Pharma's niche focus spurs US aid for antibiotics

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(AP) -- The pharmaceutical industry won approval to market a record number of new drugs for rare diseases last year, as a combination of scientific innovation and business opportunity spurred new treatments for diseases long-ignored by drug companies.

Drug companies are increasingly taking advantage of the commercial benefits of developing so-called orphan drugs, which include extra patent protections, higher pricing and a streamlined review process by FDA. Among the innovative treatments approved in the past year were the first new drug for lupus in 50 years and the first new drug for Hodgkin's lymphoma in 30 years.

But the focus on specialty drugs has put pressure on the U.S. government to ramp up its own spending on vaccines, antibiotics and drugs for more widespread health threats, which are less profitable for companies.

Since 2006, government spending on research for familiar diseases like staph infections, smallpox and botulism has increased more than 660 percent, from $54 million to $415 million last year

"Many of these are everyday, general diseases that we thought we had conquered decades ago, but we've seen some of them pop up again," said Dr. Robin Robinson, director of the Biomedical Advanced Research and Development Authority, which is tasked with acquiring vaccines, drugs and other necessities for public health emergencies.

Since 2005, BARDA has awarded $3.5 billion to outside companies to encourage research and production of antibiotics, flu vaccines and other products that are seen as less profitable than specialty drugs.

"We have pushed the envelope more toward diminishing the risk for companies so that they'll be more interested in getting involved with us and developing things like vaccines and antivirals," said Dr. Anthony Fauci, infectious diseases chief at the National Institutes of Health, which funds research into bird flu, tuberculosis and other potential pandemics. The government's role in developing new therapies goes beyond awarding contracts and includes offering assistance in designing trials and recruiting test subjects.

The need for such assistance stems in part from a new focus among pharmaceutical companies on drugs for rare diseases or unusual strains of common diseases.

Eleven of the 30 new drugs approved last year, or 37 percent, were for rare medical conditions, the highest percentage on record since the FDA began offering incentives to develop such therapies, known as orphan drugs, about 30 years ago. Additionally, nearly half of the 30 drugs were cleared under FDA's "fast track" program reserved for drugs that fill an unmet medical need.

"The companies are saying `this is actually a viable model.' Whereas back in the nineties they were skeptical, now they seem convinced," said Mark Schoenebaum, an analyst with International Strategy & Investment.

Analysts credit scientific advances and looming patent expirations with the spate of innovative products. Drugs worth a mammoth $255 billion in global annual sales are set to go off patent before 2016, according to EvaluatePharma Ltd., a London research firm.

The pharmaceutical industry reached its peak of profitability in the 1990s with heavily marketed drugs for common afflictions, like AstraZeneca PLC's Nexium pill for heart burn and Pfizer Inc.'s Lipitor for high cholesterol. In the last decade drugmakers managed to extend the patents on those drugs by tweaking their formulations, resulting in so-called `follow-on' drugs. But with most of those products on the cusp of losing patent protection, drugmakers have finally been forced to innovate, often turning to hard-to-treat diseases for which there are few existing therapies.

The FDA grants companies seven years of exclusive, competition-free marketing for each newly approved orphan drug, as well as tax breaks on the costs of developing the drugs. Orphan drugs also typically command much higher prices than other drugs. Last year French drugmaker Sanofi paid $20 billion to acquire specialty drugmaker Genzyme, whose products range from $100,000 to $300,000 for one year's supply.

One side effect of the focus on developing drugs for rare diseases is increased investment by the government to spur research into more common public health threats with the potential to cause mass outbreaks of illness. One such threat comes from so-called superbugs, or bacteria that have grown resistant to antibiotic drugs.

Robinson says government support is needed to spur antibiotic development because of how sparingly the products are used in medical practice. After decades of routine use, many first-generation antibiotics like penicillin are no longer effective against common bacterial strains, such as the staphylococcus aureaus, which causes staph infections. Physicians are encouraged to use newer antibiotics only in critical situations so that superbugs have less chance to build a resistance to them. As a result, drugmakers do not see a large commercial market for new antibiotics. Now the federal government is providing an incentive.

BARDA has awarded a series of contracts to encourage development of new antibiotics that can be stockpiled for use in a natural outbreak or during a bioterrorism attack.

- The agency has allocated up to $64 million to Achaogen, a San Francisco startup, for development of a new antibiotic against tularemia, a bacterium that can cause pneumonia and urinary tract infections. Public health officials are especially focused on Tularemia because it could also be used in a potential bioterrorism attack. Robinson says the contract is an example a new strategy of encouraging companies to produce therapies with dual uses: as federal preparatory measures and as commercial medical products.

Achaogen has received $155 million in research contracts and has several antibiotics in early and mid-stage, though none are currently available for sale.

- Under a $38.5 million contract awarded in September, BARDA will help GlaxoSmithKline PLC test an experimental antibiotic against both bioterrorism agents and infections like hospital-acquired pneumonia.

The U.S. government has used a similar pump priming strategy to encourage investment in flu vaccines. The Department of Health and Human Services wants to be able to provide enough vaccine for the entire U.S. population within six months of a flu pandemic. To meet that goal the government has tried to boost vaccine production by encouraging more Americans to get the standard flu vaccine each year. The government's hope is that by making the shots routine for more Americans, companies will invest in larger vaccine facilities that can ramp up production in the event of a pandemic.

Last month Swiss drugmaker Novartis AG opened the first U.S. vaccine facility equipped with cell culture technology, a faster method for

producing vaccines than the traditional technique using chicken eggs. The U.S. government provided half of the $1 billion investment for the facility, as part of its preparations for a potential flu pandemic.