Predictive model helps identify drugs currently in use that could be used to treat other ailments

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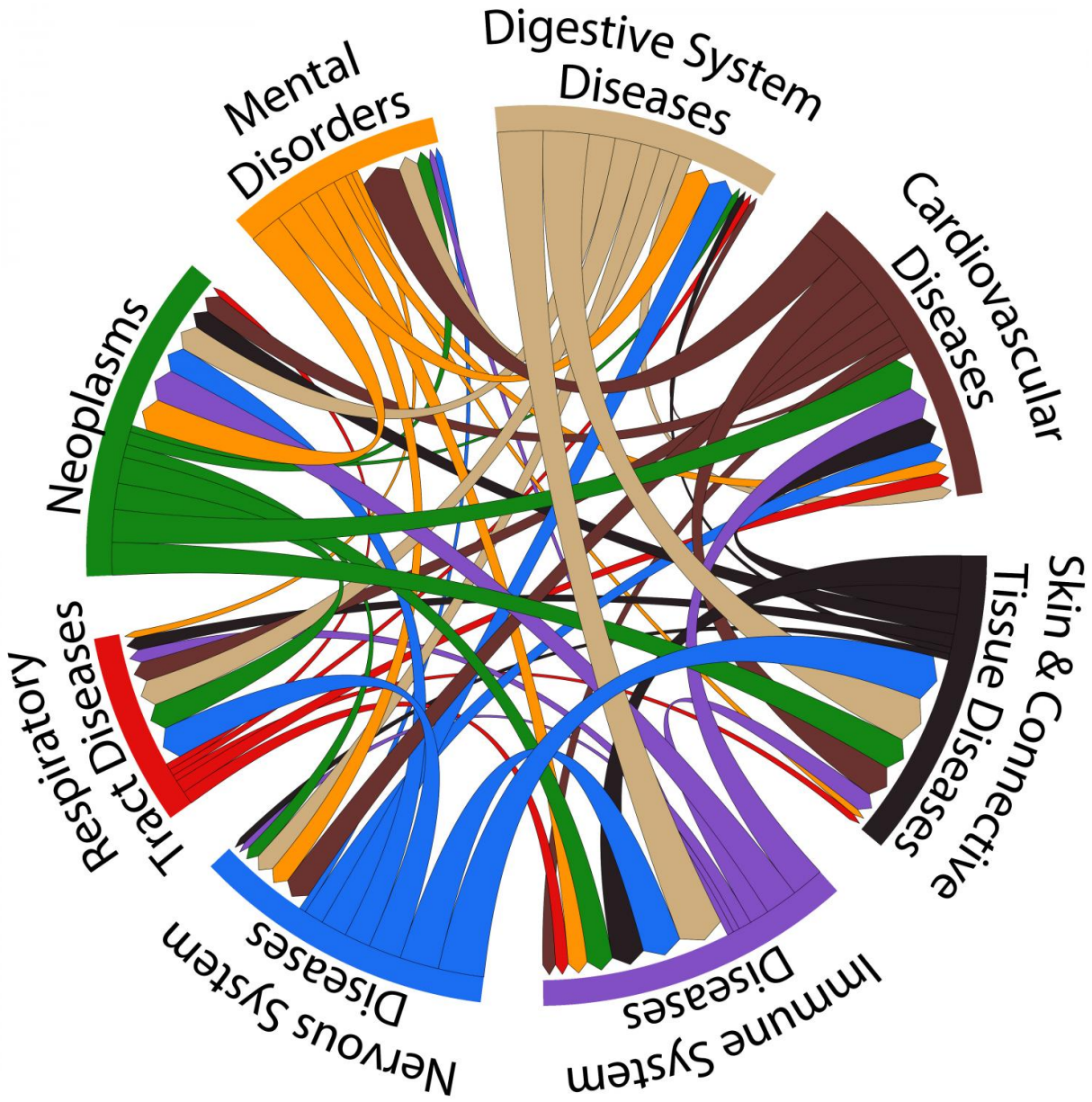

Schematic indicating different classes of drugs already approved for specific diseases that could potentially be repurposed for other conditions. Credit: C. Finan et al., Science Translational Medicine (2017)

(Medical Xpress)—A team of researchers from several institutions in the U.K. and one in the U.S. has developed a faster and cheaper way to figure out which drugs on the market might be useful for treating other ailments. In their paper published in the journal *Science Translational Medicine*, the team describes how they pulled information from databases and used it to identify possible treatment matches.

Developing new drugs to treat human ailments is notoriously slow and costly, and because of that, pharmaceutical companies are searching for new drug development techniques. In this new effort, the research team began with the idea that it is very likely that a lot of the drugs that have passed clinical trials and are now used to treat people for certain ailments might also work to treat some other ailments that have a similar genetic link. If true, such drugs could make it to patients much sooner, because they have already been shown to be safe. All a drug company would have to do is show that the drug actually helped patients with the second ailment. But figuring out which drugs might be used for other ailments is not an easy task.

To speed the process along, the researchers turned to databases of information on diseases that have been linked to a genetic cause and other databases of information about drugs currently on the market and the diseases they are meant to treat. They also developed an algorithm to sort the data from the various databases according to the genetic link between a given disease and the drug used to treat it.

Doing so offered the names of drugs used to treat ailments with certain types of genetic links tying them with other ailments with similar genetic markers. That gave the researchers a list of 4,479 potential drug possibilities. Next, a pair of clinicians looked at the possibilities and tossed out those that they knew would not work in the ways envisioned narrowing the list down to 144 drugs that could be tested to see if they actually helped patients with the targeted ailments. If so, not only would some patients receive a new therapy much more quickly, but the pharmaceutical company would save millions in development costs.

More information: Chris Finan et al. The druggable genome and support for target identification and validation in drug development, *Science Translational Medicine* (2017). DOI: 10.1126/scitranslmed.aag1166

Abstract
Target identification (determining the correct drug targets for a disease) and target validation (demonstrating an effect of target perturbation on disease biomarkers and disease end points) are important steps in drug development. Clinically relevant associations of variants in genes encoding drug targets model the effect of modifying the same targets pharmacologically. To delineate drug development (including repurposing) opportunities arising from this paradigm, we connected complex disease- and biomarker-associated loci from genome-wide association studies to an updated set of genes encoding druggable human proteins, to agents with bioactivity against these targets, and, where there were licensed drugs, to clinical indications. We used this set of genes to inform the design of a new genotyping array, which will enable association studies of druggable genes for drug target selection and validation in human disease.