

Using Real World Evidence to Support an Orphan Drug Application

CHALLENGE

As part of support for an Orphan Drug Application (ODA), MMS was asked to identify prevalence for a subset of a disease population, where there wasn't relevant literature support in scientific journals. The prevalence estimate was integral to respond to disease prevalence concerns from the Office of Orphan Products Development (OOPD) after the application had been held in abeyance on two previous occasions.

MMS SOLUTION

With the **flexibility** to handle non-traditional project requests and familiarity with the real world evidence (RWE) routinely collected from the delivery of health care, MMS immediately jumped in and assigned an internal team to begin tracking down the necessary product data. The MMS team determined that using actual sales data from the United States and shipment data from Europe for a currently marketed competitor would best support a prevalence estimate in the subset disease population. Armed with this data, regulatory writers and strategists at MMS were able to review and analyze, providing a comprehensive response that **exceeded the sponsor's expectations**.

OUTCOME

The analyzed data that MMS provided demonstrated that the population subset was less than the threshold of 200,000 patients required for orphan products. This unique approach, using RWE, allowed MMS to complete the response to the regulatory authority in time, with accurate supporting materials.

While using non-traditional data and RWE is usually less often considered, MMS believes that there is **limitless potential** for its continued use in drug development and global regulatory submissions.

*"A big thank you to the MMS team for pulling this one off through great **teamwork, effort, and commitment**. We appreciate your efforts in keeping us focused and all this data straight."*

-Executive Director, Clinical Development,
Small Pharma Sponsor

