PHARMACEUTICALS EXPORT PROMOTION COUNCIL
(Set up by Ministry of Commerce & Industry, Govt., of India)

Date: 16th March, 2012

TREAT Act would speed up drug approval process

Goldman manifesto echoes past questions about bank Arrest made in missing Anchorage barista case Boy who vanished in 2004 might soon rejoin family Small plane crash in western NC kills all 5 aboard Homeless killer suspect said he had 'killer' gene Defendants in Ariz. wildfire reach plea agreements Ohio adoptive dad indicted on 31 counts of rape Federal prosecutor taken off cases after Web posts Police: Girl, war vet dead in murder-suicide 2 men sentenced for smuggling weapons in Mexico Many homes damaged as tornado rips through Mich. Oklahoma executes man for wife's 1996 slaying Texas constable arrested on drug, firearm charges EPA: Water quality OK in Pa. gas drilling town Alleged Mexican cartel member faces bribery charge Ariz. bill could require reason for birth control Lawyer: Afghan suspect's friend had leg blown off Plane with hydraulic problem returns to Boston Senate women lead fight to renew domestic violence law Feds to halt Texas Women's Health Program funding WASHINGTON — Hundreds of thousands of people die each year from diseases for which there are no cures. But legislation being pushed by patient advocates and biotech firms could shave years off the federal drug approval process to get promising treatments to those who need them.

The TREAT Act, introduced by U.S. Sen. Kay Hagan, D-Greensboro, accelerates the review and approval process for medicines that treat an unmet medical need or significantly advance the standard of care for people suffering from deadly diseases. A similar expedited approval bill was introduced in the House last week.

Lisa Macdonald survived breast cancer, but still worries about her cancer coming back. Macdonald, executive director of the Carolina Breast Friends, a Charlotte support organization, met with a mother last week who learned she had cancer when she was breast feeding her second son. She has since been in chemotherapy for four years and is taking part in national clinical trials hoping for a cure.

“I’m looking at this young woman who has two small sons and that’s why the TREAT Act is really important,” MacDonald said. “For us, it’s all about providing hope. We haven’t won the war on cancer. Wasn’t it Richard Nixon who originally declared the war on cancer in the ’70s? It’s now the

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21st century. A lot more breast cancer patients live and thrive like me. And there are still some who don't."

The bill could be a boon for the state's smaller biotech firms that often struggle to bring drugs to market. But the legislation, which is now in committee, is expected to meet resistance from the Food and Drug Administration, those who worry that drug safety will be compromised and some large pharmaceutical companies who could potentially lose market share.

But Dr. Amy Abernethy, director of Duke's Cancer Research Program, estimated that hundreds of thousands of people could benefit from a more up-to-date, accelerated approval process.

It takes 10 to 15 years and more than $1 billion on average to bring a drug to market. The National Organization for Rare Disorders reports that of 7,000 known rare diseases, fewer than 250 have FDA-approved therapies.

People die waiting for promising therapies, said John Vernon, an associate professor of health policy and management at UNC-Chapel Hill. A former senior economic policy advisor at the FDA, Vernon said the political consequences are much greater for the agency if a bad drug is approved and someone is hurt or dies.

"They're pulled before Congress. They get a tongue lashing," he said. "But nothing happens if they spend an extra two or three years studying a drug to make sure it's perfectly safe. Think during those two or three years, how many patients die or don't get access to treatments?"

Durham-based Chimerix is developing CMX001, a treatment for life-threatening viral infections for patients whose immune systems have been compromised by disease or drugs. President and CEO Kenneth Moch said Hagan's bill could get CMX001 to patients faster.

"We're a 50-person company. We obviously have limited resources," Moch said. "This could provide additional flexibility and speed for approval. We have a drug we believe can be used for patients where there is no other available therapy."

PRESSURE ON FDA

Karen Riley, a spokeswoman for the FDA, would not comment on the proposed legislation but said the FDA has several existing programs for expediting the review of promising drugs. She said the agency is "happy to work with Congress to see if there are ways to better utilize our tools while retaining our high standards for safety and efficacy."

The FDA is often under pressure from patient advocates and biopharmaceutical companies to loosen

up safety requirements so more drugs can be brought to the market, said Erik Gordon, a business professor at the University of Michigan who specializes in the biomedical industry. But he said the FDA's decisions must be based on science and not be swayed by emotion or political will.

"The first rule of medicine, the oldest rule of medicine, and probably the best rule of medicine is do no harm," he said.

The TREAT Act would not alter existing FDA safety standards, but experts note that accelerated approval programs don't always work in ways doctors and patients hope.

Under the current accelerated program, approval is granted on the condition that clinical trials continue after the drug's release to verify its benefits. But Abernethy of Duke said keeping up with testing can be difficult and the industry needs to come up with better data collection methods.

She points out the case of Mylotarg, which was given early approval in 2000 to treat elderly patients with a form of leukemia. The drug had to be pulled in 2010 after ongoing clinical trials showed no improvement in clinical benefits. The FDA said more patients died receiving the drug than those receiving chemotherapy alone.

"It's a double-edged sword," Abernethy said. "With accelerated approval comes the expectation that we're going to continue to investigate these drugs and follow them post-approval to understand do they really do their job and work."

THE HUB OF N.C. FIRMS

The N.C. biotechnology industry employs about 36,000 people who work in pharmaceutical research, development and manufacturing. North Carolina ranks 14th among U.S. states in bioscience patents, according to the N.C. Biotechnology Center. More than 2,300 bioscience patents were granted to N.C. companies between 2004 and 2009.

According to the Biotechnology Industry Organization, a trade group for the industry, 61 percent of venture capitalists cite FDA regulatory challenges as having the highest impact on their investment decisions.

Supporters expect Hagan's proposal would ease the challenges many small companies face trying to develop and bring new drugs to market.

"We all know that new drugs come from small biotech and not big biotech," said Dr. John Powderly, an oncologist at Carolina BioOncology Institute. Powderly said smaller biotech firms "don't get a second swing at the bat" if the FDA changes expectations during clinical trials.

Powderly's Huntersville clinic is often the last place cancer patients go before entering hospice care. He's seen about 4,000 patients since the clinic opened in 2005. It provides research drugs to patients with metastatic cancer, which has spread through their bodies. Most do not make it.

"If patients can get access to a new drug and they have the right target and the drugs hit the target, there are phenomenal results," he said.

WHAT HAGAN'S BILL IS FOR

Hagan's proposal is similar to another FDA fast-track program. Its goal is to get medication to patients earlier provided researchers can show scientific proof of a drug's benefits and safety. However, the process was created through regulations, not law, and its application has been limited mainly to treatments for HIV and cancer.

The TREAT Act would allow researchers to use more types of scientific evidence that can be measured to prove safety and effectiveness of drugs being considered for early release.

Hagan said she decided the legislation was needed after hearing from parents seeking treatment for their children. She met with the parents of one child with Spinal Muscular Atrophy, a rare genetic cause of infant death, she said.

"With the small number of patients available for large clinical trials, rare diseases like SMA have a hard time clearing the FDA hurdles for approval, and you can imagine the frustration of this family, and so many others like them," Hagan said.

The National Organization for Rare Disorders, The Friends of Cancer Research, Parkinson's Action Network and the Biotechnology Industry Organization have pledged their support for the Hagan's proposal.

But some larger pharmaceutical companies have yet to fully embrace Hagan's bill

Gordon, the University of Michigan business professor, said the bill could make smaller companies less dependent on big pharma. Currently many small companies often team with their larger counterparts who have the experience and capital to run long FDA trials. In return, large pharma get a share of the profits.

The trade group, PhRMA, said it's supportive of Hagan's efforts, but has not taken an official position on the full bill.

Sarah Alspach , a spokeswoman for GlaxoSmithKline, one of the Triangle's largest employers, said the company thinks the best way of getting medicines to patients faster is through modifications of the

current accelerated approval process.

Read more here: http://www.kansascity.com/2012/03/15/3492130/treat-act-would-speed-up-drug.html#storylink=cpy

Source: The Kansas City Star, Kansas City.com