# **RWD & AI for Rare Diseases**

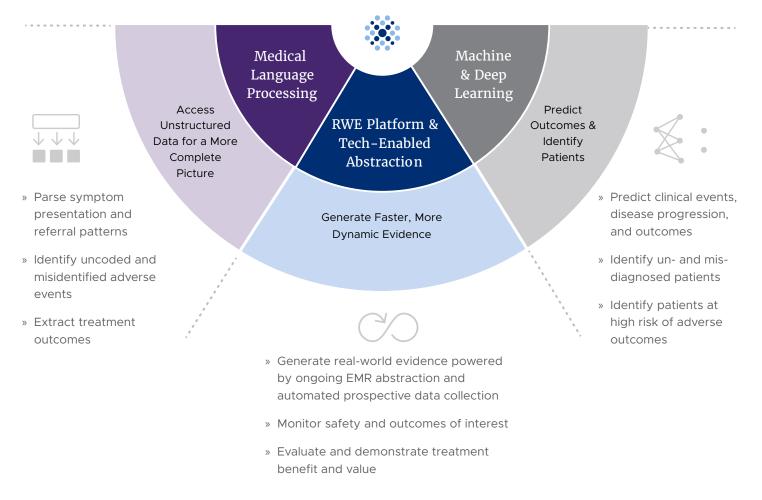
Accelerate Research, Speed Clinical Diagnosis and Improve Outcomes

# The Challenge

With an average diagnoses time of 4.8 years from first symptom onset, rare disease patients face challenges with receiving appropriate treatments (if any exist) in a timely manner. Clinicians and healthcare providers spend countless resources on trying to identify and manage the symptoms of these patients. Pharmaceutical manufacturers struggle to recruit patients and to generate the evidence needed for approval, access, and coverage.

## A RWD & Al Solution

With access to more than 300 million patient lives, we're combining the power of real-world data and artificial intelligence (AI) technologies to advance clinical development, outcomes research, and precision medicine for rare diseases. Using machine and deep learning, medical language processing, and other advanced technologies, we help life sciences companies, payers, and providers accelerate research, reduce the time to clinical diagnosis, and improve outcomes. Our platform consumes health system and payer data and identifies patients at higher likelihood for undiagnosed conditions or for positive or negative outcomes within known rare disease cohorts.



# case study Identifying High Risk Patients

# Situation

Cystic fibrosis (CF) is associated with a significant morbidity and reduced life expectancy with over 90% of deaths in patients with CF attributable to progressive obstructive lung disease. Personalized care in CF entails optimizing therapeutic approaches for each individual patient, identifying highest risk individuals, and understanding the drivers of risk. A leading patient organization was looking for ways to identify high risk patients and actionable insights to improve health outcomes.

### Solution

Using registry data from the association, linked data from the OM1 Real–World Data Cloud and machine learning technologies, OM1 built algorithms and predictive models to determine the likelihood of five outcomes of interest within specified time intervals, with the intention of the association to choose three models to move forward with optimization and implementation.

#### Results

Initial evaluation of the models revealed high confidence results. Three models were selected for further refinement: mortality, optimal nutrition at age 6 years, and acute pulmonary exacerbations (APEs).

The final results show a receiver operating characteristic **(ROC) curve of 0.94** for the mortality model and a **0.74 ROC** for the optimal nutrition model. The next steps will be implementation of the models within select centers.



