Learning Effective Clinical Treatment Pathways from Observational Data

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Background

Treatment guidelines for control and management of type-2 diabetes mellitus (T2DM) remain controversial. Evidence from randomized clinical trials do not address many important clinical questions and are limited in their generalizability by exclusion criteria. Multiple treatment guidelines for T2DM suggest Metformin as first line medication, while the choices of second line drug remains ambiguous. A study by the Observational Health Data Science Initiative (OHDSI) found considerable variation between recommended guidelines and actual practice in T2DM. Questions pertaining to factors underlying these variations, the effectiveness of a given treatment pathway and the best second line treatment for T2DM, however remains to be addressed.

Methods

We recapitulate previous work regarding variation in the choice of first line therapy, and find clinical factors that are predictive of the first line therapy choice that are consistent with biomedical knowledge of adverse effects associated with metformin. Finally, we demonstrate the feasibility of comparative effectiveness studies of second line therapies in controlling HbA1c using matched cohorts that adjust for comorbidities that might impact the treatment outcome. DPP4-Inhibitors appears to be as effective as metformin as a first line therapy, and is considerably better than other options as a second line therapy. At present, our analysis is limited to data from Stanford Hospital, but could be extended to any site that has adopted the OHDSI common data model.

Conclusions

We recapitulate previous work regarding variation in the choice of first line therapy, and find clinical factors that are predictive of the first line therapy choice that are consistent with biomedical knowledge of adverse effects associated with metformin. Finally, we demonstrate the feasibility of comparative effectiveness studies of second line therapies in controlling HbA1c using matched cohorts that adjust for comorbidities that might impact the treatment outcome. DPP4-Inhibitors appears to be as effective as metformin as a first line therapy, and is considerably better than other options as a second line therapy. At present, our analysis is limited to data from Stanford Hospital, but could be extended to any site that has adopted the OHDSI common data model.