Utilizing Augmented Intelligence to Drive Optimal Decisions Throughout the Specialty and Rare Disease Product Lifecycle

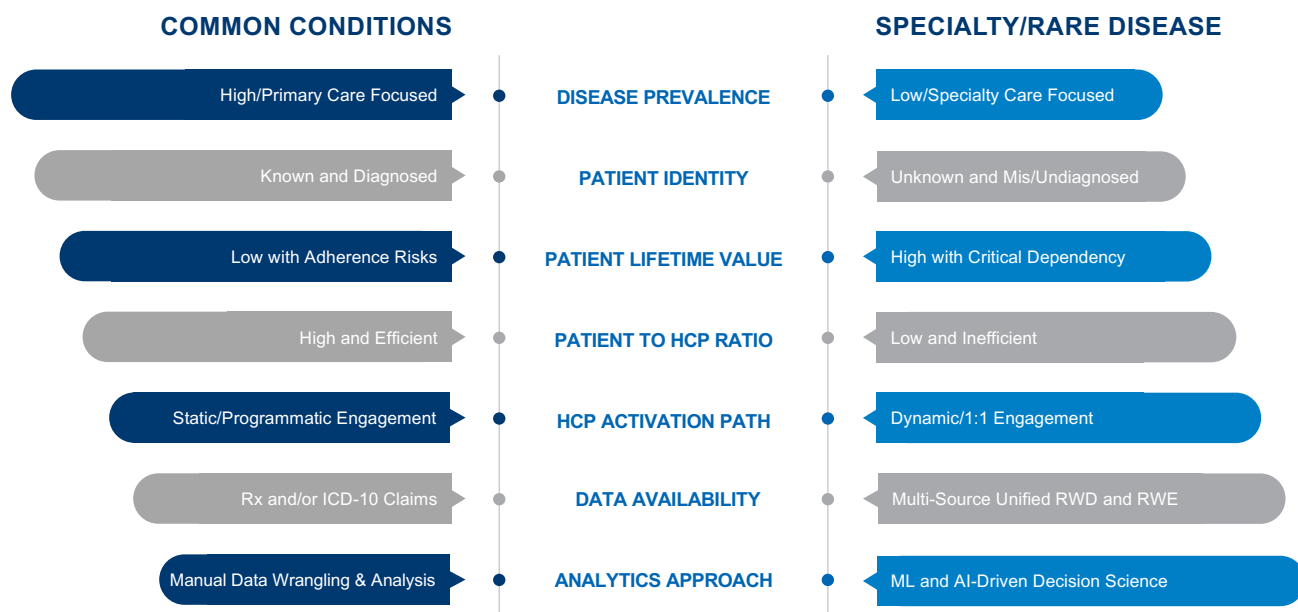## THE DYNAMICS OF THE SPECIALTY AND RARE DISEASE MARKET

Limited Patient Populations with High Unmet Needs

Specialty and rare diseases are characterized by undefined patient populations who are undiagnosed or misdiagnosed, healthcare providers who are unaware of disease states and their manifestations, as well as diagnostic and treatment journeys that are not well-understood. As the U.S. medical system is organized around specialists who treat "the organ," most physicians, even specialists, will never encounter these conditions in the ordinary course of care and symptoms may be misdiagnosed as common illnesses (albeit with the best of intentions). Consequently, patients often embark on a diagnostic odyssey, going from doctor to doctor until they are finally (hopefully!) referred to a specialist familiar with the disease. However, 95% of these conditions – many of which are chronic, debilitating and even fatal – have no pharmacological treatment options, exacerbating the misdiagnosis trajectory, and resulting in significant healthcare spend that does not improve patient outcomes. And for the small number of cases where treatments do exist, they are often complex, costly and cause significant side effects.
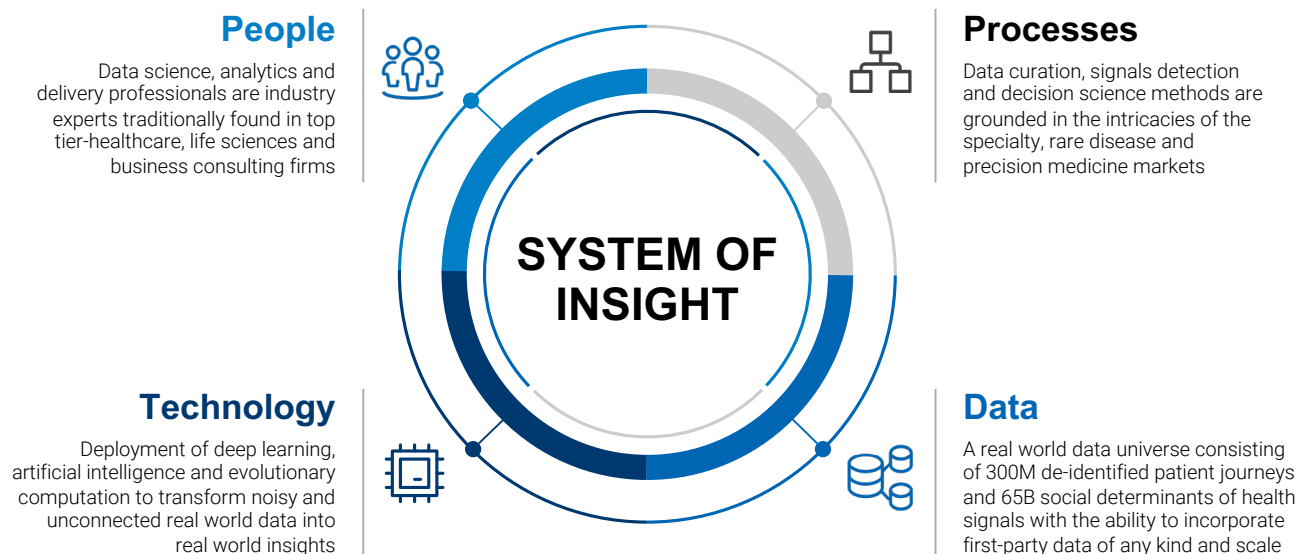
> 95% of specialty and rare conditions have no pharmacological treatment options, resulting in significant healthcare spend while not improving patient outcomes.

For those healthcare and life sciences pioneers venturing into the realm of developing and commercializing life-improving therapies for specialty and rare conditions, the typical approach – frequently adapted from the commercialization processes of therapies targeting common illnesses and grounded in data analytics – is falling short due to the profound differences in the conditions themselves, the patient profile and the health care provider ecosystem, as well as the data streams and the method of analysis.

COMMON CONDITIONS		SPECIALTY/RARE DISEASE
High/Primary Care Focused	DISEASE PREVALENCE	Low/Specialty Care Focused
Known and Diagnosed	PATIENT IDENTITY	Unknown and Mis/Undiagnosed
Low with Adherence Risks	PATIENT LIFETIME VALUE	High with Critical Dependency
High and Efficient	PATIENT TO HCP RATIO	Low and Inefficient
Static/Programmatic Engagement	HCP ACTIVATION PATH	Dynamic/1:1 Engagement
Rx and/or ICD-10 Claims	DATA AVAILABILITY	Multi-Source Unified RWD and RWE
Manual Data Wrangling & Analysis	ANALYTICS APPROACH	ML and AI-Driven Decision Science

Enter Augmented Intelligence – a concept that utilizes Machine Learning and Artificial Intelligence in conjunction with a curated pool of "smart, real-world data" to enhance, rather than replace, human decision-making. With Augmented Intelligence, healthcare and life sciences companies can transform real world data into real world

A System of Insight can, in most cases, increase a patient pool for a new line of therapy by 40x or more.

People

Data science, analytics and delivery professionals are industry experts traditionally found in top tier-healthcare, life sciences and business consulting firms

Processes

Data curation, signals detection and decision science methods are grounded in the intricacies of the specialty, rare disease and precision medicine markets

SYSTEM OF INSIGHT

Technology

Deployment of deep learning, artificial intelligence and evolutionary computation to transform noisy and unconnected real world data into real world insights

Data

A real world data universe consisting of 300M de-identified patient journeys and 65B social determinants of health signals with the ability to incorporate first-party data of any kind and scale

insights that uncover the ideal patient population and their healthcare ecosystem so they can accelerate the successful development and commercialization of life-savings therapies for specialty and rare diseases that lead to optimal patient outcomes quicker and with less risk.

TRANSFORMING HEALTHCARE AND LIFE SCIENCES WITH A SYSTEM OF INSIGHT

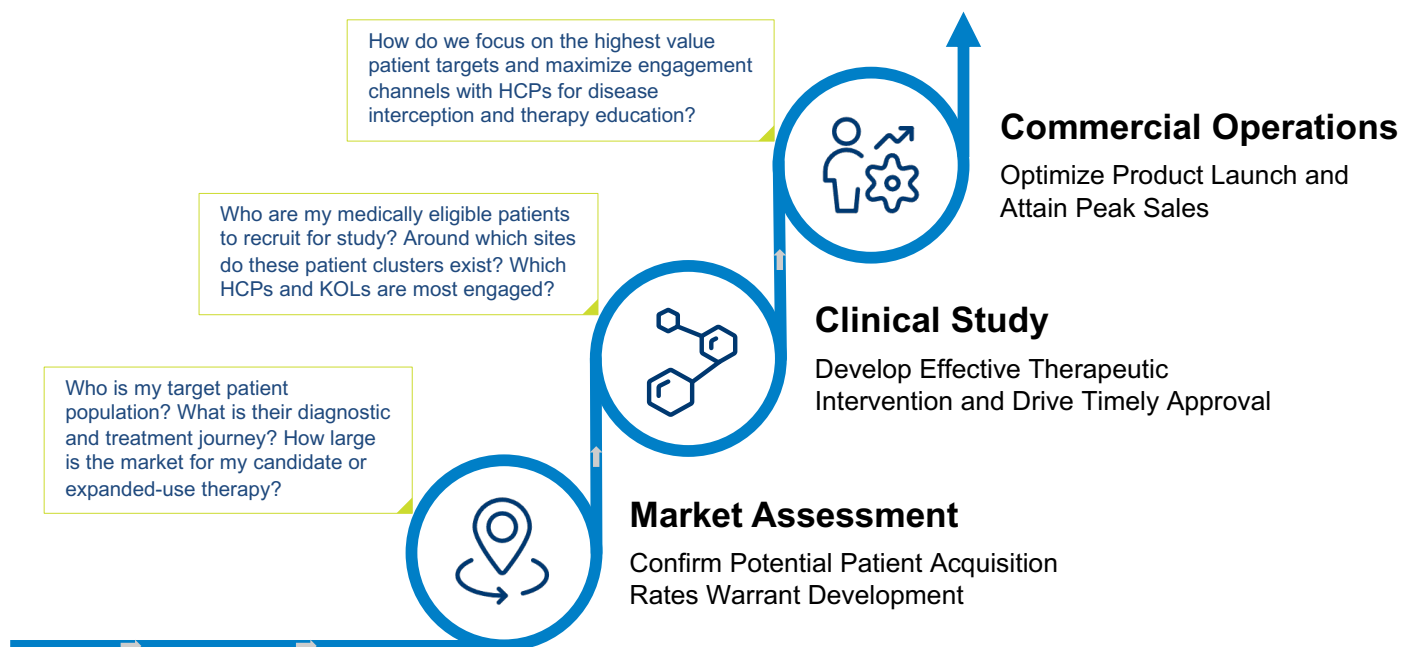### Closing the Loop Between Data, Insights, Actions and Outcomes

One of the best examples of Augmented Intelligence is the System of Insight, which carefully unifies people, process, technology and data to create a high-definition view into the specialty and rare disease marketplace.

This System of Insight is particularly effective as a single source of the patient equation throughout the product lifecycle of specialty and rare disease therapies, driving the greatest impact on decision points for three critical phases: Market Assessment, Clinical Study and Commercial Operations.

MARKET ASSESSMENT

Confirm Potential Patient Acquisition Rates Warrant Development

A crucial step in bringing a drug to market is understanding its potential reach, penetration and lifetime value. However, therapies designated for rare and specialty conditions necessitate assessment that goes far beyond combing through patient registries, mining clinical data and interfacing with support groups. While this traditional approach to market evaluation might lead to an initial pool of diagnosed patients, the analysis is severely limited, as those who are undiagnosed or misdiagnosed are typically excluded. To fully realize the ideal patient population for a new line of therapy, a System of Insight uncovers patients with a high propensity of disease affliction and, in most cases, increases a patient pool by 40x or more. Aside from accurately understanding epidemiology, symptomatology and prevalence rates, the technology can also map the typical patient journey and link treating HCPs, even those who are "hidden" because they are not formally designated as a relevant specialty care provider.

How do we focus on the highest value patient targets and maximize engagement channels with HCPs for disease interception and therapy education?

Who are my medically eligible patients to recruit for study? Around which sites do these patient clusters exist? Which HCPs and KOLs are most engaged?

Who is my target patient population? What is their diagnostic and treatment journey? How large is the market for my candidate or expanded-use therapy?

Commercial Operations
Optimize Product Launch and Attain Peak Sales

Clinical Study
Develop Effective Therapeutic Intervention and Drive Timely Approval

Market Assessment
Confirm Potential Patient Acquisition Rates Warrant Development

CLINICAL STUDY

Develop Effective Therapeutic Intervention and Drive Timely Approval

Because rare and specialty conditions have such low prevalence rates, typically lack ICD-10 designations and have few if any available therapies, HCPs are generally unable to recognize their diffuse symptomatology and thus accurately intercept the disease, provide diagnosis and recommend a course of treatment or appropriate clinical study. Even worse, misdiagnosis, or even no diagnosis, can serve to increase patient frustration over time as they continue to seek the opinions of HCPs and, in the process, prolong their diagnostic and treatment journey. The result is a skeptical patient population unable to seek appropriate care or access a potentially life-saving clinical trial. A System of Insight changes this by uncovering clusters of undiagnosed and/or misdiagnosed patients that can be linked to sites, in addition to mapping their treating and referring HCPs, who can be engaged by sponsors and educated about the clinical study. Further, the technology can be utilized to minimize protocol revisions by verifying patients are medically eligible and thus have a low propensity for screening failure, as well as incorporate behavioral variables into the analysis to assure the highest likelihood of enrollment and retention. The impact is significant, with a 35% reduction of the typical multi-year study recruitment timeline and millions of dollars saved in the process.

> **Augmented Intelligence can reduce the typical multi-year recruitment timeline for a rare or specialty disease clinical study by 35%.**

COMMERCIAL EFFECTIVENESS

Optimize Product Launch and Peak Revenue Attainment

Commercializing a specialty or rare disease therapy is to ensure the greatest number of candidates have access to treatment, fully realize patient lifetime value and maximize shareholder return on investment. Establishing a therapy as the standard of care and thus establishing a trajectory towards peak revenue recognition can be accomplished by discovering, mapping and engaging the ideal HCPs using a System of Insight. Once physicians with high probability patients under care are discovered, MSLs and field representatives can be strategically deployed for disease interception and intervention education. By precisely targeting these HCPs, resources are conserved because field personnel are not wasting time, money or effort searching for the wrong physicians and patients, and instead are focused on engaging only those treating bona-fide candidates for a therapy.

It's common to add hundreds of patient prescriptions within 6-months of deploying System of Insight-driven analysis.

Uncovering high value patients through 1:1 engagement with influential physicians maximizes effectiveness and efficiency, as specialty and rare disease sufferers trust the opinions of their HCPs over messages via programmatic, social and television advertising. In fact, it's common to add hundreds of new patient prescriptions within 6-months of deploying the analysis made possible through this technology.

For healthcare and life sciences organizations concentrating on the specialty or rare disease market, Augmented Intelligence, specifically the use of a System of Insight, is indispensable in creating a high-definition, intelligence-driven enterprise equipped to make smarter decisions with a higher-probability of success throughout the product lifecycle. Standardizing on this approach creates a healthier portfolio, provides the agility to capitalize on new opportunities and helps shape a long-term innovation agenda. And for those who embrace it, the promise of precision medicine can become an analytical reality.

About IPM.ai

IPM.ai (www.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 commercialization of precision medicine for specialty and rare diseases. IPM.ai's system of insight optimizes drug development, clinical study, product launch and commercial operations by utilizing granular-level longitudinal analytics, artificial intelligence and machine learning in conjunction with a real world data universe of over 300 million de-identified patient journeys and 65 billion anonymized social determinants of health signals.