



LAUNCHING A PEDIATRIC RARE DISEASE DRUG IN CHINA

MIXED-METHODS RESEARCH OFFERS KEY INSIGHTS TO ACHIEVING UPTAKE ACROSS 11 PROVINCES

THE CHALLENGE

A world leader in biotech for rare disease launched a drug in China for the treatment of a rare genetic metabolic disease. While the company has been engaging KOLs in Tier 1 cities, it's understanding of physician diagnosis and treatment patterns in Tier 2 and 3 cities is limited. In order to maximize penetration across all regions, the company wanted to identify potential barriers across the patient journey and address any unmet needs, as well as better understand the market landscape in the regions where they had little or no presence.

Considering that there are a number of challenges for conducting research in pediatric rare diseases, greater efforts are needed to secure the caregiver's perspective in research. Additionally, with the company not possessing a list of KOLs to be engaged in Tier 2 and 3 regions, a database needed to be generated for recruitment, and disease prevalence and reimbursement policy which could potentially vary across provinces – further complicating matters.

THE KANTAR HEALTH DIFFERENCE

Kantar Health implemented an approach that used mixed-methods research, combining qualitative and quantitative research. Using a combination of desk research and in-depth interviews, we identified the KOLs, as well as conducted interviews to identify the barriers across the patient journey and provide actionable

insights for obtaining information in the regions with little or no coverage.

In the first phase of our work we employed a mix of online research – including hospital websites, forums and publications – telephone research, and snowball sampling to build out the recruitment database. A rigorous methodology was performed to ensure data quality. In the second phase, which entailed in-depth interviews, we conducted face-to-face discussions with KOLs across 11 Chinese provinces to gain a holistic view of the diagnosis and treatment landscape for the rare disease. Additionally we gathered caregivers' testimonies that offered a perspective on the rare disease and the current treatment, as well as key insights on opportunities to improve care across the patient journey.

This client engagement was a complete success. We identified multiple opportunities across the patient journey for our client to pursue, including: a valuable summary of the current reimbursement status across provinces that will benefit the client's discussion with payors and policy makers; shared examples of patient access programs sponsored by government bodies and companies; and key insights on the roles of healthcare professionals in the treatment of this disease, which can be used to empower patients and achieve better health outcomes.

AT A GLANCE

Launching a pediatric rare disease drug in a mature healthcare market can be a challenging task. However, launching this type of medicine in a key growth market such as China comes with its own unique barriers.

Kantar Health recently implemented a mixed-methods research approach to maximize uptake and patient benefit for a critical metabolic disease medicine.

FOR MORE INFORMATION, PLEASE CONTACT
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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.