## Case 4 Drug Efficacy – A Real-World Perspective

Introduction and Background:

**LifeMed Research**, a globally renowned pharmaceutical company, prides itself on its dedication to innovation and improving patient outcomes. At the helm of LifeMed Research are Dr. Eleanor Hayes, the visionary CEO, and Mr. Daniel Carter, the seasoned Chief Medical Officer (CMO), known for his strategic acumen and commitment to evidence-based decision-making.

LifeMed has been a pioneer in the development of asthma treatments, providing critical solutions to millions of patients worldwide. However, recent discussions within the company have highlighted a pressing challenge: the need for robust real-world evidence (RWE) to support drug efficacy beyond clinical trials.

Dr. Hayes and Mr. Carter recognize that while randomized clinical trials (RCTs) are the gold standard for evaluating drug efficacy, they often fail to capture the complexities and variations seen in real-world patient populations. In clinical practice, patients are not assigned to treatment groups randomly, as in RCTs. Instead, treatment decisions are influenced by factors such as patient preferences, prior conditions, and physician recommendations. This non-randomized assignment introduces the risk of selection bias, making it challenging to compare the outcomes of different treatments accurately.

One of LifeMed’s flagship asthma treatments, **Drug\_D**, has been widely prescribed, but it is now facing competition from a new alternative, **Drug\_S**. To better understand the efficacy of these drugs in real-world settings, LifeMed has launched a groundbreaking study. This initiative is spearheaded by **Dr. Olivia Bennett**, the company's Chief Data Scientist, who brings a wealth of expertise in advanced analytics and causal inference.

In their first meeting to discuss this study, Dr. Hayes set the tone: *"Olivia, the data we collect from this study is critical. It must reflect real-world usage and account for the complexities of patient selection. We need to provide the medical community with unbiased, actionable insights into the efficacy of Drug\_D and Drug\_S."*

Dr. Bennett nodded, listening intently as Mr. Carter added: *"We’re looking at patients who were newly initiated on either Drug\_D or Drug\_S after having used other asthma treatments previously. The goal is to determine which drug reduces the number of asthma exacerbations in the first year of use. However, given the non-randomized nature of the data, we can’t assume zero selection bias. That’s where your expertise will be crucial."*

Dr. Bennett replied confidently: *"I understand. We’ll employ advanced statistical techniques to adjust for potential biases and ensure that our comparisons are as fair and accurate as possible."*

Dr. Bennett and her team began by assembling a real-world dataset of asthma patients who initiated treatment with either Drug\_D or Drug\_S. The dataset was rich with pre-treatment and post-treatment information, allowing for a comprehensive analysis of patient characteristics and outcomes.

The Challenge

**Objective**:  
To evaluate the real-world efficacy of Drug\_D and Drug\_S by analyzing patient outcomes while accounting for selection bias. The goal is to provide unbiased, actionable insights into which drug is more effective in reducing asthma exacerbations in the first year of treatment.

**Scope**:

* Address the challenges of non-randomized treatment assignment.
* Analyze key pre-treatment variables to adjust for selection bias.
* Compare the post-treatment outcomes of Drug\_D and Drug\_S fairly and accurately.

**Expected Output**:

* A detailed comparison of the efficacy of Drug\_D and Drug\_S, adjusted for confounding variables.

As LifeMed Research moves forward with this study, the company remains steadfast in its mission to improve patient outcomes through innovation and evidence-based medicine.

**Assumptions may be made as necessary to solve the problem and should be stated clearly.**

Data Set Variables:

The variables are divided into the following groups:

1. identity: patient\_id
2. pre-index demographics and comorbidities: index\_age, female, acute\_bronchitis, acute\_laryngitis, gerd, pneumonia, rhinitis, sinusitis
3. pre-index usage: previous\_asthma\_drugs, pre\_asthma\_charg, pre\_asthma\_days, pre\_asthma\_pharma\_charge, total\_pre\_charge, log\_charges=log(total\_pre\_index\_charge), log\_asthma\_charge=log(pre\_asthma\_pharma\_charge)
4. drug identifier: drug\_s=1 or drug\_s=0
5. post-index measurement (this is the outcome of interest.): post\_index\_exacerbations\_365

Here are our variables of interest:

In general, pre-index refers to activity or characteristics of the patient that existed before they were assigned to one of the drug groups. Post-index refers to measurements of activity after the drug assignment. In this case the post-index measurement is the number of exacerbations in the first year after starting the study.

* acute\_bronchitis: Indicator (0 or 1) if the patient had a diagnosis in the previous year.
* acute\_laryngitis: Indicator (0 or 1) if the patient had a diagnosis in the previous year.
* female: Indicator (0 or 1).
* gerd: Indicator (0 or 1) if the patient had a diagnosis in the previous year.
* pneumonia: Indicator (0 or 1) if the patient had a diagnosis in the previous year.
* rhinitis: Indicator (0 or 1) if the patient had a diagnosis in the previous year.
* sinusitis: Indicator (0 or 1) if the patient had a diagnosis in the previous year.
* total\_pre\_index\_cannisters\_365: Note that this is a truncated distribution due to the patient selection criteria.
* upper\_respiratory\_infection: Indicator (0 or 1) if the patient had a diagnosis in the previous year.
* index\_age: Patient’s age in years on day on which treatment started.
* pre\_asthma\_charge: Total asthma non-pharmaceutical related charges in 6 months before index date.
* pre\_asthma\_days: Total days with asthma treatment in 1 year before index date.
* pre\_asthma\_pharma\_charge: Total asthma-related pharma charges in 1 year before index date
* total\_pre\_index\_cannisters\_365: Total SABA (Short Acting Beta Agonist) canisters in 1 year before index date. SABA is given to provide fast relief from asthma. Greater use is an indicator of more severe asthma.
* total\_pre\_index\_charge: Total charges in 1 year before index date.
* log\_charges: Log of total\_pre\_index\_charge.
* log\_asthma\_charge: Log of pre\_asthma\_charge.
* post\_index\_exacerbations365: Outcome of interest. Exacerbations in the year after initiating therapy.

The case should cover the following area with project code/ workflow, PDF document and short PPT for final presentation.

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| **Sr. No** | **Method** |
| 1 | Identification of Business Problem |
| 2 | Data Preparation and Availability |
| 3 | Proposed Approach |
| 4 | Data Analysis |
| 5 | Conclusion and Findings |