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PART OF REAL CHEMISTRY

X4 Pharmaceuticals

INTRODUCTION

Specialty and rare diseases have undefined patient populations who are undiagnosed or misdiagnosed, healthcare providers who are unaware of disease states and their manifestations, as well as treatment journeys that are not well-understood. IPM.ai transforms real world data into real world insights that uncover the ideal patient, their treatment journey and their healthcare ecosystem so that life sciences companies can accelerate the successful development and commercialization of life-savings therapies for specialty and rare diseases that lead to optimal patient outcomes quicker, with less risk.

THE CONDITION

WHIM Syndrome is a rare immuno-deficiency caused by gain-of-functions mutations in the C-X-C chemokine receptor type 4 (CXCR4) gene; this hyperactivity causes bone marrow to fill with degenerating white blood cells. The condition is characterized by: **W**arts driven by underlying HPV infections that can lead to Cancer; **H**ypogammaglobulinemia where low levels of immunoglobulin result in low antibody production; **I**nfections that are chronic and can lead to devastating, irreversible morbidities; and **M**yelokathexis, a "hyper-dense" population of pre-apoptotic immune cells in the bone marrow that reduces the body's ability to achieve a healthy immune response. As a rare disorder, the prevalence of WHIM is unknown because no ICD-10 code for diagnosis exists, genetic testing is not routine, physicians lack awareness and training to diagnose, and clinical presentations make it challenging to differentiate from Severe Congenital Neutropenia (SCN) or Primary Immunodeficiency (PID).

THE CHALLENGE

With only 100 documented WHIM Syndrome cases since 1964, X4 Pharmaceuticals set out to address the ambiguity of the patient universe by conducting a market study via quantitative patient surveys, engaging in telephone interviews with health care providers, and determining prevalence calculations to identify 1,000 to 1,300 WHIM patients in the United States. While in Phase II development of Mavorixafor, a first-in-class treatment for WHIM Syndrome, X4 partnered with IPM.ai to further understand the disease prevalence, define market opportunities, identify health care providers treating patients and guide internal forecasting.

THE SOLUTION

IPM.ai built an ideal patient profile via propensity scoring of the four primary conditions and their manifestations to signal a WHIM Syndrome diagnosis. Machine learning and artificial intelligence -- in conjunction with a real world data universe of over 300 million de-identified patients -- was utilized to discover look-alike patients. Clinical criteria was then layered onto the model outputs to refine estimates into book-end scenarios and predict market size. Further patient-level analysis revealed characteristic symptomatology and the patient treatment journey, which was then used to confirm targeted health care specialists.

THE OUTCOME

IPM.ai projected the existence of ~800 to 2,400 additional undiagnosed WHIM Syndrome patients in the United States, which, along with a summary of symptoms, treatments, and healthcare utilization rates, was incorporated into a market assessment and eventual financial analyst presentations. Medical Science Liaisons were then deployed to engage target health care providers treating potential WHIM Syndrome patients to increase disease awareness and provide education.

About IPM.ai

IPM.ai, part of Real Chemistry, is an Insights as a Service (IaaS) provider that empowers the world's leading life sciences companies to better understand and improve the lives of patients through the development and commercialization of precision medicine for specialty and rare diseases. IPM's system of insight optimizes drug development, clinical study, product launch and commercial operations through granular-level longitudinal analytics, artificial intelligence and machine learning in conjunction with a real world data universe of over 300 million de-identified patients and 65 billion anonymized social determinants of health signals.