Is the future of drug discovery already on your pharmacy shelf?

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> Bringing a new drug to market is a decade-long, multi-billion-dollar endeavor, a process that often leaves significant clinical needs unaddressed for years. Drug repurposing, the strategic identification of new uses for already approved compounds, offers a more efficient path. The FDA has now formalized its approach to this expedited route, aiming to leverage existing safety and pharmacokinetic data to accelerate therapies for conditions lacking adequate treatment.

KEY TAKEAWAYS

- The Pivot: The FDA is actively advancing drug repurposing by streamlining regulatory pathways and encouraging data-driven approaches to identify new indications for existing approved drugs.
- The Data: Computational pipelines, integrating curated drug-target interaction data from resources like ChEMBL, BindingDB, and GtoPdb, can predict repositioning opportunities for FDA-approved drugs across various disease contexts, including 10 major cancer types.
- The Action: Clinicians should be aware of the increasing potential for existing drugs to gain new indications, particularly in areas of high unmet need, as regulatory bodies and researchers prioritize these expedited development pathways.

The traditional drug discovery pipeline is notoriously inefficient, demanding over a decade and substantial financial investment to bring a single therapeutic to market. This protracted process often leaves significant gaps in treatment options for numerous diseases, creating unmet medical needs that persist for years. Drug repurposing, the systematic identification of novel indications for existing approved drugs, presents a compelling alternative, offering a cost-effective and expedited pathway to new therapies.1

This approach leverages the extensive safety and pharmacokinetic data already accumulated for approved compounds, bypassing many of the early-stage development hurdles inherent in de novo drug discovery. The FDA's increasing focus on facilitating drug repurposing reflects a strategic shift towards more efficient drug development, particularly for conditions where current treatments are inadequate or non-existent.1,3

Mapping the Repurposing Landscape

A recent study by Savander, Curabaz, and Abbasi provided a comprehensive framework for data-driven drug repurposing, consolidating drug-target interaction data from three extensively curated resources: ChEMBL, BindingDB, and GtoPdb.1 These databases, with their distinct release histories, curation methodologies, and coverage of approved and investigational compounds and targets, offer a rich foundation for identifying repositioning opportunities. The

investigators manually classified ChEMBL targets into 12 high-level biological families and mapped 817 clinically approved drug indications into 28 broader therapeutic groups. This structured framework allowed for a systematic profiling of physicochemical properties among approved drugs across these therapeutic categories.1

The analysis revealed specific associations between physicochemical characteristics and therapeutic groups, providing practical guidance for indication-specific compound prioritization in repurposing studies. For instance, drugs within certain therapeutic categories exhibited distinct molecular profiles, suggesting that compounds sharing these profiles might be more likely to succeed in similar indications. The study also examined cross-indication drug approvals, identifying areas with high repurposing potential where a single drug had already demonstrated efficacy across multiple, seemingly disparate conditions.1

To demonstrate the practical application of their framework, Savander and colleagues implemented a pathway-based computational pipeline. This pipeline predicted repositioning opportunities for FDA-approved drugs across 10 major cancer types, showcasing its adaptability to other disease contexts. The computational approach integrates diverse biological data, including gene expression profiles, protein-protein interaction networks, and disease-specific pathways, to identify drugs that could modulate disease-relevant targets or pathways in new indications.1

Daclatasvir's Potential in MASLD

One compelling case study in drug repurposing involves daclatasvir, an antiviral agent initially approved for hepatitis C virus (HCV) infection. Pirola explored the potential of repurposing daclatasvir for metabolic dysfunction-associated steatotic liver disease (MASLD), a condition with significant unmet medical needs.2 MASLD, formerly known as non-alcoholic fatty liver disease (NAFLD), affects a substantial portion of the global population and can progress to more severe forms such as metabolic dysfunction-associated steatohepatitis (MASH), cirrhosis, and hepatocellular carcinoma. Current treatment options for MASLD are limited, primarily focusing on lifestyle modifications, with no FDA-approved pharmacological therapies specifically for the condition.2

Daclatasvir, a direct-acting antiviral, targets the HCV non-structural protein 5A (NS5A). But its mechanism of action extends beyond viral replication. Pirola's work highlights that NS5A inhibitors like daclatasvir may exert pleiotropic effects, including anti-inflammatory, antifibrotic, and metabolic modulatory properties. These additional effects make daclatasvir a candidate for conditions beyond HCV, particularly those involving chronic inflammation and metabolic dysregulation, such as MASLD. The rationale for repurposing daclatasvir for MASLD therapy stems from its potential to interfere with pathways implicated in MASLD pathogenesis, including lipid metabolism, oxidative stress, and inflammatory responses.2

Still, the path to repurposing daclatasvir for MASLD is not without challenges. While preclinical data and observational studies in HCV-infected patients treated with daclatasvir have shown some encouraging signals regarding liver fat reduction and improvements in liver enzymes, dedicated clinical trials in MASLD patients are essential. These trials must rigorously evaluate efficacy, optimal dosing, and long-term safety in a patient population distinct from those with HCV. The potential for drug-drug interactions, particularly in MASLD patients who often have multiple comorbidities and polypharmacy, also requires careful consideration. Clinicians managing patients with MASLD may find the Sherlock's Diseases of the Liver and Biliary System a useful reference for the latest insights into hepatobiliary disease management.2

Regulatory Pathways for Modified New Drugs

The regulatory landscape for repurposed drugs is distinct from that of entirely novel compounds. Fu, Dong, and Xie conducted a multi-dimensional comparative study of 505(b)(2) New Drug Applications (NDAs) approved by the FDA and Class 2 NDAs approved by China's National Medical Products Administration (NMPA) from 2017 to 2023. This analysis uncovered trends, characteristics, and regulatory nuances of modified new drugs, which often include repurposed compounds.3

The 505(b)(2) pathway in the US allows applicants to rely on the FDA's existing findings of safety and effectiveness for a previously approved drug, or on published literature, rather than conducting entirely new studies. This pathway significantly reduces the time and cost associated with drug development. It is frequently utilized for drugs with new indications, new dosage forms, new strengths, or new routes of administration. The study by Fu and colleagues highlighted that a substantial proportion of 505(b)(2) approvals involved drugs that were, in essence, repurposed or modified versions of existing therapies.3

The analysis identified key characteristics of drugs approved via this pathway, including their therapeutic areas, molecular structures, and the types of modifications made. Oncology and infectious diseases were prominent therapeutic areas benefiting from the 505(b)(2) pathway, aligning with the high unmet needs in these fields. The study also detailed the regulatory requirements and review processes for these modified new drugs, providing insights into how agencies like the FDA and NMPA evaluate and approve repurposed compounds. Understanding these regulatory trends is critical for pharmaceutical companies and researchers aiming to navigate the repurposing landscape efficiently.3

The Data-Driven Future of Repurposing

The consolidation of drug-target data and computational repurposing into a data-driven framework, as presented by Savander and colleagues, represents a significant advancement. This framework integrates diverse biological and chemical information to systematically identify and prioritize repurposing candidates. The use of curated resources like ChEMBL, BindingDB, and GtoPdb ensures that the underlying data is robust and reliable. These databases provide comprehensive information on drug-target interactions, including binding affinities, mechanisms of action, and therapeutic indications, which are all crucial for successful repurposing efforts.1

The manual classification of ChEMBL targets into high-level biological families and the mapping of approved drug indications into broader therapeutic groups create a structured and interpretable dataset. This organization facilitates the identification of patterns and relationships that might not be apparent in raw, unstructured data. For example, understanding the physicochemical properties associated with successful drugs in a particular therapeutic group can guide the selection of new candidates for that same group. This systematic approach reduces the element of chance in repurposing, making it a more predictable and efficient process.1

The computational pipeline for predicting repositioning opportunities, particularly its application to cancer, demonstrates the power of bioinformatics in accelerating drug discovery. By analyzing pathway-based interactions, the pipeline can identify drugs that modulate disease-relevant pathways, even if their primary indication is for a different condition. This adaptability to various disease contexts means the framework can be applied broadly, addressing unmet needs across a wide spectrum of medical conditions. The integration of such computational tools into the drug development process is essential for maximizing the potential of existing pharmacological assets.1

The open-label design of some initial repurposing studies is an obvious caveat, as is the reliance on preclinical data or observational findings. Rigorous, well-controlled clinical trials remain indispensable for validating the efficacy and

safety of repurposed drugs in their new indications. The trial was not powered to detect differences in all subgroups, and that gap matters for specific patient populations. Daclatasvir was tested only in HCV patients; whether benefits extend to the broader MASLD group remains unclear without dedicated trials.2

CLINICAL IMPLICATIONS

The FDA's renewed emphasis on drug repurposing is not merely a bureaucratic adjustment; it is a pragmatic acknowledgment of the economic and scientific realities of drug development. Clinicians should anticipate a more frequent emergence of existing drugs with novel indications, particularly for conditions that have long lacked effective treatments. This shift demands a continuous re-evaluation of familiar pharmacopoeia, moving beyond their original labels.

For general practitioners and specialists, this means staying abreast of new approvals for older drugs. The computational frameworks now being deployed, integrating vast datasets of drug-target interactions, will likely accelerate these discoveries. This data-driven approach, exemplified by the work on daclatasvir for MASLD, offers a more targeted and less serendipitous path to therapeutic expansion. It is a welcome development for patients with conditions like MASLD, where the current therapeutic arsenal is notably sparse.

The regulatory pathways, such as the 505(b)(2) in the US, are designed to expedite these approvals by leveraging existing safety data. This streamlining reduces the burden on developers, but it also places a greater onus on post-market surveillance to confirm long-term efficacy and safety in new patient populations. The industry will increasingly look to these pathways as a more efficient route to market, especially for orphan diseases or conditions with limited commercial appeal for de novo drug development.

Ultimately, the success of this repurposing push hinges on robust clinical validation. While computational predictions and preclinical data can identify promising candidates, definitive evidence from well-designed trials in the target patient population is non-negotiable. The dry wit of the scientific method still applies: a drug either worked or it did not, regardless of its previous life.

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