Use Case

Using RWD/E for Label Expansion of a Diabetes Medication using Observational Cohort Study

This study explores the use of real-world data and evidence (RWD/E) from observational cohort studies to support label expansion for a diabetes medication. The study design utilizes real-world data to assess the medication's effectiveness in a broader patient population, potentially leading to a more detailed understanding of its benefits. This approach offers advantages in terms of cost-efficiency and the findings can be applied to a larger group of people, unlike traditional randomized controlled trials.

Section	Details
Use Case Scenario	A pharmaceutical company (ABC) developed and marketed Liraglutide, a glucagon-like peptide-1 (GLP-1) receptor agonist, for type 2 diabetes (T2D) patients. Liraglutide's current label restricts its use to patients with a body mass index (BMI) greater than 25. ABC suspects Liraglutide might also benefit patients with a lower BMI and plans to expand the label. However, conducting a large, randomized controlled trial (RCT) for this specific population could be expensive and time-consuming.
Objective	Utilize real-world data and evidence (RWD/E) to support a label revision for Liraglutide, expanding its indication to include T2D patients with a BMI below 25.
RWD/E Data Sources	• Electronic health records (EHRs) from integrated healthcare delivery networks • National claims databases • Disease registries specific to diabetes

Target Population	• Adults diagnosed with T2D based on ICD-10 codes
	• Stratified by two BMI groups:
	  ○ Group 1: BMI ≥ 25 (existing labeled indication)
	  ○ Group 2: BMI < 25 (proposed new indication)
	• Propensity Score Matching (PSM) will be employed to identify patients in Group 2 with similar baseline characteristics (age, comorbidities, prior medications) to those in Group 1.

MCS' Analytical Approach	**1. Feasibility Analysis:** ○ Explore and identify the most relevant data set for the target patient population (TPP). ○ Conduct feasibility analysis to ensure that longitudinal linked anonymized data for the TPP is available from laboratory reports, electronic medical records, claims records, and pharmacy records. ○ Ensure that the study will be powered adequately to prove statistical significance for primary and secondary objectives. **2. Data Extraction and Preprocessing:**

Section	Details
	○ Extract relevant data points including demographics, lab values (HbA1c, fasting blood sugar), medication history, and adverse events. ○ Implement stringent data quality checks to ensure data completeness and accuracy. 3. **Cohort Selection:** ○ Identify patients with a confirmed T2D diagnosis. ○ Apply exclusion criteria (e.g., pre-existing cardiovascular disease, Type 1 diabetes). ○ Utilize PSM to create a balanced comparison group (BMI < 25) that closely resembles the existing labeled population (BMI ≥ 25). 4. **Outcome Measures:** ○ **Primary:** Glycemic control as measured by HbA1c reduction at 6 months and 12 months. ○ **Secondary:** Change in body weight, incidence of hypoglycemia, and any documented adverse events associated with Liraglutide use. 5. **Statistical Analysis:** ○ Utilize Propensity Score Weighted (PSW) analysis to account for potential confounding variables and estimate the causal effect of Liraglutide on glycemic control in the lower BMI group. ○ Conduct sensitivity analyses to assess the robustness of findings under different PSM assumptions. 6. **Real-World Context Analysis:** ○ Analyze treatment patterns and medication adherence for Liraglutide compared to other diabetes medications in the real-world setting. ○ Evaluate potential generalizability of the findings to broader T2D patient populations.
Expected Outcomes	• The RWD/E study aims to demonstrate that Liraglutide is safe and effective in improving glycemic control for T2D patients with a BMI below 25. • The findings will be presented in a comprehensive report with clear and interpretable results, including limitations associated with using observational data.
Regulatory Considerations	• The RWD/E study design and analysis will be conducted adhering to established guidelines for using RWE in regulatory submissions (e.g., FDA draft guidance on Use of Real-World Evidence to Support Regulatory Decisions). • The report will address potential biases and limitations inherent to observational studies and propose strategies to mitigate them.
Benefits of Using RWD/E	• Offers a faster and more cost-effective approach compared to a traditional RCT to support label expansion. • Leverages real-world data that reflects actual clinical practice and patient populations, potentially enhancing the generalizability of findings.

Section	Details
	• Provides valuable insights into treatment patterns and medication adherence in the real world. • Provides RWD based data inputs for pharmaco-economic models and value dossier content.
Additional Considerations	• While the use of RWD/E-derived synthetic control arms is gaining acceptance, regulatory agencies continue to refine their guidance and expectations. MCS can help sponsors navigate this evolving regulatory landscape and ensure compliance with the latest standards. • Data quality and representativeness are crucial for the validity and reliability of RWD-based synthetic control arms. MCS has robust data quality management processes and statistical expertise to ensure the RWD used is fit for purpose and appropriately addresses potential biases and confounding factors.

Conclusion

This use case showcases how ABC pharma company can leverage RWD/E to generate robust evidence supporting a label revision for Liraglutide. By employing a rigorous analytical approach and adhering to regulatory guidelines, ABC can potentially expand patient access to this effective diabetes medication. Maxis Clinical Sciences offers the expertise and resources to guide pharmaceutical companies like ABC through the entire RWD/E process for label revisions, ensuring a successful outcome.

If you have any questions or would like to learn more about our analytical approach and how we can support your specific needs, please don't hesitate to connect with us. We'd be delighted to discuss our services in further detail and explore how we can collaborate to drive innovation in clinical research and healthcare decision-making.

Contact us at info@maxisclinical.com or via our website contact form – Make an appointment.