TMC Consulting

CASE STUDY

Accelerating orphan drug approval in rare types of genetic obesity with TMC's expert regulatory support

Background & Objectives

Rare types of genetic obesity are caused by single-gene variants (monogenic) or genetic syndromes (syndromic), and typically affect a small number of people, often from an early age. Many of these cases remain underdiagnosed, as early-onset obesity is often not genetically tested — or diagnosed — so prevalence is most likely underestimated.

TMC, a global pharma services company, was appointed by a US-based biopharmaceutical client focused on developing and commercialising treatments for rare types of genetic and neuroendocrine diseases of obesity. TMC provided regulatory support and strategic guidance from early on in the client's drug development programme, enabling the biopharmaceutical company to market the drug globally.

Challenges & Solutions

Bringing a new drug to market is a complex, multi-phased process that demands rigorous planning, data generation and regulatory compliance.

For therapies targeting rare diseases, the pathway can be especially challenging due to small patient populations, limited clinical data and the urgent unmet medical need. Sponsors must also account for unique regulatory designations and streamlined approval mechanisms, leveraging orphan drug incentives and real-world evidence where appropriate.

TMC Consulting supported the client in successfully obtaining PRIME designation, which provided the benefit of early and proactive EMA support and the potential for accelerated assessment.

TMC was involved in the drug development programme from an early stage, supporting the client with their ODD, PIP and PRIME procedures, as well as non-clinical expertise and clinical trial support.

Because of TMC's expertise in orphan conditions, we were able to advise the client on how to build their regulatory dossier and provide their data in a desired format, as well as support them with scientific advice that added a level of pragmatism to their drug development pathway.

By addressing rare disease-specific requirements and effectively working through the **regulatory submissions checklist**, TMC helped the client avoid the pitfalls that are often made by small biotech and pharma companies trying to accomplish new drug approvals in-house, often with limited resources and expertise.

New drug approvals checklist

The team also prepared and submitted the marketing authorisation application, leveraging the efficiencies built from TMC knowing the product so well.

Additionally, as an established EU/EEA-based Marketing Authorisation Holder (MAH) with SME status, TMC was able to act as the client's MAH until they were ready to take on the role themselves and build their entity for Europe.

Outcome

Since successfully gaining marketing authorisation, the client retained their asset and has gone on to develop the drug into different indications, continuing their trusted partnership with TMC.

By engaging with TMC early, the client benefitted from faster regulatory and assessment, enabling them to advocate for a drug development pathway that prioritised the unique needs of the rare genetic obesity patient population. The client was also able to take advantage of TMC's SME status, allowing them to receive fee discounts and other benefits, such as deferred payments and the SME office preparing translations.

TMC Consulting provides expert-led strategic and operational pharma consultancy services — spanning regulatory support, clinical development, pharmacovigilance, medical affairs and quality management. Our experienced specialists partner flexibly with small to mid-sized biotech and pharma companies to help them reach critical drug development milestones compliantly, quickly and cost-effectively.

Contact TMC Consulting today at connect@tmcpharma.com to see how we can accelerate your regulatory submission and help you secure market authorisation approval — efficiently and cost-effectively.