CASE STUDY

PEDIATRIC LEUKEMIA

BACKGROUND

PPD was selected to conduct central study start-up, clinical monitoring, investigational product services, medical management, patient recruitment, pharmacokinetics, pharmacovigilance, project management and regulatory affairs activities for a Phase I/II pediatric leukemia study that evaluated study drug given to pediatric patients with relapsed or refractory acute leukemia.

OBJECTIVES

The primary endpoints were to assess the recommended pediatric dose of the drug and then to evaluate the safety and efficacy of the recommended dosing.

Key secondary endpoints were to assess the pharacokinetics of the drug, biological activity and duration of remission.

CHALLENGES

There were multiple challenges encountered on this study, which started with ensuring recruitment would end at the expected target requested by the client. When proposing this study, there were no initial opportunities to review or revise the proposed enrollment timeline; PPD had to stay focused on how the timelines could be met. After study initiation in some countries, the sponsor and PPD quickly observed that based on current study set-up, enrollments likely would not be completed by the end of the proposed timelines. The sponsor and PPD worked together, based on feasibility and past history working within this indication in many countries to select additional sites and countries as back-ups to ensure a timely enrollment of all study subjects.

This was a difficult patient population to enroll, and mitigation strategies started early in the study to ensure there were enough sites to accrue the expected amount of patients for both phases/parts of the study.

The regulatory requirements posed an extremely difficult challenge throughout the entire start-up phase of the study due to the increased amount of countries chosen to meet the timelines. During start-up there were new regulations instituted in Russia that caused delays in the start-up of this country and decreasing the amount of patients that would potentially be enrolled to the study. There were also contract issues occurring in Poland that caused additional delays in this country, decreasing the amount of patients that could potentially be enrolled in this country.

Data quality and cleaning expectations for the study changed midway through the study, causing data retrieval issues for the study. The data retrieval changed from a 75% data cut date directly from 50% to 100% causing more data to be retrieved in a shorter time period at the end of the trial.

STRATEGY

PPD worked closely with the sponsor and in the initial stages of the study to determine the appropriate amount of sites to meet the projected patient accruals. In an effort to keep sites engaged and motivated, PPD held weekly conference calls with all participating sites to assess performance, share best practices and address questions related to ongoing issues to include information-sharing on a regular basis.

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PPD's patient recruitment services were recommended to provide a study website to provide more awareness on the study.

PPD had a strong dedicated team with minimal changes throughout the course of the study. PPD also had strong relationships with the sites in the initial stages of the study and throughout. The sites were notified weekly of the status of the study along with regular newsletters, providing the data cleaning activities and timelines to ensure sites were aware of tight timelines.

RESULTS

+ After initial study start-up, PPD's mitigation strategy of country/site mix proposed initially was effective and patient enrollment was completed prior to the initial timelines. Back-up sites were chosen and pursued throughout the trial to account for the missed patients in countries with regulatory and contract delays.

+ PPD's utilization of patient recruitment services provided viable information to users that helped recruitment of patients to the study; over 4,000 users have viewed the website.

+ All data cleaning activities were provided on time for 25%, 50% and 100%.

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