Systematic Drug Repositioning: A new paradigm in Drug Discovery

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Drug repositioning offers the possibility of faster development times and reduced risks in drug discovery. With the rapid development of high-throughput technologies and ever-increasing accumulation of whole genome-level datasets, an increasing number of diseases and drugs can be comprehensively characterized by the changes they induce in gene expression, protein, metabolites and phenotypes. Here we will describe two distinct approaches that utilize these data types to systematically evaluate and suggest new disease indications for new or existing drugs. The first approach dubbed the Connectivity Map (CMap) is a collection of genome-wide transcriptional expression data from cultured human cells treated with bioactive small molecules that enable the discovery of functional connections between drugs, genes and diseases through the transitory feature of common gene-expression changes.The second approach uses genetic associations from Genome Wide Association Studies (GWAS) to find alternative indications for existing drugs. Other approaches which take advantage of the availability of clinical data will also be discussed briefly.

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