Is Drug Novelty Associated With Greater Health Benefits?

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Drugs intended to **treat a serious condition**, **offer meaningful improvements over available therapies**, and **address unmet clinical needs** had **larger health gains** than drugs without these novelty attributes.

BACKGROUND

- Health Technology Assessment agencies consider multiple criteria when judging novelty, such as whether the product provides a new approach to treatment or if it addresses an unmet clinical need.
- However, it is unclear whether drugs meeting these novelty criteria confer greater health gains than drugs that do not; in other words, the association between a drug's novelty and the magnitude of its health gains remains unmeasured.

OBJECTIVE

- To examine the association between the drug novelty attributes (1) *treats a serious condition;* (2) *offers meaningful improvement over available therapies*, and (3) *addresses unmet clinical needs* and the magnitude of added health gains offered by new drugs.

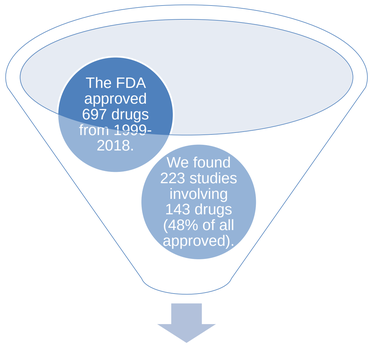
METHODS

- To obtain added health gain estimates, we searched PubMed for comparative- and cost-effectiveness studies reporting Quality Adjusted Life-Years (QALYs) for drugs approved by the FDA between 1999-2018. We included only non-industry-funded studies in the US setting that compared drugs to the standard of care at the time of FDA approval.
- To derive drug novelty attributes, we assigned binary (yes/no) indicators for three novelty attributes at the drug-indication level (e.g., vedolizumab for ulcerative colitis).

Novelty attribute	Definition*
Treats a serious condition	Included in ≥1 FDA expedited approval program (Fast Track, Breakthrough Therapy, Accelerated Approval, & Priority Review).
Offers meaningful improvement over available therapies	Included it in at least one of three FDA expedited approval programs: Breakthrough Therapy, Accelerated Approval, or Priority Review.
Addresses unmet clinical needs	Included in Fast Track program.

*Program inclusion sourced from FDA Center for Drug Evaluation and Research reports.

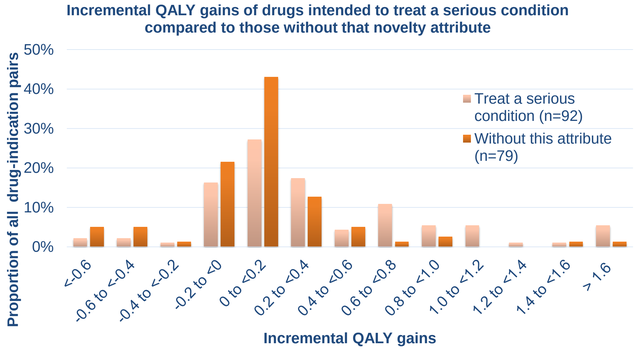
- We compared incremental QALY gains for pairs with each novelty attribute to gains for pairs without that attribute using two nonparametric tests: first using Mann-Whitney U (MWU) tests, which compare medians, and Kolmogorov-Smirnov (K-S) tests, which compare the cumulative distribution functions of two samples.



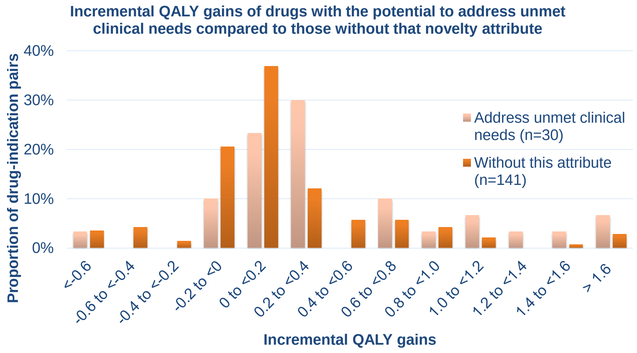
Our sample comprised 171 drug-indication pairs.

RESULTS

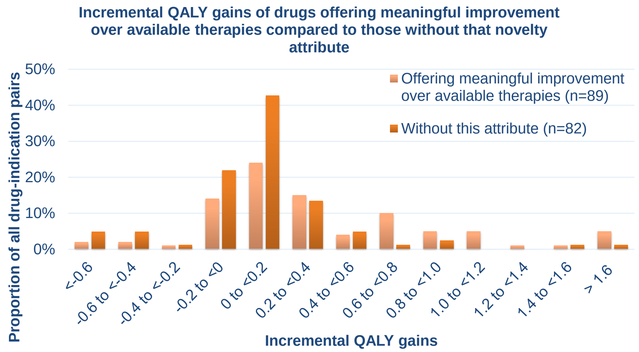
- Drug-indication pairs intended to *treat a serious condition* (n=92) had median gains of 0.21 QALY versus 0.04 QALY (p<0.01) for pairs without that attribute (n=79).



- Drug-indication pairs with the potential to *address unmet clinical needs* (n=30) had median gains of 0.21 QALY versus 0.04 (p<0.01) QALY for pairs without that attribute (n=141).



- Drug-indication pairs *offering meaningful improvement over available therapies* (n=89) had median gains of 0.28 QALY versus 0.06 QALY (p<0.01) for pairs without that attribute (n=82).



- All tests were significant at the α=0.01 level.

LIMITATIONS

- Our study examined only drugs with available incremental QALY estimates from published studies in US settings.
- We did not examine all aspects of drug novelty, for example, new methods of drug administration.

CONCLUSIONS

- Drugs with novelty attributes – (1) *treats a serious condition;* (2) *offers meaningful improvement over available therapies*, and (3) *addresses unmet clinical needs* – were associated with larger health gains than drugs without these attributes.
- Though our work does not examine all aspects of drug novelty, it is the first to study the association between key novelty attributes and drugs' added health benefits.
- Our findings suggest that the FDA's criteria for including drugs in expedited approval programs are associated with larger health benefits.
- Future research should empirically examine other aspects of drug novelty important to patients, e.g., modified dosing schedules.

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