75% of U.S. health plans reimburse off-label uses of prescription drugs

Reimbursement based mainly on published pharmaceutical compendia

- Off-label use has proliferated over the last decade, especially with monoclonal antibodies and other biologicals.
- About one-quarter of U.S. health plans do not reimburse for off-label use.
- Of the plans that reimburse off-label uses, over half impose conditions, including step therapy, indication restrictions, prior authorization, and quantity limits.
- Nearly 90% of survey respondents say pharmaceutical compendia form a basis for off-label use reimbursement decisions.
- Three-quarters of plans ascribe a very important role to peer-reviewed literature in off-label use reimbursement decisions.

Off-label use of prescription drugs in the United States, especially biologicals, has grown in recent years. Since patients do not pay directly for most off-label uses, third-party payers in these cases are crucial for market access. While reimbursement decisions with respect to on-label indications are relatively well understood, in terms of evidence requirements, off-label use reimbursement is less transparent.

To get a better understanding of the off-label use reimbursement landscape, specifically the evidence-based criteria driving reimbursement decisions, Tufts CSDD conducted a survey of third-party payers administering Medicare and Medicaid pharmacy benefits. The findings are summarized in this report.

Most importantly, the survey found that payers vary considerably in their policies regarding payment for off-label uses, as well as the sources used to justify those reimbursement decisions. As off-label prescribing grows, health plans must balance the value of rapid patient access to the best pharmaceutical care possible—sometimes through the off-label use of a drug—with the need for comprehensive data on the benefits and risks of such care. The establishment of a comparative effectiveness research clearinghouse, as a source of reliable information that can be applied in evidence-based payer decisions, could provide the infrastructure to address the issue.