MedicalXpress

Shire bid for Baxalta highlights orphan drug's appeal

August 6 2015

Faced with expiring patents and growing competition from generics, pharmaceutical companies increasingly view orphan drugs as a key pathway to growth.

In the latest sign of the appeal of medicines to treat orphan, or rare, diseases, Dublin-based Shire announced Tuesday that it proposed a $30 billion takeover of US company Baxalta, touting a combination that would create "the global leader in rare diseases."

Shire said the merged company would have multiple $1 billion-plus "high-value rare disease franchises with substantial barriers to entry."

Shire executives highlighted hemophilia and immunoglobulin therapy as two areas of "complementary" assets, and said both companies have also worked on oncology drugs.

But Baxalta promptly rejected the offer, saying Shire's bid "significantly undervalues" the company and saying it intends to pursue growth as a free-standing company.

The proposal from Baxalta comes amid a period of heavy deal-making throughout pharmaceuticals and health care. Last week alone, Israeli pharmaceutical giant Teva announced it would buy the generic drug business of Allergan for $40.5 billion while health insurer Anthem said it would buy Cigna for $54.2 billion.

Earlier transactions driven by orphan remedies included Roche's 2009 acquisition of US company Genentech and the 2014 takeover of Seragon Pharmaceuticals. Pharma giants like Pfizer and GlaxoSmithKline have also moved to fortify their rare disease portfolios.

There are some 7,000 orphan diseases affecting an estimated 25-30 million people around the world, most caused by genetic factors. Experts estimate about 450 orphan treatments under development.

The limited options to address ailments such as Crohn's disease and cystic fibrosis can mean treatments for patients fetch between $100,000 and $400,000 a year per patient.

The drug Cerezyme, used to treat Gaucher's disease, costs more than $300,000 per year in 2014. Cerezyme is manufactured by Genzyme, which was bought by Sanofi in 2011 for $20 billion.

Treatments for rare diseases accounted for about $20 billion in annual sales in 2000. By 2020, those payments are expected to reach $176 billion, according to an October 2014 report by EvaluatePharma.

"The industry has rushed to develop orphan drugs in recent years because they cost their developers less to put through clinical trials, and command higher prices when they do launch," EvaluatePharma said.

The push towards orphan drugs has been propelled by public policies, such as tax credits for research and development into rare diseases, public subsidies, a lengthening of patent durations and streamlined testing procedures.

Health regulators require pharma companies to test drugs aimed at mass markets on thousands of patients. Orphan drugs, by contrast, are typically tested on dozens.

In 2014, 17 of 39 medicines approved by the US Food and Drug Administration were for rare diseases.

Research into the sequence of the human genome has advanced knowledge of rare diseases, as has the use of more sophisticated methodologies, such as testing on robots.

Patients groups have also made it easier to conduct such research, by raising funds and providing lists of patients willing to participate in tests.