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# SPECIALTY PHARMACY NEWS

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## NIH Program Will Develop Drugs for Rare Diseases; Health Reform Could Be Issue

A new federal program aims to boost the development of drugs for rare and neglected diseases by shouldering the financial risk for the preclinical drug-development phase and gathering data from that process. But one expert urges that any health reforms take into account these patient populations so that companies are not discouraged from making investments in these therapies.

The federal budget for fiscal year 2009 (which runs Oct. 1, 2008, to Sept. 30, 2009) provided \$24 million to establish the Therapeutics for Rare and Neglected Diseases (TRND) program. TRND will take drugs through the preclinical stage of development, before drugs are tested in humans. According to NIH, the goal of the initiative is "to move candidate drugs forward in the drug development pipeline until they meet Food and Drug Administration (FDA) requirements for an Investigational New Drug (IND) application." Once TRND has developed the drug to this point, "the drug would then be handed off to an experienced organization outside of NIH, such as a pharmaceutical company, for human testing and other aspects of clinical development." In some cases, NIH says, TRND could initiate or remain involved in human studies.

NIH says that pilot projects will be conducted by the end of the current fiscal year. It has not said what the diseases are that it will investigate.

Rare diseases are defined as ones that impact fewer than 200,000 people, such as cystic fibrosis and Huntington disease. Various statistics show that anywhere from 20 million to 30 million Americans suffer from rare diseases. NIH estimates that there are about 6,800 rare diseases — but treatments are available for only about 200 of the conditions.

The Orphan Drug Act, passed in 1983, offers incentives such as tax credits and market exclusivity to companies to develop therapies for rare conditions. However, says Craig Kephart, president and CEO of Centric Health Resources, Inc., a health services company focused on rare, orphan and chronic disorders, "Look at the market size for many of the products we're talking about." Because of advances in genom-

ics, patient populations "have been divvied up into small chunks" — perhaps 500 or 1,000 patients. "For diseases like cystic fibrosis, we can target therapies for one of the gene defects," he explains. While this may be good for finding therapies that will work better for patients, when a company is faced with developing a compound for only 500 patients, "is there a viable economic model for a company to come in and commercialize a drug?" asks Kephart. "We're seeing these populations get smaller and smaller. Exclusivity is great, but how many companies are going to produce a drug for 100 people?"

Kephart says that TRND is significant from two perspectives. From the patient-community perspective, the initiative "is wonderful." Anything that can be done to incentivize companies to look for potential cures and treatments for rare, underserved patient populations is good, he says.

From an industry perspective, says Kephart, the initiative "ultimately is about reducing some of the risk manufacturers have to face when bringing new products to market." According to NIH, the preclinical phase — colloquially called the "Valley of Death" — can take two to four years and cost an average of \$10 million. The agency notes that 80% to 90% of projects fail during the preclinical process. Kephart points out that "a lot of great things have been discovered at NIH, but they're just that — discoveries." NIH's work to bring potential therapies further along in the development process will give companies "more information, data and proof that it is going to be a successful therapeutic intervention," he says. Companies will be more interested in investing in a compound that has these data than one that does not.

Only 30% of the drugs that companies invest in ever make it to market, notes Kephart. Firms "need a financial model that recoups the costs of not only the successful products but also the failed products," he maintains. "If a company can cut its costs from \$30 million to \$10 million or from \$80 million to \$20 million because the drug has already been investigated in a lab," it's much easier to make that investment.

"The federal government may be the only institution that can take the financial risks needed to jump-start the development of treatments for these disease, and NIH clearly has the scientific capability to do the work," said NIH Acting Director Raynard S. Kington, M.D., Ph.D., when the institute unveiled the program.

### Will Price Controls Undercut Orphan Drugs?

With all the focus on health care reform, Kephart says he wonders if there will be "viable markets for these kinds of drugs."

The prospect of developments such as everyone having health insurance and having it be portable is certainly a good thing, says Kephart. "But the challenge is are you going to have price controls?" he asks. "Is comparative effectiveness going to hinder the progress of your drug? Will the government make cost/value judgments?" For instance, he says, "the dynamic in the marketplace" means that decisions will need to be made on whether to spend \$100,000 per year for one person who has a rare disorder or to treat 100 patients at \$1,000 per year.

Kephart wonders whether there are "mechanisms by which rare and neglected disorders can be considered different, both from the comparative-effectiveness perspective and a reimbursement, market perspective."

A public-plan option is "good, but the government has the tendency to set prices," he says. "The fear is these communities will continue to be underserved."

Besides offering therapies for conditions that do not have ones, these drugs for rare disorders are important for another reason. "Much of what we have learned about basic diseases has come from research on rare diseases," says Kephart. He explains that there are "initiatives to bring together seemingly unrelated research." For example, a protein used with genetic emphysema patients has potential in diabetes. It is important to "recognize that the money and time spent studying rare diseases has a multiplier effect," he contends.

There is "significant concern," he says, that clinical trials could "bring drugs right to the brink, but no one is going to pick them up if they can't make money on them." He adds that he doesn't "know if members of Congress and the Obama administration would be open to some kind of language for treating these diseases differently." Work on health reform is "changing daily. It's moving so fast that one has to wonder what kind of unintended consequences can result from legislation" developed so quickly, he says.

Contact Kephart at (866) 849-4481. For more information on TRND, visit <http://rarediseases.info.nih.gov/TRND>. ✧

## CMS Denies Coverage for Cancer Test, Spurs Fears of Cost Criteria

CMS said last month that because inadequate evidence exists for CT colonography as an appropriate screening test for colorectal cancer, Medicare would not cover the procedure. One expert, though, contends that the decision ultimately was based on financial concerns, and that it demonstrates the danger of what could happen with comparative effectiveness research.

Also known as virtual colonoscopy, the noninvasive procedure is actually a three-dimensional X-ray of the colorectal area. Many commercial health plans offer coverage of the procedure.

According to the Colon Cancer Alliance, colorectal cancer is the third most common cancer in the country and the second leading cause of cancer deaths. About 150,000 Americans — most of them Medicare aged — are diagnosed with it annually. But if it is caught early, the disease has cure rates of more than 90%.

Virtual colonoscopies have "proven to be a very effective method of early detection and prevention of colon cancer. This decision now leaves millions of older Americans exposed to a higher risk of colon cancer. It also exacerbates an unequal standard of care between Medicare beneficiaries, who do not have the choice to undergo a virtual colonoscopy, and those with private insurance who do," said the alliance in a statement.

Colonoscopies can cost up to \$3,000, while colonographies can cost \$300 to \$800. The CMS decision cites guidelines from the American Society for Gastrointestinal Endoscopy that say, "Cost-effectiveness analyses indicate that under most assumptions colonoscopy is more cost-effective than" is virtual colonoscopy.

Craig Kephart, president and CEO of Centric Health Resources, Inc., a health services company focused on rare, orphan and chronic disorders, agrees that "there is some logic" to the decision. If someone has a virtual colonoscopy and the physician finds something, the patient will need to have a colonoscopy anyway, resulting in increased costs. "But this is not always how it works," he says. "At the end of the day," the decision "is an economic one," he says.

"With any procedure, there is some kind of risk," Kephart explains. He adds that older people do not tolerate colonoscopies as well as younger people

might and that a scan would be a better option for them. The X-ray, however, exposes people to radiation. In addition, proponents of the decision point out that sometimes virtual colonoscopies will detect aberrations that are not dangerous to the patient but will result in more tests, which also means more risk to the patient.

Kephart calls the decision a possible “harbinger” of things to come with comparative effectiveness. Data on better treatments will be useful to patients and physicians, but preferring some therapies over others based on cost would not be good, he asserts.

Contact Kephart at (866) 849-4481. View the decision at [www.cms.hhs.gov/mcd/overview.asp](http://www.cms.hhs.gov/mcd/overview.asp). ♦

## As Specialty Drug Costs Continue to Rise, Patients Are Still Filling Scripts — for Now

Specialty drug prices continued to rise last year, according to a recent report. And even through the economic downturn, patients for the most part have continued to fill prescriptions for these expensive medications, many of which treat chronic and life-threatening conditions. But if prices continue to rise and the financial pressure on health plans continues to increase, these patients may find it hard to continue filling prescriptions, say several sources.

According to the *AARP Watchdog Report: Trends in Manufacturer Prices of Prescription Drugs Used by Medicare Beneficiaries 2008 Year-End Update*, the average annual increase for the 144 most commonly used specialty therapies was 9.3% in 2008 — almost two-and-a-half times the general inflation rate of 3.8% (*SPN 5/09, p. 8*). Prices of brand nonspecialty drugs increased 8.7% during that time, while generic nonspecialty drugs decreased 10.6%.

With specialty drugs “popping up as the top drugs in terms of cost,” plan sponsors “are starting to ask some targeted questions” about them, says Sean Brandle, national Rx practice leader at The Segal Company. “Only a small amount of the population is on them, but plan sponsors are spending a lot on them.”

When people can’t afford these drugs, that “exacerbates an already-bad situation,” says David Knowlton, president and CEO of the New Jersey Health Care Quality Institute and a board member of the nonprofit HealthWell Foundation.

More people are using specialty drugs, he contends, in large part due to the aging of the baby boomers and the fact that more of these drugs are hitting the marketplace. But “health plans can’t pay more in claims than they take in on premiums.” Because health plans are “in the midst of contract periods, they can’t change” what they charge their members midstream. However, newer contracts will likely reflect the financial pressure that plans are under, he says.

### Companies May Be Worried About Reform

The uptick may be “in anticipation of reduced margins going forward,” says Craig Kephart, president and CEO of Centric Health Resources, Inc., a health services company focused on rare, orphan and chronic disorders. With a new presidential administration and health care having been a hot topic since last year, manufacturers may believe their profit “margins may be severely impacted,” so they are attempting to “maximize their margins while they can.... Comparative effectiveness is scary to a lot of people,” who wonder whether the government will use it to help patients and physicians make better treatment decisions or as a cost-effectiveness tool. He points to the recent CMS decision to deny coverage of CT colonographies — so-called virtual colonoscopies — to beneficiaries as a possible “harbinger” of things to come (see story, p. 3). Although “there is some logic” to the decision, “at the end of the day,” the decision “is an economic one,” he says. With health reform, “there is only so much money we have to accomplish the goals set down by the administration and others. Cost control has to be a major component,” he adds.

“Don’t get me wrong — I think comparative effectiveness is necessary,” says Kephart. “All the health plans and physicians I’ve talked to want that data” in order to “use it as another tool in the decision-making process.” Nevertheless, he says, “there are only so many dollars. We can’t have Cadillac health care for everybody with no way to pay for it.... You’ve got to figure at some point someone’s going to say that we need to put some price controls on this.”

And with all the attention on legislation to create an approval pathway for biosimilars, the prospect of facing competition from these drugs “has got to play into the psyche of anyone who is a [specialty drug] manufacturer,” Kephart says. “Their original business plan never conceived of” biosimilar competition. Manufacturers “are worried about their return on investment,” agrees Knowlton. Having this competition “affects their metric to determine return on investment.”

Beckie Fenrick, Pharm.D., director of clinical pharmacy at Blue Cross and Blue Shield of Florida,

tells *SPN* that although “we have historically seen growth in the utilization of specialty drugs,” the plan’s 12-month data through March 2009 shows specialty prescriptions are “flat.” But she adds that the plan has not looked into why this may be the case. “We did see an increase in costs, but it was not as steep as it has been historically,” she says. “There is a tempering for some reason.” She says that “there probably are some economic factors” behind this, but since the plan has implemented a new specialty management strategy, this may be a factor as well.

“On the managed care side, there is very definitely an understanding of the economy overall and the ability of people to pay for their medications,” says Steven Avey, vice president of managed care for PBM Partners Rx Management LLC. He points to the recently released *2009 Biotechnology Monitor & Survey: Marketplace Policies, Practices, & Perspectives*, which shows that health plan respondents expect their members to pick up about 12% of the cost of a biologic under the pharmacy benefit. Avey, who is also a member of the publication’s editorial board, contrasts this with a nonbiologic drug, perhaps for high blood pressure, for which the member generally will cover 25% to 30% of the cost. He adds that the 12% might be a bit high, as members of Partners Rx health plan clients pay about 4% of the total costs of biologics.

Some plans are adding another tier to their drug formulary for specialty drugs, but are still limiting members’ cost-share amounts somewhat. “With specialty, there is a lot of movement about putting these drugs on a fourth tier,” says Avey. In 2008, 55% of the commercial plans that participated in the survey said they had member out-of-pocket spending limits for biologics covered under the medical benefit, and 38% applied such limits to specialty drugs adjudicated under the pharmacy benefit.

Another way to manage specialty drugs, Avey says, is to have a defined benefit, where members pay a certain amount — maybe \$60 or \$70 — for a prescription. And then all members pay these “deductibles,” which result in offsets to cover the costs of the more expensive biologics. “I love this approach,” says Avey, noting that it doesn’t “penalize” members with biologic prescriptions like other benefit designs do. Last year, 47% of commercial plan respondents said they applied deductibles to biologic drug use under the medical benefit, with 44% applying it to these drugs under the pharmacy benefit.

Avey illustrates the problem with copayments through the following example: If a member is pre-

scribed a drug that costs \$1,000 per prescription, there will probably be a \$100 or \$150 copay for specialty drugs. “So I have a \$150 additional copay that I earned from that member,” he says. “But the problem is that there are such few numbers of prescriptions” for specialty drugs that “I might be gaining \$150 but on only 15 prescriptions.” His PBM wanted to find out what the impact on savings would be for a plan that places a \$50 deductible on brand-name drugs. “We determined this would save the plan in the ballpark of 18%,” he says. But if “you raise the copay to an arbitrary level of \$200, you save the plan in the ballpark of 3%.” According to Avey, “it makes more sense to spread [the costs] out. People didn’t ask to get cancer or rheumatoid arthritis; they got them through no fault of their own. We don’t need to penalize them.”

Health plans “need to help patients get the care they need, not drive them away with copayments,” agrees Knowlton.

In the survey, the majority of commercial/group plans and PBMs polled reported a 6% to 15% growth in the utilization of specialty drugs. Those same groups reported that biologic expenditures had mean percentages of 13% to 14% for the total pharmacy benefit budget. For Partners Rx, says Avey, 1% of the prescriptions are for biologics, but these drugs represent 10% of its drug spend. If the utilization goes up even 2% or 3%, that means “a dramatic increase in drug spend if [the trend] holds true across the board,” he says. “There is so much emphasis on how to pay for these, especially when you look at the pipeline,” which holds hundreds of specialty therapies. “A conversation about deductibles is good,” he contends.

### How Long Can Unemployed Afford Specialty Rx’s?

According to Kephart, at least for now, “patients are still getting their [specialty] prescriptions.” Other trends that he notes are “the number of people on COBRA is increasing, the length of time to pay off patient responsibility is getting longer, and the number of people seeking assistance from patient-assistance programs and copayment-assistance programs is starting to tick up.” Wondering how long people can afford COBRA if they are unemployed, he adds that it is “reasonable to expect an uptick in the number of uninsureds with these conditions....In our niche, we’re kind of at the beginning of what will be impacts” of the economic downturn. “Patient concern is much higher than it used to be.”

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