

AB001. Methylmalonic and propionic acidemias: comparative outcomes between liver transplantation versus non-liver transplantation groups

Tzu-Hung Chu, Dau-Ming Niu

Department of Pediatric Medicine, Taipei Veterans General Hospital, Taipei, Taiwan

Background: Most patients with methylmalonic acidemia (MMA)/propionic acidemia (PA) present with acute metabolic distress and encephalopathy in neonatal period, then either die immediately or survive with significant neurodevelopmental disability. The comparative outcomes regarding MMA and PA have been rarely reported in Asia.

Methods: The nationwide newborn screening survey for MMA/PA cases has been in place in Taiwan from January 2000 and data was collected until December 2016. The data including demographic data, initial presentation, and a comparison of prognosis between patients with and without liver transplantation (LT) was carried out. Clinical outcomes were analyzed.

Results: A total of 3,014,192 newborns were screened. Overall incidence of MMA mutase type was 1/130,244,

while that for cobalamin defect was 1/683,781 and that for PA was 1/683,781. The MMA mutase type patients generally have higher AST, ALT, and NH₃ values as well as a lower pH value compared to patients with the cobalamin type defect and PA ($P < 0.05$). Seventeen out of 25 MMA mutase type patients received a LT; while 2 out of 4 PA patients received a LT. Mean admission length shortened from 90.6 days/year (pre-LT) to 5.3 days/year (at 3rd year post-LT) ($P < 0.0005$). Similarly, the tube feeding ratio decreased from 67.8% to 0.50% over the same time period ($P < 0.00005$). Furthermore, the anxiety level of the caregiver was reduced from 33.4 to 27.2 after LT ($P = 0.001$) and the DQ/IQ performance of the patients was improved after LT from 50 to 60.1, even though this latter change was not statistically significant.

Conclusions: LT patients do survive and have reduced admission time, reduced tube feeding and the caregiver is less anxious. However, further research is necessary to determine what are the long-term benefits and risks associated with LT among patients with MMA/PA.

Keywords: Methylmalonic acidemia (MMA); propionic acidemia (PA); liver transplantation (LT); outcome

doi: 10.21037/atm.2017.s001

Cite this abstract as: Chu TH, Niu DM. Methylmalonic and propionic acidemias comparative outcomes between liver transplantation versus non-liver transplantation groups. *Ann Transl Med* 2017;5(Suppl 2):AB001. doi 10.21037/atm.2017.s001