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Market Round-up

V6 – 15h June, 2018

General Market News:

- ***Pfizer*** and ***Flynn Pharma Limited*** overturn £90M fine for unfair and excessive drug pricing on an anti-epilepsy drug.
  https://www.pharmacy.biz/pfizer-and-flynn-pharma-overturn-90m-fine-for-unfair-and-excessive-drug-pricing/

- ***Pfizer's*** **BESPONSA** (inotuzumab ozogamicin) approved for use by the Scottish Medicine's Consortium (SMC)
  https://www.pharmiweb.com/pressreleases/pressrel.asp?ROW_ID=280601

- ***Novartis*** presents data to reinforce **Cosentyx** leadership in spondyloarthritis at Annual European Congress of Rheumatology
  http://www.pharmabiz.com/NewsDetails.aspx?aid=109447&sid=2

- ***GSK*** wins another reprieve for blockbuster **Advair** franchise as ***Mylan*** is hit with a CRL
  https://endpts.com/gsk-wins-yet-another-reprieve-for-blockbuster-advair-franchise-as-mylan-is-hit-again-with-a-crl/

- ***Takeda*** shareholder group to block $62bn ***Shire*** deal
  http://www.gulf-times.com/story/595970/Takeda-shareholder-group-to-block-62bn-Shire-deal

- Court refuses ***MSD*** injunction against ***Clonmel Healthcare***
  https://www.irishtimes.com/business/health-pharma/court-refuses-merck-injunction-against-clonmel-healthcare-1.3528200

- ***GSK*** to pass $10B in 2024 vaccine sales, with ***Merck***, ***Sanofi*** and ***Pfizer*** rounding out the top 4: report
  https://www.fiercepharma.com/vaccines/sanofi-beats-pfizer-and-a-dark-horse-biotech-2024-vaccine-sales-rankings

- ***Regenxbio a***nnounced $100M accelerated License payment from ***Novartis***
  https://www.streetinsider.com/Corporate+News/Regenxbio+%28RGNX%29+Announced+%24100M+Accelerated+License+Payment+from+Novartis/14294702.html

Drug Approvals:

- ***Amgen's*** **Prolia** gets label approval in Europe
  https://www.zacks.com/stock/news/307311/amgens-amgn-prolia-gets-label-expansion-approval-in-europe

- ***Merck's*** **Keytruda** Gets FDA Approval for Cervical Cancer
  https://www.nasdaq.com/article/mercks-mrk-keytruda-gets-fda-approval-for-cervical-cancer-cm977702

- ***GSK*** gets good news on two-drug AIDS treatment
  https://www.businesslive.co.za/bd/companies/healthcare/2018-06-14-glaxosmithkline-gets-good-news-on-two-drug-aids-treatment/

- ***Novartis*** drugs **Rydapt**® (midostaurin) and **Votubia**® (everolimus) recommended for NHS funding
  https://www.pharmiweb.com/pressreleases/pressrel.asp?ROW_ID=280791

- ***Amgen*** Gets FDA Nod to Add **ASPIRE** Data on Kyprolis Label
  https://www.nasdaq.com/article/amgen-gets-fda-nod-to-add-aspire-data-on-kyprolis-label-cm976797

Further Reading:

- How To Inspire Employees While Changing The World For The Better - ***Astellas***
  http://hrnews.co.uk/how-to-inspire-employees-while-changing-the-world-for-the-better/

Long Read:

Pharma giants are betting billions on gene therapies that could fundamentally change how we treat diseases:

- Pharma is getting serious about gene therapy, one-time treatments designed to modify genes to cure serious diseases.
- Novartis is the first big pharma company to make a major bet on gene therapy, acquiring Avexis for $8.7 billion in April.
- Investment in the gene-therapy space could change the way certain diseases are treated, though the treatments come with high price tags.
- One-time drugs designed to cure previously untreatable diseases by modifying human genes are starting to gain traction in the US.

These cutting-edge treatments, known as gene therapies, work by inserting new genetic code into the body to start producing a protein that is missing or deficient.

Recent mergers-and-acquisitions transactions show that big pharma is betting big on gene therapy. In April, Novartis gave an $8.7 billion endorsement of gene therapy with its acquisition of Avexis, a company working on a treatment for spinal muscular atrophy, a rare genetic condition that affects muscle movement and is the leading genetic cause of infant mortality.

Pfizer has also invested the space, acquiring a gene-therapy company called Bamboo in 2016 in a deal worth as much as $645 million.

The deals illustrate "that pharma's starting to come off the sidelines and starting to believe that there's some potential for those programs," Adam Keeney, the global head of external innovation and R&D strategy at Sanofi Genzyme, told Business Insider.

For its part, Sanofi has a collaboration with Voyager Therapeutics, which is working on gene therapies for neurodegenerative diseases. Bayer has a partnership with Ultragenyx around its hemophilia gene therapy.

"We definitely feel this is important and ready for testing primetime," Chris Haskell, a Bayer vice president who leads its West Coast Innovation Center, said in an interview. "That is, we still need to see how it works in humans. You never know until you get it into patients."

Where to start

Gene therapies have been developed to treat rare conditions that affect a small number of people — and they come with high price tags.

So far, the Food and Drug Administration has approved one gene therapy that acts on a hereditary illness. The approval, in December, was for Luxturna, a treatment for a form of blindness called Leber congenital amaurosis, which affects two or three newborns out of 100,000.

The condition is caused by a gene defect that stops the retina from making a key protein. The one-time treatment is injected into the retina, and from there, a virus carrying the corrected gene can get to work replacing the faulty one and start producing the protein.

The therapy, made by Spark Therapeutics, has a price tag of $850,000, making it the most expensive drug ever approved in the US, though some analysts had expected Spark might charge $1 million.

These sky-high prices largely fall to the government and health plans.

The price tags can also be the downfall of these therapies. For example, the world's first gene therapy, a treatment for a rare genetic disorder that causes problems with the pancreas, cost $1 million and was used only once in Germany; the physician had to prepare a submission form as thick as "a thesis" and call the CEO of a German insurer to cover the cost, the MIT Technology Review reported in 2016. Last year, Uniqure, the company behind the drug, withdrew it from the European market.

As these drugs' manufacturing improves and more treatments get closer to approval, a few areas have emerged as the best places to start when exploring just how far-reaching gene therapy can get, Keeney saidThe eye is one of them. As an organ, the eye is easy to get to, and it doesn't react as strongly to foreign objects — in the case of Luxturna, a virus carrying the gene therapy.

Next up is blood disorders like hemophilia, a group of conditions in which the body has a hard time controlling blood clotting. Shire, BioMarin Pharmaceutical, and Pfizer (in collaboration with Spark) are all working on treatments for certain kinds of hemophilia.

Gene therapy could be used in this instance to reintroduce or bolster the production of proteins known as clotting factors, which help your blood clot if you get a cut or start bleeding internally.

Elsewhere, researchers are exploring the liver and the brain as targets for gene therapies. And gene therapies are in the works to treat Parkinson's disease, a neurodegenerative condition characterized by motor symptoms such as shaking in the hands and legs and stiffness and impaired balance.

What still needs to be sorted out

For now, gene therapies seem to fit best into conditions that affect smaller groups of people and can serve as one-time treatments that, ideally, reverse it.

Beyond that, it remains to be seen how these expensive therapies might play out. For example, developing a gene therapy for diabetes — a condition that affects millions of people — may not make the most sense, because there are other ways to treat it that are less expensive and less risky.

"I think the utility outside very specific rare diseases is a question mark, and then the go-to-market and the business model of how are you going to set up an incentive system and a structure to allow those molecules to deliver commercially is still a bit of an open item," Keeney said.

For now, the treatments are expensive, so whether health plans and governments could be persuaded to pay for them remains to be seen. In the first three months of the year, Luxturna made $2.4 million in net sales, with three people treated so far since its approval.

Analysts at RBC said in May that they expected gene therapy to be used in about 32 people this year, representing $21 million in sales. That's not a lot compared with a blockbuster drug that brings in billions of dollars and treats tens of thousands or even millions of people. But the promise

and the implications for the way we treat certain diseases could be bigger as more and more gene therapies make it to the market.

"Our objectives and the reason we're excited about gene therapies is because we're addressing the fundamental cause of the genetic disease," Bob Smith, the head of Pfizer's global gene-therapy business, told Business Insider.

That could mean correcting a disease at its root cause rather than treating the symptoms indefinitely.

"We have a high degree of confidence that these kinds of treatments could be truly transformational," Smith said.

FDA Commissioner Scott Gottlieb said at the BIO conference in Boston this month that he expects the FDA to approve 40 gene therapies by 2022.

And in a speech in May, Gottlieb said: "It's clear that these new technologies are going to transform medicine and human health. Gene therapy was largely a theoretical promise a few decades ago. Now we should not only expect these products to cure disease, but we also ought to demand that we reach this objective."

This article appeared in Business Insider on 11th June, 2018.

Link: http://www.businessinsider.com/companies-like-novartis-pfizer-sanofi-are-getting-serious-about-gene-therapy-2018-6?IR=T