



## BEYOND THE GUIDELINES

### UNCOVERING SUBTLE DIFFERENCES IN RARE DISEASE PATIENT MANAGEMENT

#### THE CHALLENGE

A biopharmaceutical company was preparing to launch a new gene therapy for a rare, chronic, autoimmune disease of the liver that progresses slowly. Between 30 to 50 percent of patients do not achieve a complete and sufficient response to the currently available treatment. The disease, with a prevalence of 1-5/10,000, affects about 15,000 people in France, and its management is concentrated over a few expert centers.

The company sought to gather a deep understanding of this rare gastrointestinal (GI) disease in France, as well as real life data for future forecasting, market access and other healthcare marketing activities. The company engaged Kantar Health for a better understanding of the management of this disease beyond current guidelines, including achieving a representative sample of the patient population in France and a characterization of their response to treatment.

#### THE KANTAR HEALTH DIFFERENCE

Kantar Health implemented a two-pronged approach, which included both qualitative and observational studies. The qualitative phase delivered a deep understanding of current treatment objectives, thresholds, challenges and unmet needs in a dynamic market, where the only previously available data was emanating from research centers. The observational study featured data from 56 physicians and experiences from 378 patients.

Our study findings showed that about one-third of the patients affected by this rare GI disease in France are diagnosed at a late stage of the disease. The real life data indicates that of the patients receiving the current standard therapy, 41 percent are not responding, based on Paris criteria used in the expert centers.

This research is highly significant, given that our results yielded new real life data in an area where new data had not surfaced in the previous 10 years.

## AT A GLANCE

It's all in the detail, and that's especially true for leveraging real life data to better understand the subtle differences in patient management for a rare disease, as well as characterizing the therapeutic response to current therapies.

#### FOR MORE INFORMATION

Please contact [info@kantarhealth.com](mailto:info@kantarhealth.com), or visit us at [www.kantarhealth.com](http://www.kantarhealth.com).

#### WHY KANTAR HEALTH?

Kantar Health is a leading global healthcare consulting firm and trusted advisor to many of the world's leading pharmaceutical, biotech and medical device and diagnostic companies. It combines evidence-based research capabilities with deep scientific, therapeutic and clinical knowledge, commercial development know-how, and brand and marketing expertise to help clients evaluate opportunities, launch products and maintain brand and market leadership. Our advisory services span three areas critical to bringing new medicines and pharmaceutical products to market – commercial development, clinical strategies and marketing effectiveness.