Extended Abstract | Anesthesiology and Clinical Science Research | 2021 | Vol.5 No.1

Health economics and patient outcomes of non-myeloablative hematopoietic stem cell transplantation (HSCT) versus disease-modifying therap (DMT) for relapsing remitting multiple sclerosis in the United States of America- Richard K Burt - Northwestern University

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Introduction:
To estimate differences in treatment costs and health outcomes between non-myeloablative hematopoietic stem cell transplantation (HSCT) and disease-modifying therapies (DMTs) for the treatment of relapsing-remitting multiple sclerosis (RRMS). Importance Hematopoietic stem cell transplantation (HSCT) represents a potentially useful approach to slow or prevent progressive disability in relapsing-remitting multiple sclerosis (MS). Objective To compare the effect of nonmyeloablative HSCT vs disease-modifying therapy (DMT) on disease progression. Hematopoietic stem cell transplantation (HSCT) represents a potentially useful approach to slow or prevent progressive disability in relapsing-remitting multiple sclerosis (MS). Objective: To compare the effect of nonmyeloablative HSCT vs disease-modifying therapy (DMT) on disease progression. To estimate differences in treatment costs and health outcomes between non-myeloablative hematopoietic stem cell transplantation (HSCT) and disease-modifying therapies (DMTs) for the treatment of relapsing-remitting multiple sclerosis (RRMS). Methods : We collected data on costs and reimbursements for patients who underwent HSCT for RRMS at Northwestern Memorial Hospital in Chicago (USA) between January 2017 and January 2019. The costs of HSCT were compared against those for DMTs in the United States, obtained from the literature. We also conducted a literature review to interpret the cost comparisons in terms of disease control and patients' wellbeing defined as no evidence of disease activity (NEDA), neurologic disability by the Expanded Disability Status Scale (EDSS), and quality of life by the short form SF-36, respectively.We collected data on costs and reimbursements for patients who underwent HSCT for RRMS at Northwestern Memorial Hospital in Chicago (USA) between January 2017 and January 2019. We collected data on costs and reimbursements for patients who underwent HSCT for RRMS at Northwestern Memorial Hospital in Chicago (USA) between January 2017 and January 2019. The costs of HSCT were compared against those for DMTs in the United States, obtained from the literature. We also conducted a literature review to interpret the cost comparisons in terms of disease control and patients' wellbeing defined as no evidence of disease activity (NEDA), neurologic disability by the Expanded Disability Status Scale (EDSS), and quality of life by the short form SF-36, respectively. To estimate differences in treatment costs and health outcomes between non-myeloablative hematopoietic stem cell transplantation (HSCT) and disease-modifying therapies (DMTs) for the treatment of relapsing-remitting multiple sclerosis (RRMS). Autologous hematopoietic stem cell transplantation (aHSCT) is one of the therapies used in the treatment of multiple sclerosis (MS). In relapsing-remitting MS (RRMS), disease modifying therapies have been effective in reducing disease activity; however, long-term disability is still a major issue for patients. This is even more troublesome for patients with aggressive, progressive MS, for whom irreversible disability is accelerated. Follow-up has been done in the short-term, showing that aHSCT is an effective treatment both for RRMS and aggressive MS. Burt et al. did a randomized clinical trial comparing the effect of nonmyeloablative HSCT with disease modifying therapies in patients with RRMS and found that the HSCT resulted in prolonged time to disease

progression.By using high-dose immunosuppressive therapy, which suppresses autoimmune inflammation, followed by infusing autologous hematopoietic stem cells, the immune system can be reset or reconstituted. Success for this in progressive forms of MS was demonstrated in other studies, such as the retrospective observational study done by Muraro et al. However, it is not known whether aHSCT can induce long-term drug-free remission past 5 years The costs of HSCT were compared against those for DMTs in the United States, obtained from the literature. We also conducted a literature review to interpret the cost comparisons in terms of disease control and patients' wellbeing defined as no evidence of disease activity (NEDA), neurologic disability by the Expanded Disability Status Scale (EDSS), and quality of life by the short form SF-36, respectively. HSCT mean total costs were $85,184 (range $70,635 to $120,260). Mean revenue collected was $95,268 (range $16,544 to $173,204). In comparison, according to the literature, 2019 DMT costs in the USA ranged from $80,000 to $100,000 per year per patient. After one year, HSCT becomes a significant cost savings (80,000 to 100,000 USD per year) for the health care system. Compared to DMTs, studies of HSCT reported greater improvement in no evidence of disease activity (NEDA) (figure 1 below), disability, and quality of life. Limitations of this analysis is that costs of HSCT would be expected to vary by conditioning regimen (e.g. non-myeloablative versus myeloablative) utilized, patient selection, center experience, and regional variation. Randomized trials for cost comparisons are missing and variations in HSCT designs, populations, and methodology preclude more precise cost estimates. In conclusion, the costs of non-myeloablative HSCT after which DMTs are indefinitely discontinued, are approximately the same cost as those for one year of prescription DMTs. Since DMTs assessed in this analysis are given on an ongoing basis, whilst HSCT is not, HSCT is expected to produce long-term cost-savings. When considered alongside the available clinical evidence, which suggests that HSCT generates