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Six Strategies for Accelerating Approval for Cell and Gene Therapies

(Source: A whitepaper presented by Cardinal Health)

After decades of slow and steady progress, cell and gene therapies are experiencing important breakthroughs, demonstrating potential to slow disease progression, improve outcomes and, in some cases, potentially cure an array of illnesses. However, developers of these cutting-edge therapies are encountering new challenges as they navigate complex clinical and regulatory hurdles, particularly when they are seeking accelerated approval.

There is no "playbook" for cell and gene therapy development, due to the unique, patient-specific nature of each dose and the potential limited patient populations for each therapy. The science behind cell and gene therapies is new to FDA reviewers who must review the evidence to prove efficacy for each of these new therapies. With traditional therapies that demand less complex manufacturing processes, upwards of 80% of the FDA's review process focuses on questions related to clinical efficacy and safety. Just 20% of the FDA's review process typically focuses on issues related to product manufacturing and delivery. For these products, development of the proposed formulation evolves in parallel with clinical development. This approach is flipped when it comes to cell and gene therapies, where manufacturing is more complex, and for autologous cell therapies, where "the product is the process," because the degree of control a sponsor has over the manufacturing process directly correlates to how a sponsor controls the quality of each patient's individual dose. For these new therapies, quality aspects related to the manufacturing process drive the development program.

The FDA review focuses primarily on the therapy's critical quality attributes and controlling these earlier in development, typically prior clinical studies. Data on potency, product manufacture, method of delivery and how it is administered to the patient are required earlier in development. Another key difference is that many cell and gene therapies are sponsored by small to medium sized companies who lack large teams of on-staff experts with deep experience in understanding the complexities of working with regulatory health authorities. In the case of Cardinal Health™ Regulatory Sciences, it commands four decades of experience supporting pharmaceutical and biotech companies to get their products to market quickly and exercise six proven strategies that can help cell and gene therapy sponsors reduce their risk of failure and expedite speed to market, including:

- Start with a clear, highly collaborative framework for leadership and communication among disparate internal teams
- Develop clear and consistent messaging early on and work cross-functionally to control, adapt and maintain message consistency throughout the product lifecycle
- Demonstrate a clear understanding of your product.

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In Brief...

- **AmerisourceBergen Corporation** (ABC) reported for its 2nd quarter 2020 ended March 31, 2020, revenue increased 9.5 percent to US$47.4 billion. GAAP diluted earnings per share (EPS) was US$4.64 for the March quarter of fiscal 2020, compared to $0.13 in the prior year quarter. "While we are pleased to report another quarter of strong revenue and adjusted diluted EPS growth in the second quarter of fiscal 2020, we are even prouder of the impressive work being done across our company to ensure continued patient access to pharmaceuticals in the midst of the COVID-19 pandemic," said *Steven H. Collis*, ABC's Chairman, President and CEO.
- **Walgreens**, in partnership with **LabCorp**, plans to add drive-thru testing locations for COVID-19, with a focus on underserved communities. The testing locations will be determined by the Trump administration with an expectation of having over 50,000 people tested per week. **CVS Health** has also joined the effort to increase testing with drive-thru testing sites in partnership with the Department of Health and Human Services and the National Medical Association. Both companies are also developing solutions to bring testing sites to businesses as the country starts to re-open. Separately, Walgreens' Red Nose Day campaign to end child poverty, will take place digitally on May 21st due to social distancing. (Visit www.rednoseday.org more information.)
- **Profarma Group** (Brazil) announced Q1 results for 2020, with a 27.4% increase in gross revenue and earnings of R$46.3 million (US$7.37 million), up 41.8%. Earnings for the fiscal year ended March 31, 2020 were R$203.9 million (US$32.45 million). For its distribution unit, gross revenue reached an all-time high of R$1.5 billion (US$23.87 million), up 31.6%.
- The **U.S. Department of Justice** (DOJ) cleared U.S. pharmaceutical distributors from legal hurdles to provide, *hydroxychloroquine* to coronavirus patients.

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Johnson & Johnson

IFPW Welcomes Johnson & Johnson Health Care Systems As Its Newest Manufacturer Member

Johnson & Johnson Health Care Systems Inc. (JJHCS) is the latest addition to the list of global pharmaceutical manufacturers who are part of IFPW's membership. JJHCS provides account management, contracting, supply chain and business services to key health care customers, including hospital systems and group purchasing organizations, leading health plans, pharmacy benefit managers, employers, and government health care institutions. IFPW is proud to welcome JJHCS as a member.

Six Strategies (cont.)...

- Develop — and continually adapt — realistic timelines and tools to keep development on track and aligned
- Make the most out of each FDA interaction. Listen to and fully address their feedback.
- Remember that accelerating development does not equal accelerated approval

Cell and gene therapy development programs are highly complex — and managing them effectively requires strategic alignment among all internal and external stakeholders, combined with well-planned and thorough timelines and communication that guide progress through all stages of development. Sponsors must be highly adaptable in their dealings with the FDA, as all parties work together to take a case-by-case approach to understanding and demonstrating the risks and potential benefits of each of these innovative therapies. Creating and executing clear plans and timelines require up-front time investments in the short-term but reduce risk and expedite approval in the long-term.

For more information on Cardinal Health's strategies for success, please visit *https://www.cardinalhealth.com/content/dam/corp/web/documents/whitepaper/cardinal-health-reg-sci-cell-and-gene-therapies.pdf* or email *specialtysolutions@cardinalhealth*.

Gilead's Major Challenge is a Sustainable Model for Remdesivir

(An article by Joseph Haas for Scrip)

Gilead, armed with its latest efficacy data, wants to balance economic sustainability with broad availability for its COVID-19 treatment *remdesivir.*

One day after two trials indicated that the drug offers therapeutic benefit in patients hospitalized for COVID-19 infections, Gilead Sciences Inc.'s first quarter earnings call was dominated by discussion of the antiviral. Emergency use authorization (EUA) by the U.S. Food and Drug Administration is expected soon following early data from the U.S. National Institute for Allergy and Infectious Diseases (NIAID) study and a Gilead-sponsored Phase III study both held promise for the drug.

Remdesivir was initially developed for the treatment of Ebola and is touted as the lead possibility for a COVID-19 therapeutic treatment. Gilead CEO Daniel O'Day said that the company is in ongoing discussions with the FDA with the goal of getting it to market as quickly as possible through an EUA.

"We'll allocate [the drug] accordingly as regulatory approvals come online. So yes, it is possible to charge," O'Day said. "I would just say that our goal here is to get a full approval for *remdesivir*."

O'Day added that while Gilead has donated the current 1.5 million doses for the drug for compassionate use and clinical trials, it could begin charging for *remdesivir* under an EUA. He did not say whether Gilead would do so. He did not categorically state that *remdesivir* could be a lucrative business, as has been the case with HIV or hepatitis C for Gilead.

However, he did point out that NIAID director Anthony Fauci's positive comments on the data thus far, and said those comments meant the landscape for treatment of the virus has changed in that other treatments will need to be compared to *remdesivir* or provide an add-on benefit, much like the therapeutic models developed for HIV.

"*Remdesivir* becomes kind of the base therapy, and one looks to try [for] symptomology improvement, mortality improvements, expanding patient populations. And so that is yet another factor that we'll go into as we determine how to best to create a sustainable solution for *remdesivir.* But clearly all those things we have been thinking about," he said.

Chief financial officer Andrew Dickinson said any negative impact during the first quarter was modest, while the company also saw stockpiling during the quarter that brought in roughly an additional US$200 million, particularly in its HIV franchise. In April, however, Gilead started to see more of an impact on its business as the pandemic has affected behavior through the health care sector.

IFPW's 2020 General Membership Meeting Postponed Until 2021

MORE INFORMATION WILL BE AVAILABLE IN THE COMING MONTHS.

IFPW looks forward to welcoming you to Tokyo OCTOBER 6-8, 2021!

If you have any questions, please contact Christina Tucker at c.tucker@ifpw.com

In Brief (cont.)...

AmerisourceBergen, **McKesson**, and **Cardinal Health** were given the approval to distribute the drug. AmerisourceBergen is now set to work with federal government agencies and health care providers to distribute *hydroxychloroquine* to patients with coronavirus symptoms. *Hydroxychloroquine* has been a controversial option, but its anti-malarial properties has been a treatment option for COVID-19. There are concerns exacerbated fatal heart problems and has yet to undergo any U.S. clinical trials.

- **Pfizer** and **BioNTech** are collaborating in the development of a messenger RNA-based vaccine to combat COVID-19. The vaccine is being tested on humans and the results from their testing are expected by late May or June. With one of the fastest timelines outlined against other pharmaceutical manufacturers, Pfizer is hopeful and expects the vaccine to be in circulation by late fall of 2020, dependent on an emergency use authorization (EUA) from the **U.S. Food and Drug Administration**.
- The Japanese government is tightening regulations on foreign acquisitions of pharmaceuticals, medical devices, and vaccines and blood products necessary to fight infectious disease and has now included "biopharmaceutical manufacturers". The focus of these regulations is on companies manufacturing infectious disease treatments, antibiotics, ventilators, and extracorporeal membrane oxygenation (ECMO) equipment.
- **Takeda Pharmaceutical** is considering the sale of its consumer healthcare subsidiary in Japan as part of its drive to pay down debt following its Shire acquisition. It will also further its focus of resources on prescription medications, the company announced on April 24th. The sale of **Takeda Consumer Healthcare**, which offers non-prescription drugs such as *Benza Block* cold medicines and its *Alinamin* series of energy drinks and other health products, is expected to be worth 300 to 400 billion yen (US$2.8-3.76 billion) according to informed sources.
- Pharmaceutical manufacturer, **Roche**, has been given emergency use authorization by the U.S. FDA for its COVID-19 antibody test. Roche announced that the company will be putting US$459 million into its German manufacturing facility to boost production and the German government has signed a deal with Roche. They aim to produce 100 million tests per month by the end of 2020.

(Sources: Company Press Releases, Drug Store News, Newsweek, Scrip, and Pharma Japan)