ClearBridge Investments

Alzheimer's Approval Validates Biopharmaceutical Innovation

June 17, 2021

Key Takeaways

- FDA approval of Biogen's Aduhelm for Alzheimer's should be broadly supportive of biotechnology and pharmaceutical stocks as it signals the agency is willing to be flexible to make important drugs available to patients.
- The rerating of Biogen shares following the FDA decision demonstrates the potential of innovation in the biopharmaceutical industry to generate significant alpha for investors.
- We see gene therapy, rare diseases and oncology, as well as advanced diagnostic testing as other areas where R&D spending is leading to scientific breakthroughs with the potential to create new, large addressable treatment markets and address unmet needs.

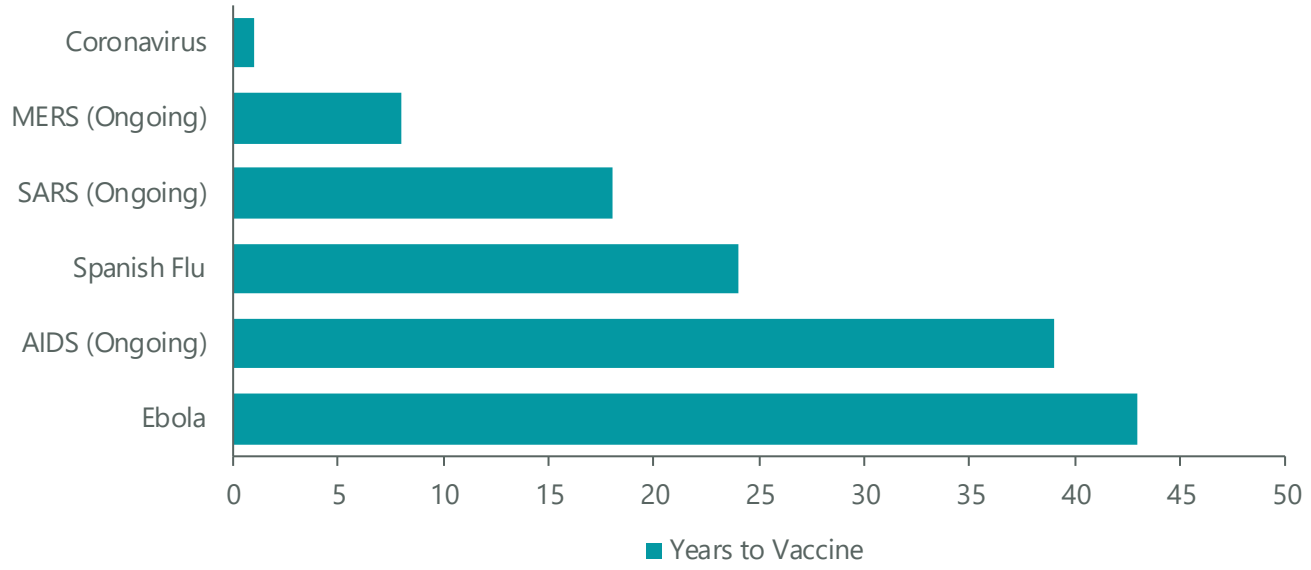
Supportive FDA an Early Step in Improving Investor Sentiment Toward Health Care

The Food and Drug Administration's approval of Biogen's Alzheimer's treatment earlier this month marks a breakthrough in addressing one of the largest unmet needs in health care. More than six million Americans are living with Alzheimer's today and Biogen's new treatment, Aduhelm, the first to address the buildup of amyloid plaque in the brain, could delay cognitive decline from the disease.

Mixed clinical trials had left the fate of Biogen's experimental treatment in doubt, but the FDA relied on Aduhelm's efficacy in plaque removal to grant accelerated approval for the drug. While using accelerated approval in this case is a new precedent, the practice is very well established in the development of oncology treatments.

Coming on the heels of unparalleled global research and development efforts to produce multiple COVID-19 vaccines in record time (Exhibit 1), the FDA's approval of Aduhelm should be broadly supportive of biotechnology and pharmaceutical stocks as it signals the agency is willing to be flexible, take risks and set new precedents to make drugs it feels are important available to patients. In the case of Aduhelm, neurologists with large Alzheimer's patient populations are excited to have any new, possibly disease-modifying therapeutic to offer their patients, and they have sufficient belief that clearing amyloid will produce a cognitive benefit that physicians will recommend Aduhelm to patients. Patient interest in the drug is extraordinary and we do not expect physicians will stand in the way of patient demand.

Exhibit 1: COVID-19 Vaccines Represent Power of R&D



Source: ClearBridge Investments. (Ebola: 1976–2019; AIDS: 1981–present; Spanish flu: 1918–1942; SARS: 2002–present; MERS: 2012–present).

A number of issues have yet to be worked out to determine Aduhelm's sales potential, but we forecast peak sales potential of many billions of dollars, depending on approval in non-U.S. markets and reimbursement decisions in the U.S. and abroad. The competitive landscape for anti-amyloid treatment in Alzheimer's is uncertain, but given the accelerated approval of Aduhelm, approval of treatments from Eli Lilly and Roche could follow quickly, or at least faster and at lower risk than originally assumed.

Aduhelm approval could also dispel concerns raised by a number of adverse regulatory outcomes in the past 12 months. More importantly, the rerating of Biogen shares following the FDA decision demonstrates the potential of innovation in the biopharmaceutical sector to generate significant alpha for investors.

We see a number of other treatment areas where R&D spending is leading to scientific breakthroughs with the potential to create new, large addressable treatment markets and address unmet needs. These areas include gene therapy, rare diseases and oncology, as well as advanced diagnostic testing for cancer and other early disease detection.

We believe there are multiple investment opportunities where the market is not valuing new product pipelines. In particular, small- and mid-capitalization biotechnology companies with promising drug candidates can generate enhanced returns based on clinical success. BioMarin Pharmaceutical, which has therapeutics in clinical trials to treat hemophilia and dwarfism, as well as Ultragenyx Pharmaceutical, a leader in gene therapy to address rare diseases, are examples of companies making progress toward commercialization of products to serve unmet needs.

About the Authors

Marshall Gordon
Director, Senior Research Analyst for Health Care
- 21 years of investment industry experience
- Joined ClearBridge Investments in 2008
- MBA from University of Chicago
- BS in Finance from the Wharton School of the University of Pennsylvania

Nicholas Wu, PhD
Director, Senior Research Analyst for Health Care
- 19 years of investment industry experience
- Joined ClearBridge Investments in 2007
- PhD in Chemistry from Stanford University
- BA in Chemistry from Washington University in St. Louis