THE CANCER LETTER

Feb. 6, 2015 • www.cancerletter.com • Vol. 41 No. 5

As Biosimilar GCSF Looms Over U.S. Market, Big Players Line Up for Emerging Competition

By Matthew Bin Han Ong

In the next few weeks, FDA will announce its decision on Novartis's Zarxio, a granulocyte-colony stimulating factor biosimilar to Amgen's Neupogen.

If it's approved—and outside observers uniformly believe it will be—Zarxio will become the first biosimilar agent to enter the U.S. market. Indeed, the FDA Oncologic Drugs Advisory Committee last month unanimously gave it thumbs up.

Being the first, Zarxio will provide a case study in the pricing of these agents.

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Conversation with The Cancer Letter

Conti: Don't Count on an 80% Price Drop From the Launch of Neupogen's Biosimilar

Introduction of biosimilar biologics will not bring about the same price drops as introduction of generic small-molecule drugs, said Rena Conti, an economist at the University of Chicago, whose work focuses on drug pricing.

With small-molecule drugs, price drops within two years of patent expiration and the introduction of generics can amount to 80 to 90 percent off the branded price.

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In Brief

Candance Johnson Named President of RPCI

CANDACE JOHNSON was named the 15th president and CEO of **Roswell Park Cancer Institute.** Johnson will be the first female leader for the 117-year-old comprehensive cancer center.

She served as deputy director of Roswell Park, as well as chair of the

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The question of price is particularly important, because an entire generation of expensive biologics is set to come off patent, and, with the stakes being high, major pharmaceutical companies are vying to enter the biosimilars business.

The most recent example: Pfizer earlier this week entered a definitive merger agreement to acquire Hospira, a major manufacturer of injectable, infused drugs and biosimilars. Pfizer is paying $90 a share in cash in a deal valued at about $17 billion.

"There are significant upfront costs for the biosimilar company related to learning how to make to make this drug, setting up the dedicated facilities to manufacture the drug and getting FDA approval to do so for the U.S. market," said Rena Conti, an assistant professor at the University of Chicago Department of Pediatrics, in the Section of Hematology/Oncology.

"That entails a series of inspections and fees. After the company builds the dedicated facilities for biosimilar production, FDA approval alone takes 18 months and possibly longer."

The Cancer Letter asked Conti to analyze the potential pricing and cost of Zarxio and its impact on the U.S. health care system. The conversation appears on p. 1.

Under normal circumstances, FDA consults ODAC on matters related to the design and conduct of clinical trials and clinical significance of safety and efficacy results.

In the case of Zarxio, the committee was also asked to review data on analytical similarities between the two agents and determine whether there was any meaningful clinical difference between the Amgen and Novartis versions of the GCSF.

Since ODAC is composed of clinical experts, it has few or no experts on analytical and manufacturing issues on which the biosimilar applications turn. Insiders anticipate that the advisory committee would now be augmented by outside experts in these areas.

Considering that Zarxio has been extensively used in Europe, it doesn't pose a daunting dilemma for the agency. Observers say the application went to ODAC because the biosimilar GCSF will set a precedent for future applications and thus presents an opportunity for the agency to discuss its approval standards at a public meeting.

The approval process could continue through March.

Zarxio is a biologic primarily indicated for treating and lowering the risk of chemotherapy-induced infections by increasing the number of white blood cells.

Manufactured by Sandoz Pharmaceuticals, a unit of Novartis, the agent has been marketed as Zarzio in Europe since its approval in 2009. According to Sandoz, usage of the drug outside the U.S. adds up to more than 7.5 million days of patient exposure.

No regulatory pathway existed in the U.S. for generic versions of biologic drugs until the Biologics Price Competition and Innovation Act of 2009 was passed as part of the Affordable Care Act that President Barack Obama signed into law on March 23, 2010.

The BPCI Act created an abbreviated licensure pathway for biological products like Zarxio.

A biosimilar can be licensed if it is shown to have the same mechanism of action, route of administration, dosage form, and strength as the reference biological product. Licensure is possible only for the indications of the reference biological product.

Under this act, products can be deemed either (1) biosimilar or (2) both biosimilar and interchangeable.

The latter category has an advantage: it allows pharmacists to use products interchangeably, without approval by a physician. The Novartis application was submitted for biosimilarity alone—not biosimilarity and interchangeability.

"This is not a bioidentical, it's a biosimilar. Identical properties are not necessary," Louis Weiner, director of the Georgetown Lombardi Comprehensive Cancer Center, said to ODAC during its hearing Jan. 7.

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Editorial, Subscriptions and Customer Service:
202-362-1809 Fax: 202-379-1787
PO Box 9905, Washington, D.C. 20016
General Information: www.cancerletter.com

Subscription $405 per year worldwide. ISSN 0096-3917. Published 46 times a year by The Cancer Letter Inc.

"The analytical components, the structure function and bioactivity are either identical or highly similar."

Though both clinical and analytical data were presented, ODAC spent most of the meeting focusing on clinical data. In the end, Zarxio aced ODAC, garnering a 14-0 vote with no abstentions.

"I voted yes, and I'm willing to bet my life on it," said patient representative Randy Hillard, a professor of psychiatry in the College of Osteopathic Medicine and the College of Human Medicine at Michigan State University.

All the data presented to ODAC pointed to convincing evidence for biosimilarity, said panel member Bernard Cole, a professor in the Department of Mathematics and Statistics at the University of Vermont.

"What really moved me was the very strong evidence shown by the sponsor for biosimilarity—numerous studies, the structure function, clinical performance," Cole said. "Although there appears to be some possibility of small differences in some [pharmacokinetics] parameters, the clinical data demonstrate equivalence in a critically important endpoint, namely, duration of severe neutropenia."

According to the agency, biosimilarity can be demonstrated by the following types of data:

• Analytical studies demonstrating that the biological product is "highly similar" to the reference product, notwithstanding minor differences in clinically inactive components

• Animal studies, including toxicity assessments.

• A clinical study or studies, including the assessment of immunogenicity and pharmacokinetics or pharmacodynamics, to demonstrate safety, purity, and potency in one or more appropriate conditions of use for which the reference product is licensed and for which licensure is sought for the biosimilar product.

The agency's guidance to industry states that it will use a totality of the evidence approach to review applications for biosimilar products.

FDA presentations repeatedly emphasized that the goal of the biosimilar application process is not to repeat the demonstration of safety and efficacy studies that led to the approval of the innovator agent. Rather, clinical trials in a biosimilar application should address residual uncertainty after the review of analytics, PK and PD studies.

A comparative clinical study is required if there are residual uncertainties about whether there are clinically meaningful differences between the biosimilar and the reference products, based on structural and functional characterization, animal testing, human PK and PD data, and clinical immunogenicity assessment.

How Generic GCSF Will Be Priced

Will the approval of Zarxio reduce its cost for patients? At the ODAC meeting, Georgetown's Weiner said yes.

"I'm a medical oncologist with an interest in targeted therapies and antibodies, and have experience with antibody engineering as well," said Weiner, who served as a consultant to Sandoz. "I'm here because I believe biosimilars offer enormous promise to reduce the cost and improve access to biologic agents for the treatment of cancer.

"GCSFs have been used widely around the world for over two decades. This is a molecule which has unquestioned clinical value that clearly helps patients," Weiner said. "It was shown that improved usage of GSCF can reduce emergency room admission rates significantly, from about one quarter down to about 10 percent with associated savings related to the cost of care necessitated by emergency room admissions and subsequent hospitalizations.

"While I believe it's pretty clear that by increasing the availability of reagents through the biosimilar approach, that competition will occur, that this competition will likely reduce costs, and the data from Europe support that. There has been increased utilization of guidelines since the institution of Zarzio [the European version of Zarxio], there have been improved clinical outcomes."

However, payers and patients should not expect the price of biosimilar GCSF to bring about the same price drops as generic small molecule drugs, economists say.

The reasons include the cost of proving biosimilarity and manufacturing the agents.

FDA has previously approved a drug with similar indications in August 2012 called Granix, by Teva Pharmaceutical Industries, another large biologics manufacturer. However, Granix, or tbo-filgrastim, was approved in an original biologics license application, not as a biosimilar to Neupogen.

Neupogen is a blockbuster drug that brought in more than $1 billion in sales for Amgen in 2014. Global sales of the agent decreased 36 percent year-over-year, mainly due to a $155 million order from the U.S. government in the third quarter of 2013, according to an Amgen statement on Oct. 27, 2014. Amgen acknowledged that underlying demand for Neupogen was "slightly" impacted by competition.

Amgen and Sandoz declined to comment on future pricing for Neupogen and Zarxio.

"We don't comment on potential uptake or sales forecasts, but we believe that customers and patients

in the U.S. can benefit greatly from a competitively priced filgrastim manufactured in accordance with strict GMPs and high quality standards. We would not comment on competitors in this context," Sandoz said to The Cancer Letter.

Though in the case of GCSF, Amgen, the sponsor of the branded product, is becoming a major player in the biosimilars arena.

In fact, Amgen has nine biosimilars in development, and expects to launch five of these biosimilar products between 2017 and 2019. www.amgenbiosimilars.com

"The initial three biosimilars (adalimumab, trastuzumab, bevacizumab) are all in pivotal studies, two additional biosimilars are clinical ready and four are in process development," an Amgen spokesperson said to The Cancer Letter. "The first ODAC meeting to discuss a proposed biosimilar was an important milestone in the progression of the biosimilars approval pathway.

"Amgen supports a science-based, patient-centric, regulatory framework for all biosimilars that will be approved in the U.S.

"Under the Biologics Price Competition and Innovation Act, FDA is granted the scientific discretion to determine clinical requirements for biosimilarity and, as a biosimilar sponsor, we are pleased with the approach the FDA is taking in its draft guidance and, we encourage a continued trajectory of sound, patient-based decisions for a sustainable pathway.

"All biologics, including biosimilars, are highly complex molecules and each manufacturer's product will be distinct. Therefore, FDA's commitment to sound science and manufacturer accountability will foster U.S. physician and patient confidence in biosimilars. Amgen believes that both transparency of clinical data and knowledge of the specific product administered to a patient will be integral to establishing this confidence.

"We appreciated the meeting's discussion, and while we do not have all of the information available to the FDA, we will be interested in the FDA's ultimate approval decision."

Sandoz: Cost—Not Price of Drug—Will Drop

Responding to vigorous questions from the advisory committee, Sandoz said that the acquisition cost of Zarxio would likely be lower for payers and patients.

However, Sandoz cannot promise that the price of Zarxio would be lower than Neupogen due to complicating factors, such as negotiations with specialty pharmacies, said Mark McCamish, global head of development for Sandoz, to panel member James Liebmann, an assistant professor of medicine from the University of Massachusetts.

The text of their exchange follows:

James Liebmann: In previous meetings with this committee that I've been to, I think that cost has been sort of the elephant in the room that nobody acknowledges, and I was actually pleased to see that your consultant [Louis Weiner] acknowledged it prominently, and said that he expects that if this is approved, this will lead to significantly lower costs.

I then noticed that in the final estimation from the company, there was no mention made about that. So my question is: Is the consultant correct? Would this really bring down costs? (Laughter.)

Mark McCamish: I like elephants in the room, so let's talk about that. That's our passion, to have an impact on use, and we do that through cost.

So let me give you a bit of information about our European experience, and then I'd like to ask Dr. Blackwell to come out and comment on the clinical side as well, based on her experience, and how she would use this in anticipation and access.

In Europe, with the introduction of the biosimilar in 2009, there has been a substantial increase in the use, so we are addressing access, and there has been a substantial reduction in cost because of the competition that's there.

JL: May I just suggest that pricing in the United States and health care is markedly different than pricing in Europe. So I'm not sure that that's a relevant model to point to.

MM: I agree with you that the models are in fact different, as is price, but what I was mentioning is cost, so you're absolutely right. Price will be very complicated, and it could be that our price would be at parity, but the cost would be lower. And there are all sorts of things that come into that, whether it's rebates and other types of situations.

But what I can give you is the experience we've had. In Europe, there are many different systems—some of which may be more applicable than others, and that has had a huge impact on the use as well as on price.

And most people acknowledge a 20 to 30 percent price reduction, but it depends on the state and the area.

Dr. Blackwell, if you would like to come up and comment on this?

Kimberley Blackwell: Sure. I'm Dr. Kimberley Blackwell, a medical oncologist, and I do have a conflict in that I'm being compensated for being here today as well as my participation in the [Data and Safety Monitoring Board].

As an American medical oncologist, I've not had an opportunity to prescribe Zarxio to my patients, but I think it is an elephant in the room, not just in terms of cost, but access to some of these very costly supportive care medicines, and as someone who sees patients three full days a week, it's not just the total cost of the drug, it's access.

It's the copays associated with it, it's the formulary decisions, and even in this week, I've had patients receiving adjuvant TAP chemotherapy, who have chosen to actually take off work to come and get their GCSF so that they don't have to pay the $20 to $40 copay associated with the cost of some of these medicines. It's not even the total cost, it's the cost to the patient and it's the cost to society.

So although I can't predict what the pricing would be—the sponsor would have to address that, and hopefully make a significant contribution to the cost to the patient, whatever that might be. Thank you.

JL: I was just hoping that this…you know, let's be honest—in fact, it's not complicated. There is a price of Neupogen. You could simply say that, as a new entrance to the market—and I don't expect you to, and trust me, I'm not going to base my vote on the cost, because that's not an issue that comes up in our vote here—but you could simply say, 'Yes! We're going to price it less than Neupogen.' Alright? And, if you're honest, that will be delightful.

MM: I understand. Let me say that we can't say that the price will be less, because in some situations, the price will be at parity, because of other relative terms that will come into existence. That's there. The cost will be less to the consumer, to the payer, to the health care economy. It has to be. Otherwise, it doesn't make sense, but price is a relatively complex situation.

I can give you examples. Now, this is the biosimilar file to come to the States. We have had experience with a biosimilar drug that we took through a 505(b)(2) approach in the States, because the 351(k) wasn't available. And that's another protein growth hormone. And we were the seventh to the market with growth hormones, and when we came to the market—this was quite a ways back—there was a learning on our part, because of the complexities that you've actually mentioned.

And we priced this quite low from the beginning, and that reduction was substantial, almost half. With that, we had difficulties selling the drug at all, because the incentive for a specialty pharmacy was that they get a percentage of the price of the drug, and that's about a 6 percent incentive.

So by pricing it that low, they had a huge disincentive not to use the drug. Now, for managed care organizations, that disincentive doesn't exist, because they're looking at the total overall price, and with that we had very good penetration, very good use.

But that was a huge learning to us, that price is not as easy as one would expect, and we can't just say that price is going to be X, because various components work differently. But the reality is, we moved from number seven in the marketplace, to competing with number two or three because the cost of using our product was lower.

A Nonproprietary Labeling Dilemma

It's unclear whether the Novartis product and the Amgen product, would share the nonproprietary name filgrastim. The agency hasn't announced guidelines on nonproprietary, or generic, naming of these drugs.

During the public hearing portion of the ODAC meeting, several organizations expressed concerns about the potential for confusion and asked the agency to set policy on label regulation.

Overall, sponsors of reference products are more likely to wish to distinguish their agents from biosimilars, while sponsors of biosimilars would prefer not to see this distinction.

Also, groups that focus on drug safety argue that distinct names—perhaps differentiated by a prefix or another identifier—would make it easier to separate adverse events related to reference products from those related to biosimilars.

Groups interested in fostering greater use of less expensive drugs advocate having the biosimilars and reference products share the generic name.

"To ease confusion among prescribers, pharmacists and patients, approved biosimilars must be permitted to use the same international, nonproprietary name as the reference product," said Mary Jo Carden, senior director of regulatory affairs at the Academy of Managed Care Pharmacy. "This will help encourage substitution of biosimilars when appropriate, by ensuring consistency among products and ensure comparable safety and efficacy based on FDA standards.

"The use of manufactured names, national drug codes, and lot numbers may continue to effectively differentiate batches for purposes of safety monitoring. FDA must provide specific rules for the designation of interchangeable products."

Richard Markus, vice president of global development for Amgen's biosimilars portfolio, recommended specific policies for regulating biosimilars.

"In 2020, there could be 10 biologic medicines, each with four biosimilars. Including the reference biologics, that's 50 unique products that need to be accurately tracked and can independently be accountable for the safety period potency of their products," Markus said to the advisory panel. "It's to those ends that we urge the FDA to adopt the following scientific and public health policies:

"One, nonproprietary naming should be distinguishable for every biologic, including biosimilars, to enable accurate medical records, manufacturer accountability and informed appropriate use.

"Two, product labeling should be specific and transparent. The prescribing information should identify the product as biosimilar or interchangeable and should identify the pivotal clinical safety and efficacy data.

"And three, when appropriate, post-market studies should be carried out to further assess immunogenicity in the most sensitive populations, especially if those are extrapolated indications.

"Policies related to interchangeability designations must address both scientific and real-world considerations, including requiring studies in sensitive patient populations and multiple mechanisms of action accounting for multiple interchangeable biologics, each compared only to the reference product and not to each other, and preventing inappropriate and inadvertent substitution of non-interchangeable biologics."

The generic name filgrastim should be on both the Zarxio and Neupogen labels, panel member Liebmann said.

"I was impressed that so many of the public statements had to do with the name of the drug," Liebmann said in his closing comments. "I think that this has been clearly shown to be filgrastim, in fact, and I think that to name it anything else would be misleading."

The FDA guidances on biosimilars are available on the agency's website, with additional information here.

Conversation with The Cancer Letter

Rena Conti Lays Out Impact of Biosimilars on U.S. Health Care

(Continued from page 1)

The price drop for biologics when biosimilars enter the market will be less dramatic, in part because only a small number of companies have the ability to produce these agents, reducing the competitive pressure that drives down prices. In addition, the costs of manufacturing biosimilar agents is higher than those associated with manufacturing generic small-molecule agents.

The Cancer Letter asked Conti, an assistant professor at the University of Chicago Department of Pediatrics in the Section of Hematology/Oncology and Public Health Sciences, to trace the manner in which any cost savings from biosimilar agents would reverberate through the U.S. drug distribution system.

Two factors complicate the price drops from biosimilar biologics:

"The first is that there are not that many manufacturers that have the scale and knowledge to make injectable or infused drugs with similar requirements to biologics," Conti said.

"The big companies supplying the U.S. market with generic infused and injectable drugs are Hospira, Fresenius and Teva. There have also been some significant mergers and acquisitions between branded and generic pharmaceutical companies in recent years. Just this past week, Pfizer announced they were purchasing Hospira.

"That implies to me that we may not see a lot of supplier entry into the biosimilar biologics market. There are simply not that many companies that have the capability to make these drugs.

"With small-molecule drugs, we can see the number of independent generic companies entering into the generic market to sell a top selling 'blockbuster' drug, like Prozac, on the order of eight and 12. That is not going to occur here.

"There's another reason to expect that the generic price of biosimilar drugs will not drop as low as generic small-molecule drugs, related to their costs of production:

"The incremental cost of making generic small-molecule drugs is essentially zero. The incremental costs of making the biosimilar biologics are not zero. In fact, they are likely significant.

"These costs will get passed on to purchasers."

Conti spoke with the Matthew Ong, a reporter with The Cancer Letter.

Matthew Ong: *How does the manufacturing process for biologic drugs affect market pricing? Are they more expensive and difficult to make?*

Rena Conti: Making these drugs is more costly than your average small-molecule, orally administered drug.

The expenses are largely related to the acquisition of the base chemicals and putting them into a manufacturing process that is sterile, and that reliably produces a drug on the other end that has no safety or quality concerns, and meets the "biosimilar" definition established by FDA in relation to the original branded drug.

The base ingredients for these drugs can be of variable quality and supply. The prices of these base ingredients can fluctuate on the international market for many reasons.

In addition, the expertise and processes that ensure biosimilarity and consistent control requires a lot of ongoing investment and vigilance.

There are also significant upfront costs for the biosimilar company related to learning how to make to make this drug, setting up the dedicated facilities to manufacture the drug and getting FDA approval to do so for the U.S. market.

That entails a series of inspections and fees. After the company builds the dedicated facilities for biosimilar production, FDA approval alone takes 18 months and possibly longer.

There are just not that many companies who have the in-house knowledge and capacity to manufacture these agents. There are gains to scale and scope from manufacturing these types of agents. Only a handful of companies are going to have the ability to manufacturer biosimilar GCSF.

MO: *You're saying we can't expect a slow slide from monopoly to perfect competition in this case?*

RC: Exactly.

Biosimilar GCSF is going to be demanded by many purchasers—Medicare spends billions annually on branded Neupogen. Biosimilar entry into this market is likely worth hundreds of millions of dollars over the next couple of years.

In a best-selling agent like this, one would expect there would be a lot of manufacturers who would want to enter the biosimilar market over time after the period of biosimilar generic exclusivity ends for Sandoz. When more manufacturers enter, the price should drop.

In the case of biosimilar GCSF, constrained supply and non-zero production costs will significantly complicate these expectations.

MO: *Another interesting thing that the sponsor highlighted during the ODAC hearing was that while the cost to patients might be reduced, they cannot promise that the price would not be at parity to Neupogen. Can you explain that dynamic?*

RC: Sure.

Neupogen is not a drug sold in the retail pharmacy market. It is a drug infused into patients. Typically, hospitals and doctors' offices purchase these agents and then administer them to patients.

This means there are two prices operative in this market—one price is the wholesale acquisition price that hospitals and physician's offices pay for acquiring the drug another price is the reimbursement a patient and their insurer are required to pay to the hospital or physician office for the drug's use.

It is clear that biosimilar GCSF's wholesale acquisition cost to the hospital and physicians' offices will be lower than that charged for branded Neupogen.

It is not clear that patient's out-of-pocket spending on biosimilar GCSF treatment will differ much from that associated with branded Neupogen treatment.

Typically, if you're a patient insured by Medicare's fee-for-service program—the dominant payer of branded neupogen treatment—your copayment amount for getting Neupogen treatment is set at 20 percent of Medicare's reimbursement.

But most people insured by Medicare's fee-for-service program have supplemental insurance coverage that pays the 20 percent copayment for treatment with infused drugs in the outpatient setting.

As a consequence, the potential price savings from the availability of biosimilar GCSF for treatment in the outpatient setting may accrue over time to hospitals and physicians' offices and insurers, but won't be felt by patients in the form of lower out of pocket costs for their treatment. It is likely these cost savings won't get passed onto patients in the form of lower deductibles or insurance premiums either.

This is important for the overall cost savings that biosimilar GCSF will have in the market because consumers' price sensitivity will likely not determine the use of biosimilar GCSF when it becomes available.

MO: *Sandoz also delineated the difference between specialty pharmacies, as opposed to managed care organizations, during the ODAC hearing. How might this be a factor in their pricing strategies?*

RC: Specialty pharmacies handle drugs that are infused and injected or require other types of special handling.

It is an alternative distribution channel for these types of drugs. It works differently than the system I

just described where hospitals and physician offices acquire the drug at a wholesale price, using it to treat a patient and then waiting for reimbursement from insurers and patients.

In this alternative system, patients receive a prescription for these drugs and get it filled at a specialty pharmacy. The patient can walk the drug over to their doctor's office for infusion therapy, a behavior sometimes called "brown-bagging" or more commonly the physician's office fills the prescription on behalf of the patient and the pharmacy ships the drug to the office in anticipation of the patient's appointment, a process colloquially called "white-bagging."

This is becoming an increasingly common set of practices among some hospitals and physician practices that do not wait to risk their practice solvency based on insurer reimbursement policies.

When a drug is distributed through the specialty pharmacy channel, patients can face significant copayment amounts. Here, insurer policies will determine how much patients will save when patients are prescribed biosimilar GCSF, rather than branded Neupogen. In addition, consumers' price sensitivity may help determine the use of biosimilar GCSF when it becomes available.

However, specialty pharmacies do not face any direct financial incentives to stock and dispense biosimiliar Neupogen over branded Neupogen when faced with a given patient's prescription. This is because they are paid a dispensing fee that is not related to the acquisition costs or reimbursement price paid by insurers.

When a small-molecule drug loses patent protection and becomes available in generic form, generic substitution by pharmacies is allowed or sometimes even mandated, although in some states pharmacists must contact the prescribing physician for permission to substitute.

It is unclear whether generic substitution policies apply to biosimilar agents, such as Neupogen.

As a consequence, the importance of specialty pharmacies in the distribution of Neupogen-based treatment is a big deal for assessing how much savings the availability of biosimilar GCSF will have for payers in the next couple of years.

While insurers may stand to save a lot of money from the use of biosimilar GCSF over branded Neupogen, some distribution channels for these drugs simply don't face incentives that are aligned with payers.

Medicare to Begin Coverage Of CT Lung Cancer Screening

By Paul Goldberg

Computed tomography screening has become a benefit for Americans covered by Medicare.

The Centers for Medicare and Medicaid Services Feb. 5 published a final decision to cover screening of current and former smokers, provided they meet stringent eligibility criteria.

Beneficiaries will go through counseling, health professionals will be required to provide documentation that "shared decision-making" took place, technical criteria for screening will be met, and data will be collected. CMS has never mandated shared decision-making as a gateway to paying for a service.

The agency's final decision largely follows the draft decision published Nov. 10, 2014 (The Cancer Letter, Nov. 14, 2014).

The age parameters in the final decision are 55 to 77. In the draft decision, the age eligibility was set as 55 to 74, which matched the ages screened in the NCI National Lung Screening Trial.

However, the U.S. Preventive Services Task Force, when it assessed lung screening, relied on modeling to project benefit to a larger age group: ages 55 to 80.

To be eligible, the patients will have to have a 30 pack-year smoking history, be current smokers, or have quit within the past 15 years. A pack-year amounts to smoking one pack per day for a year.

The American College of Radiology Lung Cancer Screening Registry has applied for CMS approval to help providers efficiently meet registry reporting requirements.

The decision a landmark case in setting payment policy:

• It translates the findings of the NLST, a large randomized trial conducted by NCI into payment policy and standards of care in the community (The Cancer Letter, Nov. 5, 2010).

• It draws on the recommendation of the U.S. Preventive Services Task Force (The Cancer Letter, Aug. 2, 2013, Aug. 9, 2013, Jan. 10, 2014). With the B grade from USPSTF, lung screening became an Essential Health Benefit under the Affordable Care Act, which means that private insurers will have the obligation to cover the service next year.

• It's informed by the clinical judgment of a CMS advisory committee, called the Medicare Evidence Development & Coverage Advisory Committee, or

Data Type	*Minimum Required Data Elements*
Facility	Identifier
Radiologist (reading)	National Provider Identifier (NPI)
Patient	Identifier
Ordering Practitioner	National Provider Identifier (NPI)
CT scanner	Manufacturer, Model.
Indication	Lung cancer LDCT screening – absence of signs or symptoms of lung cancer
System	Lung nodule identification, classification and reporting system
Smoking history	Current status (current, former, never). If former smoker, years since quitting. Pack-years as reported by the ordering practitioner. For current smokers, smoking cessation interventions available.
Effective radiation dose	CT Dose Index (CTDIvol).
Screening	Screen date Initial screen or subsequent screen

Source: Decision Memo for Screening for Lung Cancer with Low Dose Computed Tomography (CAG-00439N)

MEDCAC (The Cancer Letter, May 9, 2014),

• It rejects several key aspects of a proposal for broader coverage, advanced by patient groups, researcher Claudia Henschke's International Early Lung Cancer Action Program, and some professional societies (The Cancer Letter, March 21, 2014, April 18, 2014, June 13, 2014, Jan. 9, 2009, March 28, 2008, March 14, 2008, Nov. 3, 2006).

"The CMS decision is very appropriate," said Otis Brawley, chief medical officer of the American Cancer Society. "It recognizes the benefits and the harms demonstrated in the randomized screening trial done in 33 extraordinary university hospitals. It takes measures to assure that positive benefit to harm ratio is available to all those who undergo screening throughout the U.S. It also encourages patients be informed of those benefits and risks and be allowed to decide for themselves about screening."

David Howard, a member of MEDCAC and a professor at the Emory University Department of Health Policy and Management, estimates that in 2012 there were 3.2 million Medicare-eligible persons between ages 55-74 who have at least 30 pack-years of smoking history and are current smokers or quit within the past 15 years.

By way of comparison, there were 3.9 million persons ages 55-80 who have at least 30 pack-years of smoking history and are current smokers or quit within the past 15 years.

These figures were calculated using the Health and Retirement Study, a national survey of persons 50 years and older.

The decision largely follows the elements of a letter requesting that the agency conduct "a national coverage analysis" for lung screening, submitted by Peter Bach, director of the Center for Health Policy and

Outcomes at Memorial Sloan Kettering Cancer Center.

The policy is also consistent with joint guidelines from the American College of Chest Physicians and the American Thoracic Society.

Though the CMS decision requires collection of data, this decision isn't made under the agency's Coverage with Evidence Development, or CED.

• The CED mechanism requires collection of data for the purposes of formulating a coverage decision in the future. In this case, the coverage decision has been made.

• In the context of CED, participating cites operate under approval of the Institutional Review Board, as a clinical experiment, which requires formal informed consent and is subject to rules and regulations governing research.

• In the case of this coverage decision, consent is replaced by documentation that demonstrates that shared decision-making has occurred and that the provider meets the criteria for providing the service. The two parties involved in this are the patient and the healthcare provider. Had this been a CED, a research investigator would be involved in the interaction.

The CMS decision doesn't fully meet the requests of some advocacy groups and professional societies.

In a joint letter and a 43-page paper addressed to CMS, a group of professional societies and advocacy groups pressed for broader coverage (The Cancer Letter, Oct. 3, 2014).

They sought:

• Unrestricted broad national coverage of LDCT lung cancer screening for the patient population recommended by the USPSTF with standards and a clinical practice registry, plus

• Coverage with Evidence Development for other high-risk individuals where evidence is promising to inform future coverage decisions. This would include "category 2" patient groups identified in the National Comprehensive Cancer Network screening guideline.

This category of current and former smokers includes individuals with a 20 pack year history who have at least one additional risk factor for lung cancer, such as self-reported occupational exposure, previous cancers, chronic obstructive pulmonary disease or pulmonary fibrosis, high radon exposure, a family history of cancer.

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Obama Proposes $1 Billion Increase for NIH in 2016

By Matthew Bin Han Ong

NIH would receive a $1 billion funding boost in President Barack Obama's $4 trillion 2016 budget—a 3 percent increase—should Congress pass his proposal.

The additional funds would bump the NIH budget to $31.3 billion, which the White House said would support greater research in cancer, Alzheimer's and other diseases. The proposal provides $38.8 billion in discretionary funding to the Department of Health and Human Services.

The proposal also includes $135 million for NIH's contribution to the BRAIN Initiative, a research project announced by Obama on April 2, 2013, aimed at accelerating the development and application of innovative technologies to map the human brain.

The president's new precision medicine initiative, announced Jan. 20 during his State of the Union address, would receive $215 million (The Cancer Letter, Jan. 30).

"Under 2016 sequestration levels, assuming roughly current funding patterns, research funding adjusted for inflation would reach its lowest levels since 2002—other than when sequestration was in full effect in 2013," the White House said in the overview of the proposal. "By comparison, the Presidents' Budget would increase funding by nearly 6 percent over 2015, including investments in Precision Medicine, the BRAIN Initiative, and other areas."

Other budget increases include: $1.2 billion for an inter-department project by HHS, Department of Defense, Department of Veterans Affairs, and the Department of Agriculture to combat antibiotic-resistant bacteria, and increasing FDA's budget 9 percent, to $4.9 billion.

In a section of the budget overview titled "Reversing Mindless Austerity," the White House proposes to end sequestration—fully reversing it for domestic priorities in 2016—and matched by equal dollar increases for defense funding.

"We will make these investments and end the harmful sending cuts known as sequestration, by cutting inefficient spending, and closing tax loopholes," Obama said in his budget message.

The proposal calls for $400 billion in reductions to federal health spending, including increases in Medicare premiums, particularly for wealthier seniors, and a surcharge on supplemental Medicare insurance plans.

"This budget accurately reflects the challenges

FDA faces in a global regulatory environment, which is becoming increasingly complex and scientifically demanding," said FDA Commissioner Margaret Hamburg, who will be stepping down in March 2015. "As FDA's mission expands on several fronts—from the regulation of tobacco products to supporting the development of personalized medicine to ushering in a new era of food safety—we must possess the resources to run a modern agency that fosters innovation and ensures the safest possible drug and food supply for the American people."

The 5.5 percent increase for research and development is critical and desperately needed, said Joseph Haywood, president of the Federation of American Societies for Experimental Biology.

"They should be part of a multi-year investment in the nation's future," said Haywood. "We call upon Congress to pass legislation to ensure that we are able to maintain our leadership in science and technology."

The proposal would help the biomedical research enterprise recoup its losses from flat funding and inflation, said Research!America President and CEO Mary Woolley.

"We are pleased that the President's FY16 budget proposal calls for the elimination of sequestration and makes a down payment on the bipartisan goal of accelerating medical progress. We see this as starting point," Woolley said. "It is absolutely important to invest in initiatives that focus on precision medicine, Alzheimer's, antimicrobial resistance and other growing health threats, but these investments should supplement, not supplant, the imperative of making up for a decade's worth of lost ground.

"We believe that Congress and the White House can, and must, unify behind a moonshot as envisioned in the bipartisan Accelerating Biomedical Research Act. Medical progress is not just a health imperative, it is a strategic imperative, integral to national security, fiscal stability and economic progress. Leaders on both sides of the aisle clearly appreciate that the time is now to turn ideas into reality. It may be a truism, but where there's a will, there's a way."

United for Medical Research President Carrie Wolinetz commended the proposal for making de-sequestration a top priority.

"We welcome President Obama's FY16 budget proposal to increase National Institutes of Health funding and eliminate harmful sequestration," Wolinetz said. "NIH has fostered remarkable advancements in human health, but has suffered from inadequate funding for the past decade. Additional resources will help defeat our nation's most harmful diseases—including cancer, heart disease and diabetes—and fuel job creation in the life sciences sector—a win-win.

"We also commend the president for his precision medicine proposal. Investing in precision medicine and NIH 'patient-powered research' will continue to transform how diseases are treated, harnessing the power of the human genome, heath informatics and medical imaging to better understand individual patients' unique needs.

"Precision medicine is an extraordinary example of how previous research discoveries build the foundation from which to launch cutting edge medical advancement, illustrating how NIH funding of today saves lives both present and future.

"Given the many economic, societal and health benefits borne from investments in medical research, we call on lawmakers on both sides of the aisle to make increasing NIH funding and eliminating sequestration a top priority in FY16 and beyond."

FDA Commissioner Hamburg To Step Down Next Month

By Matthew Bin Han Ong

FDA Commissioner Margaret Hamburg announced that she will be stepping down at the end of March 2015.

In an email to colleagues Feb. 5, Hamburg, 59, reflected on her six-year run in the top job at the agency, and called her decision to leave "not easy."

"My tenure leading this agency has been the most rewarding of my career, and that is due in no small part to all of you," she wrote.

Hamburg was nominated by President Barack Obama and confirmed by the Senate in 2009.

"As hard as it is to leave this agency, I am confident that the leadership team that we have in place will enable FDA to capitalize on, and improve upon, the significant advances we've made over the last few years," Hamburg wrote. "Many of these leaders have been with the FDA throughout my tenure, and I am proud to say that we've recently made some wonderful new additions.

"And with respect to the agency's senior leadership team, I am pleased that Dr. Stephen Ostroff has agreed to serve as acting commissioner when I step down. Since joining the agency in 2013, and most recently serving as FDA's chief scientist, Dr. Ostroff has successfully overseen numerous significant

initiatives, while helping to ensure that scientific rigor, excellence and innovation are infused across the agency. I have every confidence that he will take on this new role with the same energy, dedication and care."

Last week, Robert Califf was named FDA Deputy Commissioner for Medical Products and Tobacco, a de facto No. 2 post at the agency (The Cancer Letter, Jan. 30).

Califf, 63, an expert in cardiology, clinical research, and medical economics, is leaving his job as vice chancellor of clinical and translational research at Duke University. He will join the agency in late February.

He will oversee the FDA Center for Drug Evaluation and Research, the Center for Biologics Evaluation and Research, the Center for Devices and Radiological Health, and the Center for Tobacco Products. He will also oversee the Office of Special Medical Programs in the Office of the Commissioner.

Research advocates commended Hamburg for her service.

"Under Dr. Hamburg's leadership, we have made great strides in cancer treatment and in how we implement precision medicine," said Richard Schilsky, chief medical officer of the American Society of Clinical Oncology. "Dr. Hamburg has been at the forefront of ensuring that the FDA is keeping pace with this rapid scientific change and using available mechanisms to speed patient access to safe and effective therapies."

Schilsky said Hamburg's contributions included increasing the speed and efficiency of medical product reviews and expansion of FDA-expedited approval mechanisms, including the development of the Breakthrough Therapies designation for therapies to help patients with serious or life-threatening diseases.

According to ASCO, nearly half of the novel new drugs approved received expedited approval, and the agency established a regulatory pathway for biosimilar biological products as well as approval of companion diagnostic tests.

"Commissioner Hamburg's leadership and her commitment to patients are unsurpassed," said Ellen Sigal, chair and founder of Friends of Cancer Research. "She has changed the direction of the FDA, creating an environment of science-based collaboration that has fostered a new era of regulatory science focused on expediting the best treatments to patients."

Hamburg has worked to hasten the pace at which safe and effective drugs and medical products reach patients, said Research!America President and CEO Mary Woolley.

"She has worked to accelerate regulatory science to further medical progress and has recognized public-private partnerships as essential to fulfilling the agency's mission," Woolley said. "Her commitment to patient engagement and science-based regulation has led to an impressive increase in FDA approval of new molecular entities and biological products including a record high of 42 FDA approved orphan drugs and novel therapeutic biologic products in 2014.

"During her tenure, average FDA review times have been faster than regulatory agencies in other countries, contributing to our nation's well-earned role of global leadership in innovation. Patients with serious or life-threatening diseases have a new avenue of hope with the FDA's Breakthrough Therapy Designation, which allows the agency to expedite the development and review of certain drugs.

"Overall, her tireless efforts to streamline the drug approval process will leave a lasting imprint on patient care. We are deeply grateful for her public service."

The full text of Hamburg's email to FDA staff follows:

Dear FDA Colleagues:

It has been a privilege to serve as your FDA Commissioner for almost six years. So it is with very mixed emotions that I write today to inform you that I plan to step down as FDA Commissioner at the end of March 2015.

As you can imagine, this decision was not easy. My tenure leading this Agency has been the most rewarding of my career, and that is due in no small part to all of you—the dedicated and hard-working people that make up the heart of this Agency. While there is still work ahead (and there always will be), I know that I am leaving the agency well positioned to fulfill its responsibilities to the American public with great success.

I feel so fortunate to have worked at an organization as remarkable and productive as the FDA. The expertise, dedication and integrity of our people and the unique nature and scope of FDA's role make this Agency truly special.

Every day, FDA employees around the world recommit themselves to the Agency's work, to quality science, to facilitating innovation, and to the protection of public health. And because of your dedication and your service, we have been able to achieve so many significant milestones over the past years.

From creating a modernized food safety system

that will reduce foodborne illness; advancing biomedical innovation by approving novel medical products in cutting-edge areas; and responding aggressively to the need to secure the safety of a globalized food and medical product supply chain, to taking critical steps to reduce the death and disease caused by tobacco, we have accomplished a tremendous amount in the last six years.

We can honestly say that our collective efforts have improved the health, safety and quality of life of the American people.

At the heart of all of these accomplishments is a strong commitment to science as the foundation of our regulatory decision-making and of our integrity as an Agency. And while there are far too many significant actions, events, and initiatives to count, there are some highlights of the past years that I particularly want to mention.

In the foods area, we have taken critical actions that will improve the safety of the food Americans consume for years to come. These include science-based standards developed to create a food safety system focused on preventing foodborne illness before it occurs, rather than responding after the fact.

We have taken several significant steps to help Americans make more informed and healthful food choices. These include working to reduce trans fats in processed foods; more clearly defining when baked goods, pastas and other foods can be labeled "gluten free;" updating the iconic Nutrition Facts label; and, most recently, finalizing the rules to make calorie information available on chain restaurant menus and vending machines.

We have also made great strides in advancing the safety and effectiveness of medical products. Some of these important steps include new oversight of human drug compounding and provisions to help secure the drug supply chain so that we can better help protect consumers from the dangers of counterfeit, stolen, contaminated, or otherwise harmful drugs.

We are continuing to increase the speed and efficiency of medical product reviews. We just had another strong year for novel drug approvals, with most of these drugs being approved on or before their PDUFA goal dates and most being made available to patients in the United States before they were available to patients in Europe and other parts of the world.

We launched a powerful new tool to accelerate the development and review of "breakthrough therapies," allowing FDA to expedite development of a drug or biologic to help patients with serious or life-threatening diseases. In fact, almost half of the novel new drugs approved in 2014 received expedited review with a combination of breakthrough designation, priority review and/or fast track status.

These included drugs for rare types of cancer, hepatitis C, type-2 diabetes and idiopathic pulmonary fibrosis, as well as a number of groundbreaking vaccines. We have also established a regulatory pathway for biosimilar biological products that will create more options for patients.

On the medical device side, the average number of days it takes for pre-market review of a new medical device has been reduced by about one-third since 2010. The percentage of pre-market approval (PMA) device applications that we approve annually has increased since then, after steadily decreasing each year since 2004.

We also published the Unique Device Identification (UDI) final rule that is intended to improve the tracking and safety of medical devices. And we proposed a risk-based framework for laboratory developed tests (LDTs) to help ensure patients and providers have access to safe, accurate and reliable tests, while continuing to promote innovation of diagnostic tests to help guide treatment decisions.

We have ushered in the era of personalized medicine across all of our medical product centers. For example, many cancer drugs are increasingly used with companion diagnostic tests that can help determine whether a patient will respond to the drug based on the genetic characteristics of the patient's tumor. A growing percentage of our recent approvals have involved targeted therapies, offering many patients more effective response profiles and/or reduced likelihood of side effects.

We made significant progress in implementing both the letter and spirit of the Family Smoking Prevention and Tobacco Control Act. Our tobacco compliance and enforcement program has entered into agreements with numerous state and local authorities to enforce the ban on the sale of tobacco products to children and teens; conducted close to 240,000 inspections; written more than 12,100 warning letters to retailers; proposed the extremely important foundational "deeming" rule; and broken new ground for FDA with the launch of the Agency's first public education campaigns to prevent and reduce tobacco use among our nation's youth.

As Commissioner, my goal has been to shape and support an FDA that is well-equipped to meet the challenges posed by scientific innovation,

globalization, the increasing breadth and complexity of the products that we regulate, and our new expanding legal authorities.

I have worked hard to advocate for FDA and our unique and essential mission, including building new partnerships to support our work. The Agency has received numerous votes of confidence with the bi-partisan enactment of a series of landmark bills extending our authority in the areas of tobacco, food safety and medical products

In addition, we have achieved a dramatic increase in our budget, from some $2.7 billion in FY2009 to almost $4.5 billion in FY2015.

As hard as it is to leave this Agency, I am confident that the leadership team that we have in place will enable FDA to capitalize on, and improve upon, the significant advances we've made over the last few years. Many of these leaders have been with the FDA throughout my tenure, and I am proud to say that we've recently made some wonderful new additions.

And with respect to the agency's senior leadership team, I am pleased that Dr. Stephen Ostroff has agreed to serve as Acting Commissioner when I step down. Since joining the Agency in 2013, and most recently serving as FDA's Chief Scientist, Dr. Ostroff has successfully overseen numerous significant initiatives, while helping to ensure that scientific rigor, excellence and innovation are infused across the Agency. I have every confidence that he will take on this new role with the same energy, dedication and care.

I want to extend my deepest gratitude to each and every one of you for your service and for making FDA an agency that is not only an exciting and rewarding place to work, but also a place of remarkably meaningful achievement and impact on the health and well being of Americans.

Margaret A. Hamburg, M.D.
Commissioner of Food and Drugs

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World Cancer Day

UICC: Adding $18 Billion a Year Could Drop Cancer Deaths By 30% in Poorer Countries

New data projects that an $18 billion increase in funding per year by the international community could result in a 30 percent reduction in cancer deaths in low- and middle-income countries by 2030.

On World Cancer Day 2015, held annually Feb. 4, public health experts from the Union for International Cancer Control said that millions of lives can be saved through affordable increases in the investment into cancer services throughout the world.

• Increased annual international community funding of $18 billion globally could save three million lives per year by 2030 and many more in succeeding decades, through prevention, earlier detection and improved care.

• Increased funding will also provide pain relief to ease the deaths of millions who will die of cancer during this period.

• A tripling of tobacco taxes alone would raise tax revenue available to governments to $400 billion annually, and could encourage one-third of smokers to quit, according to new figures.

"More than eight million people a year die from cancer, of which more than 60 percent of those deaths occurred in low- and middle-income countries, the majority in the middle-income segment," Tezer Kutluk, president of UICC, said in a statement.

"The absolute number of cancer cases in developing countries is set to rise dramatically because of population growth and aging, so action must be taken now. Whilst the US$18 billion package is affordable for many countries, it's unrealistic to expect the world's poorest nations to contribute to this investment without international support."

The World Health Organization recommended "best buys" for non-communicable diseases in 2011, including these cancer prevention interventions:

• Tobacco taxation, regulation and control to reduce tobacco-related cancers.

• Hepatitis B vaccination to prevent liver cancer.

• Screening and treatment for precancerous cervical lesions.

DCP3: Disease Control Priorities, Third Edition, adds treatment and pain control interventions to this list:

• HPV vaccination for adolescent girls to prevent cervical cancer.

• Pain control for advanced cancer.
• Treatment of selected paediatric cancers.
• Diagnosing and treating early-stage breast and colon cancers.

CVS Provided Counseling To 67,000 in Four Months After Halting Sales of Tobacco

A year after it announced its decision to stop tobacco sales, CVS Health released data that shows how its stores are working to deliver the anti-smoking message.

According to CVS, from the launch of the program on Sept. 3, 2014, through December 2014, its pharmacists counseled more than 67,000 patients filling a first prescription for a smoking cessation drug or prescription nicotine replacement therapy.

CVS said pharmacists have also consulted with thousands more smokers seeking advice about over-the-counter NRT products.

Prescriptions for smoking cessation medications have increased by 63 percent on a monthly basis in the timeframe and visits to MinuteClinic for smoking cessation counseling were up 61 percent compared to the prior eight months, the drug store chain said.

"One year ago, we knew that removing cigarettes and tobacco products from our stores would not be enough on its own to make a meaningful difference in the lives of our customers and patients who smoke," said Troyen Brennan, chief medical officer at CVS Health, said in a statement. "We believe our combined efforts of eliminating pharmacy-associated access to tobacco products, and a devoted smoking cessation program through our channels will help our patients on their path to better health."

The CVS Health smoking cessation program combines the elements that are the most effective smoking cessation components: an assessment of the smoker's readiness to quit, medication support, coaching and education.

CVS/pharmacy also offered ready access to over-the-counter NRT products that assist smokers trying to quit.

Purchases of these products increased 21 percent in September through December over the previous four months. Additionally, customers picked up 2.3 million tobacco cessation brochures at CVS/pharmacy and thousands of "Last Pack" encouragement toolkits, reaching millions of additional smokers with education, information and support.

Smokers also sought out quitting support through a Smoking Cessation Hub on www.cvs.com which neared one million visits through December. Approximately 25,000 smokers completed Nicotine Dependency Quizzes during these website visits and more than 2,500 of them followed up with calls to a smoking quit line (1-844-265-4321) operated by the American Cancer Society for additional support and access to services in local communities.

CVS Health operates 7,800 retail pharmacies, more than 900 walk-in medical clinics, a leading pharmacy benefits manager with nearly 65 million plan members, and specialty pharmacy services.

Funding Opportunity

Crowd Research Initiative Taking Proposals in Myeloma

The Myeloma Crowd Research Initiative is accepting research proposals for high-risk multiple myeloma until the end of February, through the Myeloma Crowd website. The Myeloma Crowd is a division of the CrowdCare Foundation.

The initiative combines myeloma specialists with educated "epatients" to select and fund promising research projects in myeloma. This is the first time a united group of patient activists have helped steer the direction of myeloma research.

The MCRI has selected high-risk myeloma including genetic features (del 17p13, 4;14, 14;16 and 14;20) and aggressive features in relapsed/refractory myeloma patients as its main area for research funding.

Proposals from selected applicants will be reviewed by both a Scientific Advisory Board and Patient Advisory Board who will select final projects for crowdfunding campaigns. Due to the nature and incidence of high-risk myeloma, collaborative proposals between facilities and investigators are welcome.

In Brief

Candace Johnson Named CEO Of Roswell Park Cancer Institute

(Continued from page 1)

Department of Pharmacology and Therapeutics, the Wallace Family Chair for Translational Research and professor of oncology. During that time, she has helped secure the NCI's coveted Cancer Center Support Grant twice.

Since November 2014, she has also served as cancer center director, and served as interim president and CEO of the institute since last October.

Before coming to Roswell Park, she served as deputy director of basic research at the University of Pittsburgh Cancer Institute, and professor of pharmacology and medicine at the University of Pittsburgh School of Medicine.

THOMAS RUTHERFORD was named network physician director of cancer services for the **Western Connecticut Health Network.**

Rutherford will help create a new model of cancer delivery, building on the existing programs at the Praxair Cancer Center at Danbury Hospital, the Diebold Family Cancer Center at New Milford Hospital and the Whittingham Cancer Center at Norwalk Hospital.

Prior to WCHN, he practiced at Yale Gynecologic Oncology. He served as professor of gynecologic oncology and director of Gynecologic Oncology Fellowship at Yale University School of Medicine, a position he held for 12 years.

Rutherford has focused on prevention, early detection, and treatment of ovarian cancer and other gynecologic malignancies.

CAROLYN COMPTON will lead the scientific advisory board of **Indivumed GmbH.**

Compton is the former director of the NCI Office of Biorepositories and Biospecimen Research. She also served as chief medical officer of the National Biomarkers Development Alliance, life sciences professor at Arizona State University, and professor of Laboratory Medicine and Pathology at the Mayo Clinic School of Medicine.

According to a statement, Indivumed is planning to expand its current cancer biorepository and clinical data warehouse comprising 20,000 patients to more than 150,000 patients within five years.

ELI LILLY AND COMPANY announced a commitment of $1 million to **AMPATH** in Eldoret, Kenya, provided by the Lilly Foundation. The funding, in conjunction with World Cancer Day, will help equip a new oncology center, hire additional staff and train local healthcare professionals.

AMPATH, Academic Model Providing Access to Healthcare, was created in response to the HIV crisis in Western Kenya in 2001. It is built on a partnership with Moi Teaching and Referral Hospital and the Moi University School of Medicine in Eldoret, and a consortium of North American academic health centers, led by Indiana University.

AMPATH has continually expanded its successful HIV approach to include more diseases, including diabetes, hypertension and cancer. It now provides healthcare services to a population of 3.5 million in Western Kenya.

The AMPATH Oncology Institute was launched in 2009, with a single physician and nurse. Public-private partnerships have allowed the institute to expand its staff and services to a current team that includes 10 clinicians, six core nurses and an oncology pharmacist.

The institute received nearly 10,000 patient visits

last year and screened more than 10,000 women for breast cancer and 20,000 women for cervical cancer. The institute is one of only two cancer centers in Kenya, a country of 42 million people.

The $1 million commitment will be paid out over four years. Additionally, the foundation's funding will support the creation of a research and training institute focused on cancer prevention, screening, treatment and supportive care.

Drugs and Targets

FDA Grants Accelerated Approval To Ibrance in Breast Cancer

FDA granted accelerated approval to Ibrance (palbociclib) to treat metastatic breast cancer.

Ibrance inhibits cyclin-dependent kinases 4 and 6, which are involved in promoting the growth of cancer cells. Ibrance is intended for postmenopausal women with estrogen receptor-positive, human epidermal growth factor receptor 2-negative metastatic breast cancer who have not yet received an endocrine-based therapy. It is to be used in combination with letrozole.

The FDA granted Ibrance a breakthrough therapy designation because the sponsor, Pfizer Inc., demonstrated through preliminary clinical evidence that the drug may offer a substantial improvement over available therapies. It also received a priority review. Ibrance is being approved more than two months ahead of the prescription drug user fee goal date of April 13.

The drug's efficacy was demonstrated in 165 postmenopausal women with ER-positive, HER2-negative advanced breast cancer who had not received previous treatment for advanced disease. Clinical study participants were randomly assigned to receive Ibrance in combination with letrozole or letrozole alone.

Participants treated with Ibrance plus letrozole lived about 20.2 months without their disease progressing (progression-free survival), compared to about 10.2 months seen in participants receiving only letrozole. Information on overall survival is not available at this time.

FDA approved the Koning Breast CT system and KBCT-guided biopsy bracket. KBCT is intended to provide three-dimensional images for diagnostic imaging of the breast.

KBCT, developed by the Koning Corporation, is the first commercially available 3D breast CT scanner designed to image the entire breast with a single scan without compression of the breast tissue. The system acquires hundreds of images in 10 seconds. The biopsy bracket enables KBCT-guided breast biopsies of suspicious lesions, and a collimator which is used to limit the x-ray beam to the area of interest. The biopsy bracket provides 3D targeting at comparable or lower radiation exposure compared to stereotactic guided biopsy.

Over 680 patient scans on KBCT were conducted at Elizabeth Wende Breast Care and the University of Rochester Medical Center, with additional collaboration at the University of Massachusetts Medical Center which culminated in a large reader study conducted at the Medical College ofSouth Carolina.

FDA granted priority review to Yondelis (trabectedin) for the treatment of patients with advanced soft tissue sarcoma, including liposarcoma and leiomyosarcoma subtypes, who have received prior chemotherapy including an anthracycline.

Yondelis obtained orphan drug designation for STS by the European Commission in 2001 and by the FDA in 2004. A priority review designation means FDA's goal is to take action, following the validation and acceptance of the NDA, within six months as compared to 10 months under standard review.

Yondelis is sponsored by Janssen Research & Development and PharmaMar.

Yondelis is available in 77 countries for the treatment of advanced soft-tissue sarcoma as single-agent, and in 70 countries for relapsed platinum-sensitive ovarian cancer in combination with pegylated liposomal doxorubicin.

FDA granted a second breakthrough designation to the immunotherapy MPDL3280A (anti-PDL1).

The designation was for patients with PD-L1 (Programmed Death-Ligand 1) positive non-small cell lung cancer whose disease has progressed during or after platinum-based chemotherapy and an appropriate targeted therapy for those with an EGFR mutation-positive or ALK-positive tumor.

The designation is based on early results of MPDL3280A in people whose NSCLC was characterized as PD-L1 positive by an investigational test being developed by Roche. All studies of MPDL3280A are prospectively evaluating PD-L1 expression.

MPDL3280A is sponsored by Genentech, a member of the Roche Group. MPDL3280A previously received a breakthrough designation for bladder cancer in 2014.