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**PRESS RELEASE**

# Delays to Medicaid Coverage of Accelerated Approval therapies in the US would adversely impact between 66,000 and 319,000 of patients further causing health disparities among those the program is intended to support

## Spending on Accelerated Approval therapies accounts for just 0.5% of total state Medicaid budgets

## Tuesday 15 November, 2022, 8am ET

New research from the consultancy Vital Transformation found that nearly half of accelerated approval treatments - many for rare diseases- could be at high-risk of not coming to market or being developed at all if recent proposals to restrict the Food and Drug Administration's accelerated approval pathway are advanced. More than 300,000 patients annually could be adversely affected by proposals aimed at cutting costs despite spending on accelerated approval therapies accounting for less than half a percentage of total state Medicaid budgets.

Under the FDA's accelerated approval pathway, treatments developed to fulfill an unmet need, like a rare disease or cancer, could be approved based on surrogate endpoints, rather than large, randomized clinical trials. 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. Some policymakers have suggested proposals to deny Medicaid coverage unless the drug developer provides additional evidence that is often increasingly difficult to gather given small population sizes and complex progression of the disease. Given that many of these treatments are for patient populations with life-threatening conditions, delays in access could have devastating consequences. Specifically, the study calculated the impact 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.

According to Vital Transformation CEO Duane Schulthess, "75% of accelerated approvals provide their required evidence to the FDA within four years. For the 25% that take longer, a statistically established relationship indicates that those therapies often target small orphan conditions that require clinical trials involving long-term outcomes. Without the accelerated approval pathway, many rare disease therapies become no longer economically viable. As a result, we would likely see many of the treatments for the more than 95% of untreated rare diseases remain undeveloped if states limit

reimbursement procedures for accelerated approval therapies within Medicaid."

When analyzing the 206 therapies approved under an accelerated pathway over the past 20 years, Vital Transformation found that the total US spending on those specific drugs within Medicaid consumed less than 0.5% of all state budgets. Despite the limited budgetary impact, states like Oregon have tried to use the Centers for Medicare and Medicaid Services (CMS) 1115 demonstration waivers to exclude coverage for accelerated approval therapies in a misguided attempt to save on cost. To date, CMS has denied those exclusions.

"If you are trying to meaningfully reduce state budgetary healthcare expenditures, restricting access to accelerated approved therapies within Medicaid seems to be a strange place to focus your efforts, as in many cases that spending accounts for little more than a rounding error," according to Harry P Bowen, Vital Transformation's consulting economist.

Efforts to reduce Medicaid spending on accelerated approved therapies also have been proposed by the Medicaid and CHIP Payment and Access Commission. In 2021, MACPAC made recommendations to increase the Medicaid rebate until confirmatory trials are complete. New research from Vital Transformation finds that this policy would put one therapy for HIV and one for cancer at risk, depending on the size of the final agreed rebate.

Today's increased understanding of rare disease and advances in targeted drug development are making it possible to realize the promise of accelerated approval for rare diseases where current treatments don't yet exist, yet these policies could be detrimental l to realizing this potential.

"The traditional drug approval process does not always work for rare diseases due to small population sizes that are slow and variable from patient to patient, and patients that rely on this pathway should not fear risk of losing access to life-improving treatments. The accelerated approval pathway, which is subject to the same stringent, evidence-based clinical review at FDA, provides another option for companies to bring treatments to patients with orphan indications," said Amanda Malakoff, Executive Director of the Rare Disease Company Coalition. "The proposed changes to coverage for accelerated approval drugs could render the development of therapies for many untreated rare diseases economically untenable – while creating minimal cost savings and only increasing the burden of rare diseases on society at large. It's a lose-lose scenario for all."

The full research pack and underlying data is available for download at www.vitaltransformation.com. This study was funded and supported by BIO, Rare Disease Company Coalition (RDCC), Global CEO Initiative on Alzheimer's Disease (CEOi), Sarepta, Travere Therapeutics, Gilead, Alexion, AstraZeneca Rare Disease and Bristol Myers Squibb.

**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.

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Contact: info@vitaltransformation.com