Background:

- Duchenne Muscular Dystrophy (DMD) is a rare, X-linked, rapidly progressive, and severely disabling muscle-wasting disease that occurs in approximately 1 in 3,500 male births.

- Without treatment, the mean expectancy for DMD patients is around 19 years.

- Despite corticosteroids being part of the standard of care for DMD, up to 54% of DMD patients are not currently receiving corticosteroid therapy.

- Emflaza® (deflazacort) is the first and only FDA-approved corticosteroid treatment for DMD in the United States.

- Emflaza® is based on natural history studies and a meta-analysis of plasticity (corticosteroid) arms of 2 independent phase 3 clinical trials in DMD indicate greater benefits in slowing disease progression with deflazacort vs prednisone.

Methods:

- An Excel-based budget impact model was developed to estimate the costs associated with deflazacort for the treatment of DMD patients from a managed care decision maker's perspective.

- The model compares deflazacort with no therapy.

- The model is based on previously published evidence from natural history studies and a meta-analysis of plasticity (corticosteroid) arms of 2 independent phase 3 clinical trials in DMD.

- Costs inflated to 2017 dollars.

- The model evaluates a 3-year time horizon, which does not capture the long-term benefits of deflazacort treatment for boys with DMD.

- The model utilizes drug acquisition costs, treatment costs, treatment rates, and prevalence numbers based on published sources; these values may not reflect the actual amount paid by health plans.

- Indirect costs associated with caregiver burden or family loss of income are not reflected in this model.

Results:

- For a health plan with 10,000 members, 16 patients aged 5 to 24 years are estimated to be affected by DMD.

- Due to reduced costs resulting from delayed DMD milestones including spinal surgery, loss of ambulation, nocturnal ventilation, and cardiomyopathy, the total annual cost per patient treated with deflazacort using real dosing is estimated to be approximately $10,000 less than patients who are not treated.

- Based on an estimated deflazacort market uptake of 10% in Year 1, 15% in Year 2, and 20% in Year 3, the budget impact of deflazacort results in savings of $81,931 in Year 1, $92,091 in Year 2, and $258,514 in Year 3 for a total savings of $751,536 (Figure 2).

- When factoring in the Medicaid rebate for a Medicaid population, the average cost per patient for deflazacort decreased further, resulting in additional budget savings.

- The per-member-per-month (PMPM) impact of deflazacort was $89,000 in Year 1, $89,000 in Year 2, and $89,000 in Year 3 for a total PMPM impact of $89,000.

- When factoring in the Medicaid rebate for a Medicaid population, the average cost per patient for deflazacort decreased further, resulting in additional budget savings.

- The PMPM impact from the Medicaid perspective was $89,000 in Year 1, $89,000 in Year 2, and $89,000 in Year 3 for a total PMPM impact of $89,000.

Limitations:

- The model did not include an active comparator as there are no other FDA-approved corticosteroid agents indicated for the treatment of DMD.

- The model evaluates a 3-year time horizon, which does not capture the long-term benefits of deflazacort treatment.

- The model utilizes drug acquisition costs, treatment costs, treatment rates, and prevalence numbers based on published sources; these values may not reflect the actual amount paid by health plans.

- Indirect costs associated with caregiver burden or family loss of income are not reflected in this model.

Conclusions:

- Treatment with deflazacort is a less costly alternative to no treatment in patients with DMD due to reduced costs associated with spinal surgery, loss of ambulation, nocturnal ventilation, and cardiomyopathy.

- From a US health plan’s perspective, the introduction of deflazacort for the treatment of DMD results in moderate budget impact savings.

References:


