Basic Science & Translational Research Category
Best Oral Paper Presentation

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How Data-driven Network Biology Can Help Achieve Precision Medicine
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Aims: There are an estimated 8,000 single gene diseases, and this number is increasing as genome sequencing enters mainstream healthcare. With very few medicines currently licensed for a rare condition, one strategy to improve the lives of people living with a rare disease is to reposition existing drugs with known safety to new clinical indications (drug "repurposing").

Methodology: I have previously demonstrated that it is possible to computationally predict sets of regulators that can induce one somatic cell type to transdifferentiate into another (Rackham et al, Nature Genetics 2016). This was possible by applying a carefully designed network-based algorithm to gene expression profiles taken from a large set of healthy cells and tissues. In recent years there has been a growth in the use of induced pluripotent stem cells (iPS) derived models of disease. This approach uses a disease-associated genotype to seed a population of iPS cells that, in turn, are differentiated into the relevant cell-type to study a disease. Taken together these disease models and my network-based approach make it possible to define the networks that underpin a disease and reposition existing drugs based on their ability to direct the gene expression and epigenetic profiles towards the healthy state.

Result: Using publically available RNA-seq data from iPS models of disease I will show a proof of principle that it is possible to use a data-driven approach to identify drugs using this approach.

Conclusion: We have already seen our ability to define disease more accurately increase dramatically through technological innovation, however our ability to identify treatments based on these definitions has not progressed at the same rate. Since it is becoming routine to generate patient-specific iPS cells and as a number of large consortia are making iPS cell banks publically available these results represent the first step towards a data-driven platform for drug repositioning.