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Drug Repurposing as a Strategic Approach to Accelerate Therapeutic Innovation

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Opinion

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Abstract

Drug development is characterized by substantial financial investment, lengthy timelines, and high attrition rates between discovery and regulatory approval. Drug repurposing, also known as drug repositioning, provides a complementary strategy by identifying new therapeutic applications for existing medicines or investigational compounds. Because repurposed candidates may have previously characterized pharmacological, pharmacokinetic, safety, and manufacturing profiles, the approach can potentially reduce some of the uncertainty, cost, and time associated with conventional drug development.

Keywords: Drug Repurposing; Drug Repositioning; Therapeutic Innovation; Drug Discovery; Drug Development; Artificial Intelligence; Precision Medicine; Translational Medicine; Clinical Trials

Abbreviations

Drug Repurposing; Drug Repositioning; Artificial Intelligence; Machine Learning; Real-World Evidence; Randomized Controlled Trial.

Introduction

The development of new medicines traditionally involves target identification, lead discovery, optimization, preclinical evaluation, clinical trials, regulatory assessment, and post-marketing surveillance. Although this process has produced major therapeutic advances, it remains time-consuming, expensive, and associated with substantial attrition. Drug repurposing has consequently emerged as an attractive complementary strategy for identifying new therapeutic uses for existing medicines. Repurposing may offer advantages because information concerning a candidate's pharmacology, safety, formulation, or human exposure may already be available, potentially reducing some elements of development uncertainty.

At the canter of modern drug repurposing is the ability to connect existing medicines with previously unrecognized disease mechanisms. Traditional repurposing can arise from clinical observations or serendipitous findings, whereas systematic approaches increasingly use molecular databases, disease signatures, drug-target networks, transcriptomic profiles, electronic health records, and computational models. These resources can help researchers identify relationships between medicines and diseases that may not be obvious from conventional drug-development pipelines. Computational methods can therefore function as a powerful hypothesis-generation tool for prioritizing candidates for subsequent laboratory and clinical investigation [1].

Artificial intelligence and machine-learning approaches are further changing the landscape of drug repurposing. By

integrating heterogeneous biological and clinical datasets, computational systems can identify potential drug-disease associations, predict molecular interactions, and prioritize compounds for experimental testing. Such approaches may be particularly valuable when large-scale biomedical information is available but conventional screening of every possible drug-disease combination would be impractical. Nevertheless, computational predictions should not be interpreted as evidence of clinical effectiveness. Their primary value is to narrow the search space and generate hypotheses that require independent experimental and clinical validation.

The COVID-19 pandemic provided an important real-world demonstration of both the potential and limitations of drug repurposing. Existing medicines were rapidly investigated because developing completely new treatments would require considerably more time. Computational approaches were used to prioritize candidate medicines, while laboratory and clinical studies evaluated their potential against SARS-CoV-2. However, several medicines that appeared promising through early experimental or observational evidence did not subsequently demonstrate meaningful clinical benefit. These experiences emphasized that rapid identification of candidates must be followed by appropriately designed clinical studies and careful interpretation of evidence.

An important challenge is therefore the transition from repurposing hypothesis to therapeutic evidence. A medicine may possess a biologically plausible mechanism but fail to achieve adequate concentrations at the relevant site of disease. Differences in disease stage, patient characteristics, dosage, treatment duration, drug interactions, or disease biology may also influence outcomes. Consequently, the fact that a medicine is already approved for one indication does not automatically establish its safety or efficacy for a new therapeutic purpose. New indications may require additional pharmacological, toxicological, pharmacokinetic, and clinical evaluation [2].

Regulatory and economic considerations are equally important. Repurposing an established or off-patent medicine may reduce certain development risks, but commercial incentives can be weaker when market exclusivity is limited. At the same time, evidence requirements for a new indication may remain substantial. Recent research highlights scientific, clinical, regulatory, economic, and implementation barriers that can prevent promising repurposing candidates from progressing from laboratory findings to patient access.

A strategic repurposing framework should therefore combine multiple evidence streams rather than depend on a single discovery method. Computational predictions can identify candidate compounds; molecular and cellular studies can investigate mechanisms; pharmacokinetic analyses can determine whether therapeutically relevant exposure is achievable; and clinical trials can establish efficacy and safety in the proposed indication. Real-world evidence may subsequently contribute information about effectiveness, treatment patterns, and outcomes in broader populations.

The integration of repurposing with precision medicine represents another important opportunity. A medicine that does not demonstrate substantial benefit across an entire disease population may still have value in a biologically defined subgroup. Genomic profiles, biomarkers, disease subtypes, molecular signatures, and clinical characteristics could therefore be used to identify patients most likely to respond. This approach may transform repurposing from broad candidate screening into a more targeted strategy for matching existing pharmacological mechanisms with specific unmet clinical needs.

Drug repurposing should consequently be viewed neither as a shortcut that eliminates conventional development nor as a replacement for new-molecule discovery. Rather, it can function as an additional pathway within the therapeutic innovation ecosystem. The strongest repurposing programs are likely to be those that combine computational prediction with biological plausibility, experimental validation, appropriate clinical design, regulatory planning, and clearly defined patient needs.

References

1. Ushpakom S, Iorio F, Eyers PA (2019) Drug repurposing: progress, challenges and recommendations. Nature Reviews Drug Discovery 18(1): 41-58.

2. Galindez G, Matschinske J, Rose TD (2021) Lessons from the COVID-19 pandemic for advancing computational drug repurposing strategies. Nature Computational Science 1: 33-41.

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