

the electronic journal Diabetes Care and gray literature. Several combinations of terms were used, including disease terms, interventions and type of study. The results evaluated were: glycosylated hemoglobin; weight gain; occurrence of severe hypoglycemia; total insulin dose; and, fasting capillary glycemia. Methodological quality was assessed using the Newcastle scale. The meta-analyses were performed in Review Manager® 5.2 software using a random effects model. Protocol number CRD42017054925 (International Prospective Register of Ongoing Systematic Reviews).

RESULTS:

A total of 705 publications, eight cohort studies were included. The quality of included studies was classified as high. In the meta-analysis, the results for episodes of severe hypoglycemia ($p = 0.002$), measurements of fasting capillary glycemia ($p = 0.01$), and weight gain ($p = 0.001$) were favorable for detemir. The glycosylated hemoglobin endpoint ($p = 0.49$, heterogeneity = 89 percent) revealed high heterogeneity and no statistically significant difference between groups, showing no difference between the interventions for glycemic control.

CONCLUSIONS:

Although some results are favorable to detemir, it was not possible to identify significant differences in effectiveness and safety between the two analogues evaluated, requiring new long term studies and better quality of methodological studies.

PP46 When Composite Measures Or Indices Fail: Data Processing Lessons

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INTRODUCTION:

Index mining is a new discipline that aims to search for the composite measures or indices most relevant to the contexts or outcomes. After reviewing three frailty indices and principal component (PC)-based indices, we hereby show certain occasions that can lead to ineffective indices, which consist of bias or fail to represent the theories.

METHODS:

We reproduced and reviewed the three frailty indices and the 134,689 PC (principal component) -based indices from previous publications. The impact of aggregating the input variables on the final indices was analyzed using forward stepwise regression.

RESULTS:

Several methods to combine the input variables were related to ineffective projection of information onto the indices. The most common causes leading to ineffective summation of input variables were shown in three examples involving different types of input variables, which were positively or negatively correlated or uncorrelated to the outcome. Ineffective indices were created often because of the summation of redundant information or uncorrelated variables.

CONCLUSIONS:

The creation of ineffective indices can be avoided if the relationships between input variables and outcomes are properly scrutinized. The creation of composite measures and indices is still a discipline under active development. The three examples we identified are the mistakes that may be repeated unintentionally and need to be addressed with explicit rules. A reporting guide for the creation of composite measures has been proposed. A proper review of index objectives, data characteristics, and data limitations before creating composite measures or indices is recommended.

PP47 Defining The Needs And Preferences Of Patients With Dry Eye Disease

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INTRODUCTION:

Dry eye disease (DED) is a common condition that significantly impacts patients' quality of life. Previous studies have explored the impact of DED on patients' lives qualitatively; however, patients' preference structures have not been thoroughly explored quantitatively.

METHODS:

A targeted literature review and social media listening project guided design of a discussion guide for in-depth patient interviews (n=12). These, in turn, guided construction of a quantitative questionnaire administered to moderate to severe DED patients, 40 per country in Australia, Germany, United States and United Kingdom (total n=160). Patients' preference structures were explored through an online survey using a self-explicated conjoint methodology, because of its high respondent-friendliness. Additionally, we administered the EQ5D-5L instrument to determine the health states/utilities of patients. Reaction to a hypothetical novel treatment was further obtained to check for convergent validity with the self-explicated conjoint. Finally, we asked respondents to rate the ease and relevance of the questionnaire to them.

RESULTS:

Qualitative research uncovered important patient perspectives that were built into the quantitative survey. For example, patients seek medical advice when their symptoms are not improving. Patients' lives are most affected by sensitivity to light, itchy and tired eyes and an inability to perform computer/screen work; however, of most concern/worrying to them is that their DED will get worse and they go blind. Results from the quantitative preference research will also be shared and its implications for future clinical trials in DED outlined. The results of the patient research and preference study are to be shared with health technology assessment (HTA) bodies and regulators through the early dialogue scientific advice process.

CONCLUSIONS:

A process of using qualitative research to determine what matters to patients and then quantification through respondent-friendly preference research can identify outcomes that are most patient-relevant, to inform future drug development strategies.

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PP48 Caregiver Perceptions And Experiences Of Diagnostic Genome-Wide Sequencing

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INTRODUCTION:

The objective of this qualitative analysis was to explore caregiver perceptions and experiences of receiving or not receiving a genetic diagnosis following genome-wide sequencing (GWS) in children with suspected genetic disorders.

METHODS:

One caregiver for each child completed an online survey two weeks after enrolling in the Clinical Assessment of the Utility of Sequencing and Evaluation as a Service (CAUSES) study, beginning in January 2016, and again about six months after receiving the GWS results. The survey covered the caregivers' experiences and quality of life and children's healthcare resource utilization, and provided open-ended questions for comments. The follow-up survey was completed by twenty families who had received a diagnosis with their GWS results and by twenty-two families who had not received a diagnosis. A thematic analysis of the free-text comments from both groups was performed using NVivo 11.4.2.

RESULTS:

Caregivers from both groups expressed similar experiences of negative socioeconomic effects of caregiving, particularly related to employment and time burden. Caregivers who did not receive a diagnosis with the GWS results were generally hopeful of receiving a diagnosis in the future and reported expectations of a positive benefit from receiving a diagnosis, both in terms of access to additional resources and of positive psychological effects. The absence of a diagnosis was a source of anxiety for many caregivers. By contrast, caregivers who had received a diagnosis reported positive, neutral, and negative psychological effects from the knowledge gained; no participants commented on the consequences for access to additional services or other socioeconomic effects.

CONCLUSIONS:

Our findings suggest that caregivers may have high expectations for what a diagnosis can provide to them and their families, which may not be fully met once a diagnosis is obtained. The study underpins the importance of patient-centered communication of genomic testing results so that families can set realistic expectations of what having a diagnosis will achieve.

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