Ongoing Accelerated Approval Trends and What It Means for Patients

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Non-oncology drugs make up **one-third of total accelerated approvals (AA),** with the majority being in rare diseases; Payer exclusions to non-oncology AA drugs may have **devastating consequences** for patients with rare diseases.

BACKGROUND

- The Food and Drug Administration (FDA) created the Accelerated Approval (AA) Program "to allow for earlier approval of drugs that treat serious conditions, and fill an unmet medical need based on a surrogate endpoint."[1]
- As of January 1, 2025, Independence Blue Cross (IBX) in Pennsylvania has excluded non-oncology drugs from reimbursement for 18 months after accelerated approval.[2]
- Payer exclusions to novel non-oncology AA-approved drugs may have devastating consequences for patients, yet the number of ongoing non-oncology accelerated approvals impacted by this policy has not been explored.

OBJECTIVES

- We sought to explore how Independence Blue Cross' policy impacts the current landscape of ongoing AA non-oncology drugs.

METHODS

- We analyzed the FDA AA program drug list at different timeframes between the first AA (September 6, 1996) and the start of the IBX commercial medical policy (January 1st, 2025), to understand the ongoing AA drug mix by therapeutic area for non-oncology drugs.
- We included all ongoing oncology and non-oncology AA drug indications that have not been withdrawn from the market as of January 1st, 2025, and have yet to convert to full FDA approval.
- The FDA's Orphan Drug Product Designation database was used to determine if a disease was classified as rare.[3]

RESULTS

Figure 1. Ongoing Accelerated Approvals from 1996-2024, (N=96)

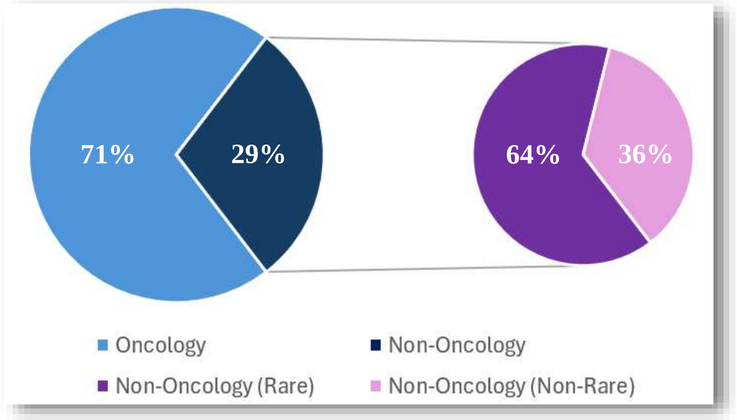
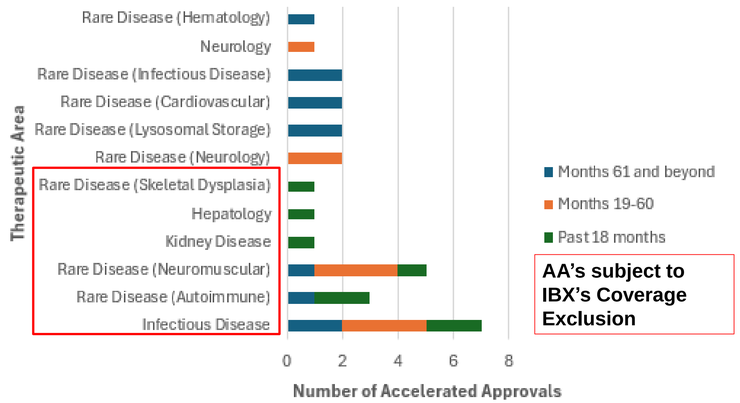


Figure 2. Timeline of Ongoing Non-Oncology Accelerated Approvals, (N=28)



RESULTS (Cont.)

- Since 1996, we found 96 total ongoing drug-indication accelerated approvals, of which 29% were non-oncology indications (28/96); almost two-thirds of total non-oncology indications are within rare diseases (18/28, or 64%). See **Figure 1** for more details.
- In the past 18 months, there was a similar proportion of non-oncology drug indications (29% or 8/28; **Figure 2**).
- From 2019-2024, we found 17 ongoing non-oncology drug-indication AA; most of these drug-indications were for rare diseases (9/17, or 53%), and the remaining non-rare indications were for infectious, kidney, hepatic, or neurological diseases.

DISCUSSION & CONCLUSION

- We found that non-oncology drugs make up approximately one-third of total AA and that rare diseases make up the majority of non-oncology AA.
- Rare disease patients often have high unmet medical needs and economic burdens.[4]
- Payer exclusions to non-oncology AA drugs may have devastating consequences for patients with high unmet needs.
- A past analysis found that commercial plans were more likely to impose coverage limits on AA non-oncology and/or orphan drugs than oncology drugs;[5] future research is needed to understand how delayed coverage may impact patients with high unmet medical needs.

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