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PRESS RELEASE

Changes to the Accelerated Approval Pathway Would See 66% of Those Therapies No Longer Being Developed

Lost treatments cumulatively address the needs of 850k to 3.6 mil patients

Tuesday, June 14th, 2022

New research to be presented at the BIO International Conference in San Diego by the healthcare consultancy Vital Transformation shows that if the FDA's accelerated pathway was not available, or significant restrictions were placed on accelerated approval drugs, it would likely result in as many as two-thirds of these treatments failing to reach patients. This impact was calculated by using an estimated delay in market access of three years, the median time it currently takes to provide the required evidence to the FDA.

The importance of the accelerated approval was first noted decades ago: "Mindful of the fact that it may take an extended period of time to measure a drug's intended clinical benefit, in 1992 FDA instituted the Accelerated Approval regulations. These regulations allowed drugs for serious conditions that filled an unmet medical need to be approved based on a surrogate endpoint. A surrogate endpoint is a laboratory measurement, radiographic image, physical sign, or other measure that …is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on irreversible morbidity and mortality."

However, there are some payers, academics, and state and federal policymakers who believe that the pathway needs to be significantly altered or restrictions applied to drugs approved through the accelerated approval pathway.

To test the impact of these changes to the pathway, Vital Transformation calculated the Net Present Value (NPV) of 93 primary accelerated approval therapies—those drugs with only one FDA-approved indication—which were approved by the FDA from 2001-2021.

According to Vital Transformation's CEO Duane Schulthess, "For 93 accelerated approvals from 2001 – 2021, we built an economic model simulating the impact of a delay in FDA's marketing authorization of 2, 3, 4, and 5 years due to changes in the use of the pathway. What emerged was 82% of therapies harnessing the accelerated approval are orphan conditions, and the size of the population being

treated by the new drug has an enormous impact on how long it takes to generate the confirmatory study evidence required by the FDA. The smaller the patient population for the FDA-approved indication, the longer the confirmatory study needs to be, and the less likely it is for the drug to have a positive net present value (NPV), which is a standard financial calculation to determine if the investment is feasible. If the NPV is negative, a company may no longer invest in the therapy, or its development could be halted. Our study found that with a delay of 3 years, nearly two-thirds of the NPVs we tested turn negative."

"This report highlights the clear value of the accelerated approval pathway for millions of Americans. The accelerated approval pathway has increased patient access to lifesaving and life-enhancing medicines for a variety of debilitating conditions. The report data show that up to 3.6 million patients benefitting from these therapies – particularly those suffering from a rare disease - could lose this access if onerous proposals advance," said John Murphy, Chief Policy Officer for the Biotechnology Innovation Organization.

The accelerated approval pathway also allows for additional 'secondary' indications, which are increasingly seen with skepticism by some payers and academics, despite each indication meeting the established criteria for use of the accelerated approval pathway. Vital Transformation's study shows that Gleevec, Opdivo, and Keytruda, three highly effective therapies on the WHO's essential medicines list, are responsible for over 50% of all secondary FDA-approved indications, as they are highly effective late-stage treatments for multiple different cancers. Outside these three therapies, the vast majority of therapies with secondary indications only have one additional FDA-approved indication.

While the research also shows that additional indications appear to both ensure an overall net positive NPV and help to keep drugs in the market for patients in need, genetically-targeted therapies, like RNA technologies, gene and stem cell therapies with a genetic biomarker, don't have the opportunity for secondary indications. They are for specific, often very small orphan indications. Thus, the accelerated approval pathway plays a critical role in ensuring genetically targeted technologies for orphan diseases—particularly slowly progressing diseases where measuring a clinical endpoint will take years—will be developed for patients with high unmet needs.

In fact, according to research published in Nature, 85% of all untreated orphan indications have incidence rates of less than 1/1,000,000, representing a total US population of 330 people. Changes to the accelerated approval pathway by setting an arbitrary timeline for completion of confirmatory trials to five years or less, as proposed by Energy and Commerce Committee Chairman Frank Pallone, Jr. (D-NJ), will likely render the development of 85% of currently untreated orphan conditions economically untenable.

Vital Transformation will present their research at the 2022 International BIO conference at the San Diego Convention Center on Wednesday June 15th, 2022, at 11am (Pacific). The full research pack and underlying data is available for download at https://vitaltransformation.com/2022/06/calculating-the-value-and-impact-of-accelerated-approvals/

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About Vital Transformation:
Vital Transformation understands the implications of new medical procedures, technologies and policies. We measure their impact on current clinical practices in close collaboration with health care professionals, researchers, and regulators. Through our web platform and client network, we are able to communicate our findings with international decision makers and stakeholders. Our Vital Transformation branded roundtables, webinars, and conferences are often oversubscribed, and are regularly presented in partnership with global thought-leaders and organizations.

The full research pack and underlying data is available for download at www.vitaltransformation.com.

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