A PROBABILISTIC BUDGET IMPACT ANALYSIS OF CYSTIC FIBROSIS TREATMENT OPTIONS: A U.S. PHARMACY BUDGETS

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OBJECTIVES: Cystic fibrosis (CF) is one of 7,000 rare diseases as defined by the Food and Drug Administration. Since the Orphan Drug Act of 1983, CF has evolved into a successfully treated chronic disease through the development of new therapies. The purpose of this analysis was to estimate the potential budget impact of a novel CF therapy, ivacaftor, on hypothetical managed care plans of varying size. METHODS: A literature search was conducted in MEDLINE to identify the current prevalence and adherence estimates of G551D mutation CF patients treated with ivacaftor. Additionally, Medi-Span drug knowledge data was used to estimate wholesale acquisition cost for ivacaftor. A probabilistic Monte-Carlo simulation model was constructed in Microsoft Excel using literature supported CF-related health outcomes (i.e., CF Foundation Registry, African-American CF patient data). The model predicted monthly drug costs per patient based on adherence estimates. The model evaluated iterations of three hypothetical managed care plans made up of 500,000 (plan A); 2,000,000 (plan B); and 4,000,000 (plan C) patient lives and captured annual projected ivacaftor patients and treatment expenditures. Simulations were conducted a 1000 times to derive confidence intervals under each scenario. RESULTS: The model estimated that for health Plan A there would be approximately 1.67 treated patients (95% confidence interval [CI]: 1.55-1.80) with annual expenditures of $334,476 (95% CI: $306,817-$362,136). Plan B treated 6.52 patients (95% CI: 6.05-6.99) after annual expenditures of $1,270,713 (95% CI: 1,169,781-1,371,645). Plan C treated 13.37 patients (95% CI: 12.9-13.46) with annual expenditures of $2,631,001 (95% CI: $2,419,785-$2,842,217). The percent increase in pharmacy spending due to this new orphan drug was estimated to be $0.05. CONCLUSIONS: New therapies for rare conditions, such as ivacaftor, can have a substantial impact on the overall budget of a health plan even though a small number of patients are treated. The high annual cost of ivacaftor translates to a sizeable increase in the pharmacy PMPM.

A SIMULATION TO PREDICT REDUCTIONS IN LIFETIME MEDICAL EXPENDITURES AFTER OBESITY ADOLESCENTS UNDERGO ROUX-EN-Y GASTRIC BYPASS SURGERY

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OBJECTIVES: One third of children and adolescents in the United States are overweight and over 18% are obese. Childhood obesity has been associated with increased morbidity in childhood and frequent tracking into adulthood. Weight-loss surgery has recently become an accepted treatment for severe adolescent obesity. We have constructed a microsimulation to quantify potential savings in lifetime (age 18 to 80) medical costs after obese adolescents undergo Roux-en-Y gastric bypass surgery. METHODS: Published literature and data from the 2004–2010 rounds of the Medical Expenditure Panel Survey (MEPS) were used to inform health state costs and transition probabilities. Surgical complication rates are included and the costs of these complications are estimated using Medicare reimbursement rates and the AHRQ HCUP National Inpatient Sample Query Tool. RESULTS: The costs of complications ranged from $120 to $16,900, but the complications occurred only in 0.2% to 1.3% of patients. Without the cost of the initial procedure, we find the present value (discounted 3% annually) of reductions in lifetime medical expenditures for an 18-year-old male with a BMI of ≥40 to be between $19,535 and $13,073, if his post-operative BMI is between 25 and 30, respectively. The present value of these savings for females are between $20,446 and $13,851. All of these savings are statistically greater than zero (P < 0.01). However, because the prevalence of extremely high BMI is rare, the costs assigned to individuals with BMI ≥40 are more representative of individuals with a BMI near 40. Savings from the treatment of more extreme obesity are likely greater. CONCLUSIONS: Given the cost of Roux-en-Y gastric bypass surgery for adolescents is approximately $25,000 to $30,000 and the potential $20,000 to $30,000 in future medical costs saved, this type of surgery is likely not cost-saving for the majority of obese adolescents. However, the procedure may still be cost-effective, depending on quality-of-life improvements.

PAYER MANAGEMENT AND PAYING DYNAMICS FOR NON-ONCOLOGY PHARMACY BENEFIT ORPHAN DRUGS FOR RARE DISEASES IN THE UNITED STATES LAUNCHING a DIGITAL TOOL TO HELP Payers Identify and Manage Orphan Drugs

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OBJECTIVES: In the U.S., orphan drugs for rare diseases have historically been able to achieve favorable price and access on formulas of Managed Care Organization (MCO) due to the severity of the conditions and their minimal budget impact. However, with the advent of new orphan drugs, payers are likely to look for ways to reduce or contain the cost. This is particularly true among pharmacy benefit products where payers have more effective utilization management tools at their disposal when compared with non-oncology medical benefits. METHODS: The Wholesale Acquisition Cost (WAC) of all non-oral FDA designated pharmacy benefit orphan drugs that were launched 2004-2014 and plotted these against disease prevalence. The most common type of management introduced was either a ‘preferred product’ or ‘step edit’. Integrated plans introduced stronger management more frequently when compared to national and regional plans. RESULTS: Given the increased payer spend in the orphan drug category, payers have begun to manage products in areas where cost savings can be achieved without sacrificing outcomes, irrespective of rare disease prevalence. In disease areas where multiple drug options exist, regardless of the presence or type of management, integrated plans were only required a confirmation of a disease diagnosis through a Prior Authorization. While integrated health systems tend to have the tightest control on orphan products given their close physician relationships, national and regional plans have also begun to implement a similar level of control for select products.

ECONOMIC BURDEN OF PSORIATIC PATIENTS IN THE BRAZILIAN HEALTH SYSTEM

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OBJECTIVES: To estimate the economic burden of psoriatic patients with varying levels of psoriasis severity in the Brazilian Private Health System. METHODS: The Psoriasis Severity Index (PSI) was validated in the Brazilian Private Health System (NHIS), a national representative sample of insured individuals in the country (N=12,000), a nationally representative health survey conducted among adults in Brazil, was used as the data source in this study. All respondents who reported a diagnosis of psoriasis severity were included in the analyses (n=210). Economic outcomes (work productivity, presenteeism, provider visits and hospitalization) across levels of severity, exception was the rate of obesity which increased from 25.5% to 38.1% to 54.5% for mild, moderate, and severe, respectively, p<0.05. Comparing the total cost (absenteeism, presenteeism, provider visits and hospitalization) across levels of severity, the cost of severe psoriasis was BRL 224,251, six times the cost of those with mild severity (BRL 34,508) and over twice the cost of those with moderate severity (BRL 79,427). CONCLUSIONS: The disease cost is proportional to the level of psoriasis severity in the Brazilian Private Health System, suggesting improved disease management (e.g., improved access to treatments, earlier diagnosis, etc.) may alleviate substantial societal costs.