molecule drugs for the treatment of prostate cancer, announced today the receipt of a \$1.2 million payment from the Cancer Prevention Research Institute of Texas.

The payment is part of a total non-dilutive grant of \$12.0 million originally awarded in July 2014, and is repayable out of potential future product revenues. The payment recognizes eligible expenditures made by ESSA in conducting the phase I dose escalation clinical trial currently underway, and also costs incurred in preparation for the phase II dose expansion clinical trial expected to begin later this year.

The company is eligible to receive a further \$229,200 upon completion of financial and compliance filings with CPRIT.

“The financial support from CPRIT since 2014 has been instrumental to ESSA in building a top-tier team in Houston to guide the clinical development of EPI-506,” said David Parkinson, ESSA president and CEO.

The company initiated the phase I/II clinical trial of EPI-506 in late 2015 and continues to expand dosing patients in the Phase 1 portion of the study at sites in the U.S. and Canada. The clinical trial is designed to demonstrate the safety, tolerability, maximum tolerated-dose, pharmacokinetics and efficacy of EPI-506 in the treatment of prostate cancer patients who have failed treatments using abiraterone or enzalutamide or both, the current standard-of-care drugs in metastatic castrate-resistant prostate cancer.

## MD Anderson, Oncora Medical cooperate on precision radiation oncology

Oncora Medical, a precision radiation oncology software company, and MD Anderson Cancer Center announced a strategic alliance focusing on building the next generation of precision medicine software for radiation oncology.

During phase I, MD Anderson oncologists and information technology professionals will work with Oncora’s team of data scientists and engineers to install Oncora’s Precision Radiation Oncology Platform, a software system built to assist radiation oncologists in the development of personalized treatment plans based on outcome predictions.

Oncora’s platform will be fueled by data from MD Anderson’s electronic medical record system, tumor registry, radiation therapy planning system, and Brocade, an innovative software product developed by MD Anderson in 2014. Brocade was developed by Benjamin Smith, associate professor of radiation oncology, and author of a study published in the April 2016 issue of the Journal of the American College of Radiology that demonstrated a 70 percent reduction in the time physicians spend documenting clinical data using Brocade.

Brocade, a web-based clinical documentation tool used by MD Anderson radiation oncologists, enables intuitive collection of structured data about patient diagnosis, treatment and radiation side effects, and generates narrative-style clinical documentation for medical records. Oncora will engineer complete interoperability between their Precision Radiation Oncology Platform and Brocade to explore the potential value of a combined product.