Strategic Shift in Drug Repurposing: Addressing Rare Diseases.

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INTRODUCTION

Drug repurposing presents a transformative, cost-effective strategy for developing new treatments, particularly for rare diseases. Unlike *de novo* drug discovery, which is often hindered by long timelines and exorbitant costs (~ $314M-$2.8B per drug) repurposing leverages known pharmacological profiles and existing safety data. This approach accelerates therapeutic access and mitigates risk, often achieving regulatory approval in 3 to 12 years compared to the decade or more required for new drugs for rare diseases, where limited patient populations pose unique challenges, repurposing addresses critical unmet needs with greater efficiency, accelerating development timelines and improving patient outcomes.

OBJECTIVES

• To analyze trends in repurposing within FDA-approved CDER Biologics License Applications (BLAs) and New Drug Applications (NDAs), identifying the increasing prevalence of repurposed drugs (RD) over time.

• To highlight the therapeutic areas most frequently targeted by drug approvals transitioning into new RD indications, emphasizing significant impacts and trends in addressing rare diseases.

• To illustrate the original therapeutic indications and their transitions to RD indications, providing insights into strategic pathways and industry dynamics within drug repurposing.

METHOD

- A comprehensive database was constructed, encompassing all CDER FDA-approved BLA's and NDAs from 1985 to present.
- Data collection involved detailed review and validation using the FDA's Drugs@FDA repository, with cross-referencing of pharmaceutical registries and relevant scientific literature to ensure accurate verification of repurposing status and therapeutic indications
- Key parameters captured include approval dates, therapeutic targets, associated pharmaceutical companies, and detailed timelines for drug repurposing, specific (regulatory) designations, approval details providing a broad view of drug lifecycle dynamics.
- The database emphasizes capturing significant therapeutic innovations and their transitions to new indications, deliberately excluding line extensions and redirection to focus on strategic shifts and impactful therapeutic advancements within the pharmaceutical industry.

RESULTS

Our data shows a clear increase in repurposing approvals over the years (Fig. 1), reflecting the industry's response to rising R&D costs and regulatory complexities, often magnified by the patent cliff. This shift towards repurposing is economically driven but strategically aligned with reducing development time and leveraging existing safety profiles.

Therapeutic Focus on Rare Diseases

RD are predominantly developed in the field of genetic disorders, representing approximately 18% (74 out of 415) of the total repurposed cases (Fig. 2)., highlighting the industry's focus on rare diseases. This prominence can be attributed to regulatory incentives such as the Orphan Drug Act (USA, 1983), which encourages innovation in small patient populations through benefits like market exclusivity and expedited regulatory review. The prioritization of rare genetic conditions emphasizes both clinical need and strategic market positioning, making repurposing a highly efficient pathway for impactful treatments.

Strategic Pathways of Repurposing

The Sankey diagram (Fig. 3) illustrates the flow of repurposed drugs, showing their original disease target and their repurposing into new therapeutic areas. Key areas like cardiovascular diseases and neurology often serve as starting point, with compounds transitioning into areas like genetic disorders and oncology. This interconnectedness reflects a strategic approach where a shared molecular mechanism of action, such as inflammation modulation or targeted molecular pathways, enable broader applications of existing compounds. The diagram underscores how pharmaceutical companies maximize therapeutic utility while adapting swiftly to evolving healthcare needs.

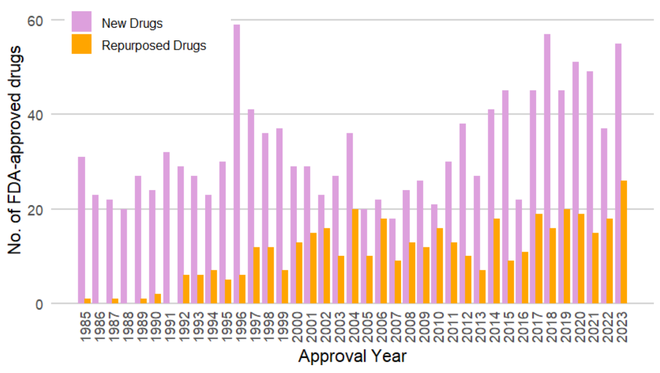

Figure 1: Comparison of New and Repurposed FDA-approved Drugs (1985-2023)

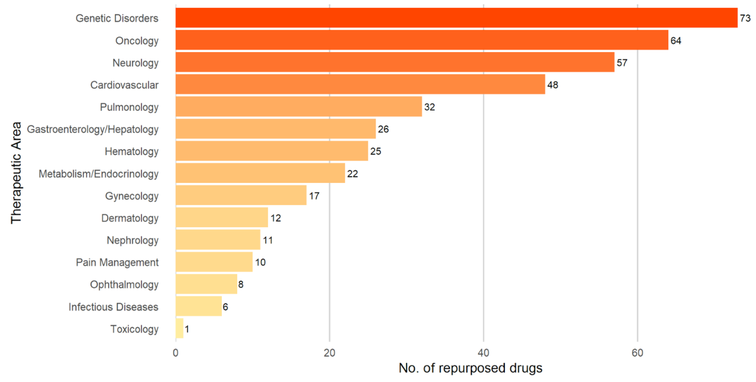

Figure 2: Classification of FDA-approved Repurposed Drugs by Therapeutic Area (1985-2023)

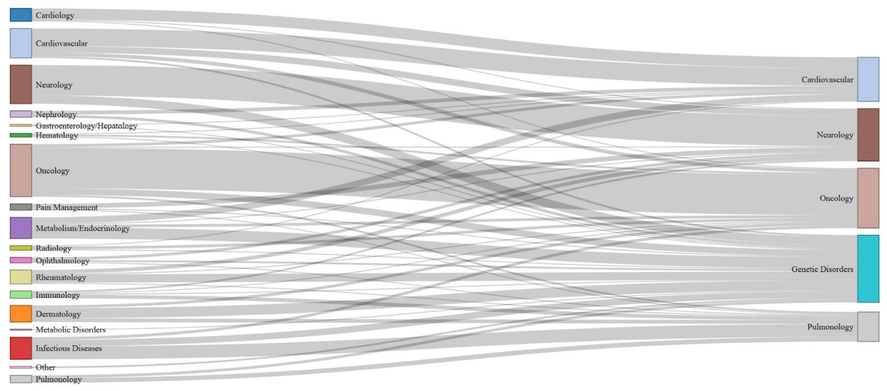

Figure 3: Sankey Diagram of FDA-Approved Drugs Showing Transitions from Original to top 5 Repurposed Therapeutic Areas

CONCLUSIONS

Key Insights

Drug repurposing has emerged as a transformative strategy to address rare diseases, providing faster, more cost-effective routes to market while maximizing existing therapeutic assets. The increasing frequency of FDA RD approvals and the dominance of genetic disorders as target areas underscore a strategic alignment between industry priorities, regulatory incentives, specific market exclusivities, and pressing public health needs. By leveraging known pharmacological profiles, repurposing not only accelerates access to vital treatments, but also exemplifies innovation in navigating regulatory and R&D challenges. This approach highlights how industry-driven repurposing efforts, supported by policy measures, can align profit motives with public health goals, ultimately bridging therapeutic gaps for rare disease patients and creating a meaningful, lasting impact across healthcare.

REFERENCES

Data compiled from a proprietary database of CDER-approved BLAs and NDAs (1985–present), constructed using source data from FDA's Drugs@FDA repository.

Upcoming Publication: Akodad S. et al. "Strategic Insights into Drug Repurposing for Rare Diseases." To be submitted for peer review.

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