PP531 Quality Of Life Of Patients With Long-Chain Fatty Acid Oxidation Disorders Before And During Treatment With Triheptanoin

Eliza Kruger (EKruger@ultragenyx.com), Deborah Marsden, Arielle Bensimon, Erin Cook, Eli Orvis, Hongbo Yang, Jill Mayhew and Nina Thomas

Introduction. Long-chain fatty acid oxidation disorders (LC-FAOD) are a group of serious diseases in which patients are at risk of metabolic decompensation, resulting in cardiomyopathy, hypoglycemia and rhabdomyolysis and premature mortality. In addition, LC-FAOD are a burdensome disease that adversely effects quality of life (QoL) via symptoms of muscle pain, fatigue, and a difficult diet. Previous studies have reported improvements in QoL during treatment with triheptanoin as measured by short form (SF) instruments. This study sought to convert the QoL measure into a utility value for a sample of patients with LC-FAOD at baseline and 78 weeks during treatment with triheptanoin.

Methods. In an open-label Phase 2 study of triheptanoin (UX007-CL201, NCT01886378), caregivers of patients (n = 9/23 enrolled) or patients aged 18+ years (n = 6/6 enrolled) completed the SF-10 or the SF-12v2, respectively. Component summary scores at baseline and 78-week during treatment period were converted to EuroQol-Five Dimension (EQ-5D) utility (with zero representing death and 1.0 perfect health) using a published conversion algorithm (Lawrence and Fleishman 2004). Generalized linear mixed-effects models with individual-level random effects were used to estimate the utility values.

Results. At baseline, patients' utility was estimated to be 0.365 (standard error [SE] = 0.090) compared with 0.629 (SE = 0.072) 78-weeks during treatment, a significant improvement ($p = 0.0073$). In a sensitivity analysis using SF-12v2 data only (that is, only adult patients), utility estimates were 0.498 (SE = 0.084) at baseline versus 0.690 (SE = 0.068) during treatment ($p = 0.0499$). No patients had a major clinical event during the SF instrument recall period, indicating the benefit was driven by day-to-day improvement in QoL.

Conclusions. Treatment with triheptanoin resulted in a substantial improvement in daily QoL for patients with LC-FAOD. Limitations of this study include that the estimation of utilities is from a single-arm study with small sample sizes and that the assessment of utility was based on a conversion algorithm rather than direct measurement. Nevertheless, results indicate significant improvement in QoL for patients treated with triheptanoin.

PP538 Burden Of Disease For Patients With Acute Myocardial Infarction Combining Dyslipidemia In China

Mengran Zhang, Hongchao Li, Aixia Ma and Pingyu Chen (chenpingyu220@163.com)

Introduction. Prevalence of dyslipidemia in Chinese adults is increasing rapidly. Dyslipidemia is one of the most important risk factors for acute myocardial infarction (AMI), which represents a serious disease burden to the country. However, there is no published research on the costs of Chinese patients diagnosed with AMI combining dyslipidemia. This study aimed to report key findings of the disease burden in China, including direct medical costs and direct non-medical costs.

Methods. Six hospitals from different geographic areas were selected in China for data collection. Patients who were hospitalized due to AMI combining dyslipidemia from January 1 2016 to December 31 2016 in the six sites were enrolled. Direct medical costs including inpatient and outpatient costs were extracted through electronic medical records; medical costs occurred in other healthcare institutions and direct non-medical costs were collected by a face-to-face questionnaire survey. Results were analyzed with descriptive statistics.

Results. Data of 900 patients were analyzed. There were more males (78.40%) than females. The mean age was 62.1 (SD: 11.5). The times of inpatient and outpatient per year were 0.57 and 8.67, respectively. Medium direct medical costs and medium direct non-medical costs were 31,440 RMB (Interquartile range (IQR): 21,533–48,202) (4,443 USD: 3043–6812) and 665 RMB (IQR: 351–1328) (94 USD: 50–188), respectively; while corresponding medium indirect costs per year were 659 RMB (IQR: 226–1579) (93 USD: 32–223).

Conclusions. This is the first study comprehensively analyzing the disease burden of patients diagnosed with AMI combining dyslipidemia in China. The results suggested that the medical cost of this population is still high. Hospitalization cost accounted for 81 percent of the total cost, which was around 1.3 times of the annual per capita disposable income over the same period. Therefore, the importance of providing effective clinical management as well as dyslipidemia prevention and control intervention should be highlighted, especially for middle-aged and elderly males with dyslipidemia.

PP548 The Design Of Long-term Care Insurance Payment: Base On Pilot Practice In Jingmen

Tiantian Du, Junting Yang, Ying Li, Meng Zhang and Yuehua Liu (liu_yuehua@163.com)

Introduction. With the aging of population, miniaturization of family size and changes of diseases spectrum, the demand for long-term care of Chinese elderly is increasing, which is challenging the existing long-term care system. China is currently carrying out pilot work for a long-term care insurance system, and Jingmen is one of the pilot cities, however more detailed research on payment is needed. Therefore, this paper draws on case-mixed-adjusted prospective payment system to provide designs for long-term care insurance in pilot cities.

https://doi.org/10.1017/S0266462320001981 Published online by Cambridge University Press