CTA and Independent EU/UK Legal Representative for a Pivotal Paediatric Trial

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The Situation

In 2023, a biotechnology company was planning to initiate a Phase 3 pivotal clinical trial of an investigational medicinal product intended to treat a rare neuromuscular disorder affecting children. As this pivotal programme was to form the basis of the company's planned Marketing Authorisation Application (MAA), the trial is subject to heightened inspection readiness expectations, including Good Clinical Practice (GCP) audit scrutiny.

The company wished to find an independent, trusted partner who had the appropriate expertise to manage their EU and UK Clinical Trial Application (CTA) submission activities alongside provision of Legal Representative (LR) services, thus ensuring heightened documentation and process compliance for their high-profile clinical trial. The company asked DLRC to act as their strategic partner due our in-depth EU and UK regulatory expertise in paediatric development, clinical trials, EU presence and our breadth of complimentary regulatory services on offer.

DLRC's Solution

Over a three-year period, DLRC and Orphix provided end-to-end regulatory and Legal Representative support, covering CTA submission and all subsequent maintenance activities, including modifications, milestone and safety notifications, DSUR and IB updates, and coordination of any ensuing health authority communications. Acting as the company's independent and trusted partner, we ensured ongoing compliance with EU and UK clinical trial requirements while working collaboratively alongside the sponsor's chosen Clinical Research Organisation (CRO).

Our Legal Representative onboarding and submission-readiness workflows include trial set-up reviews and compliance checks to identify potential risks to timely CTA submission. These activities align closely with our CTA preparation and sponsor review cycles, allowing issues to be resolved early and reducing the risk of validation comments. Together, these processes established a robust foundation for ongoing regulatory maintenance, strengthened overall trial compliance, and positioned the programme for future MAA-related inspection readiness.

The DLRC clinical trial and Orphix Legal Representative teams worked seamlessly to deliver high-quality submissions to agreed timelines. This integrated approach enabled faster Legal Representative review, as potential CTA issues were identified and resolved upfront by the DLRC CTA submission team which proved to be particularly valuable when responding to tight question or submission deadlines.

The Outcome

DLRC has supported this company for over three years, providing strategic input to the clinical development programme to position it optimally for successful EU and UK MAA submissions. Our Legal Representative team has also advised them on the management of unforeseen situations, ensuring ongoing compliance with EU and UK legal requirements and timely submission of all required notifications.

To date, we have supported the submission of an additional open label extension study in both the EU and UK within challenging timelines. Building on our long standing partnership and deep knowledge of the product and its clinical development programme, we are well placed to support the upcoming MAA and deliver further efficiencies throughout the process.