

# CASE STUDY:

## Rare Disease Patient Identification and Commercialization of Therapy

### *The Case for Real-World Data (RWD) in Delivering Targeted Patient Care*

#### BACKGROUND

Rare diseases are complex, and with limited research available, therapeutic options are often limited. Gene therapy, a growing area of clinical research, is showing great promise in treatment that may be life-altering for patients with many rare diseases.

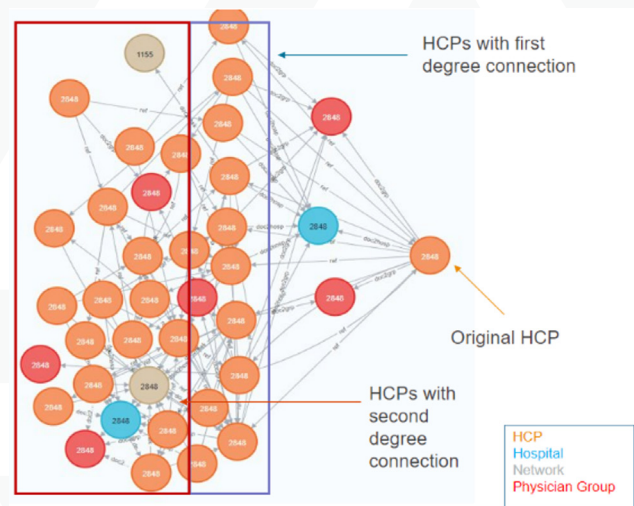
#### OBJECTIVE

A client who specializes in gene therapy for a particular rare disease came to EVERSANA looking to better identify and target their therapy to patients and healthcare providers. They looked to gain a deep, data-driven understanding of:

- The patient journey across multiple age groups of patients.
- Prescribing patterns of healthcare providers who specialize in this disease.
- Geographical factors impacting patient care.
- Cost of care.
- Additional factors impacting patients and healthcare professionals in the treatment of this disease.

#### CHALLENGES

While frequently diagnosed at birth, symptoms and comorbidities of this disease can change over time, making identification of suitable patients challenging. The client had limited data available from primary research and clinical trials, but was lacking in real-world data that examined healthcare professional behaviors and the patient journeys across their lifetimes.



*Influence Maximization: Visualizing the Impact of the Most Influential Node*

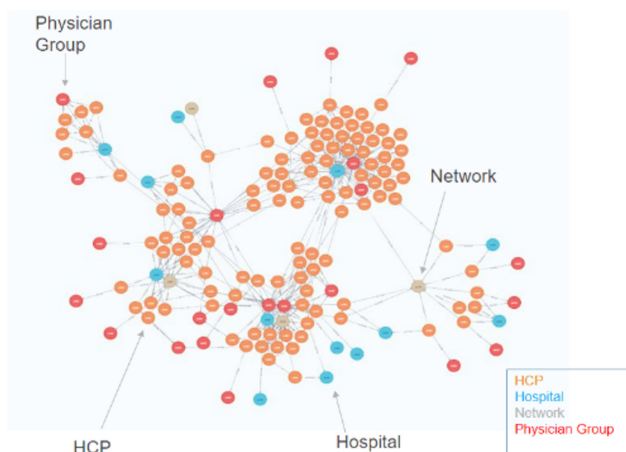
## STRATEGY

Through a strategic and consultative approach, EVERSANA established a structured and holistic plan that delivered an interactive dashboard of RWD and actionable insights, powered by **ACTICS** by EVERSANA™, a premier, tech-enabled solution that delivers commercial analytics, integrated patient analytics, pricing and market access analytics, along with Real World Data (RWD) and Health Economics & Outcomes Research (HEOR). This dashboard was critical to inform the client's clinical and go-to-market strategy and included:

1. Successfully defining a patient study cohort with input from the client and EVERSANA's clinical team for further analysis.
2. Generating separate insights into the disease of patients in different age groups using machine-learning methods such as Mutual Information.
3. Analyzing the existing payer landscape via cost-of-care analysis.
4. Assessing a graph-based referral network to generate communities and identify key opinion leaders.

### Community Detection: Community 1 (Large Community)

- There are **115 HCPs**, **13 Hospitals**, **23 Physician groups**, and **4 Networks** associated with the community.

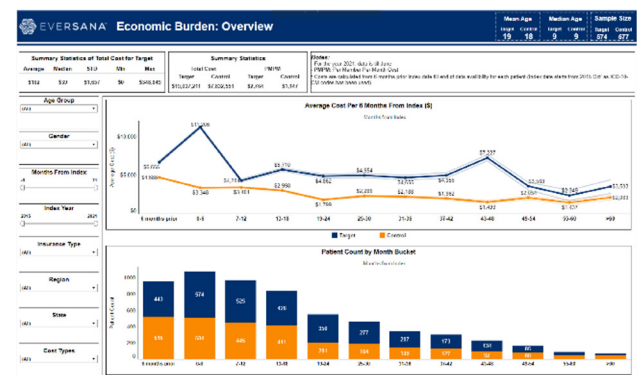


### Referral Analysis

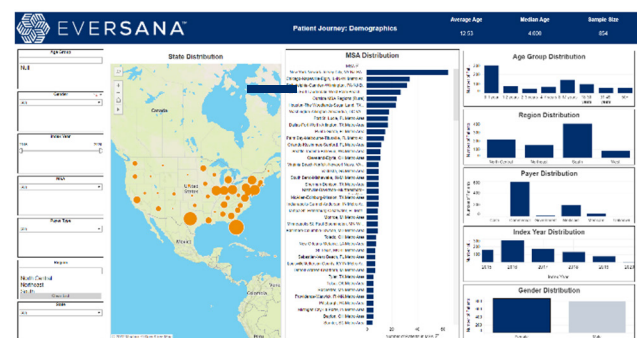
## RESULTS

Based on the data and analysis provided by EVERSANA, the client successfully:

- Established guiding principles for therapy/ indication by creating a patient definition.
- Identified high-value targets.
- Characterized the existing market landscape.
- Addressed key business questions through a detailed, longitudinal cohort analysis.
- Visualized the results through custom and interactive Tableau dashboards.



### Economic Burden Overview



### Patient Journey Demographics

Learn how EVERSANA leverages scale, expertise, data and analytics services to accelerate preclinical/clinical development and commercialization of cell and gene therapies for companies and research institutions. Visit [EVERSANA.com/products/ACTICS](https://EVERSANA.com/products/ACTICS) to request a demo or to contact our team of experts.