Case Study:

NAVIGATING AGGRESSIVE RECRUITMENT TIMELINES: SUCCESSFUL STRATEGIES FOR PHASE II DUCHENNE MUSCULAR DYSTROPHY TRIAL

A Phase II study to evaluate the effects of small molecule designed to selectively inhibit type II fast skeletal muscle myosin in patients aged 4-9 years old with Duchenne Muscular Dystrophy.

66 ENROLLED PATIENTS
79 screened

14 SITES
11 Sites Initially

1 COUNTRY
United States

CHALLENGES

- Aggressive recruitment timelines
- Patient recruitment was challenging due to the rare disease nature of the study with limited patient populations
- Delays in study start-up
- Study cohort design caused multiple pauses in recruitment that could cause sites to become disengaged
- Higher than expected screen fail rate

RESULTS

Study successfully enrolled 66 patients over a 15-month period, which included 4 four-week recruitment pauses due to study cohort design

First Patient First Visit achieved within 4 months after project kick-off

Achieved enrollment timelines on schedule

THE SOLUTIONS

Strong Site Relationships and Site Engagement

- Engaged sites by notifying them of competitive enrollment and when expected screening windows were to open
- Ahead of screening windows opening, the team would routinely reach out to sites to see how many potential patients the sites currently had targeted for the study and made sure they met eligibility based on pre-screening information. This helped ensure there were enough patients in the pipeline for each subsequent cohorts' screening windows. If there was not enough, the Sponsor would be notified, who would reach out to advocacy groups to help identify referral patients to the study
- Site engagement was critical to get sites invested in the study and willing to help recruit their own patients to the study
- Sponsor attended site initiation visits to provide in person training on their IP, spotlighting the mechanism and how it differed from other drugs on the market or currently in trials

Recruitment Strategies

- Cast a large net for recruitment - made sure to select sites from different regions throughout the United States
- Advertised the study with advocacy groups or a third-party recruitment organization (e.g., myTomorrows)
- Drafted a Cohort Management Plan that outlined procedures on when sites were allowed to screen a patient
- Requested sites provide ample pre-screening information to minimize the chance of patients screen failing
- Additional sites were added to the study when the Sponsor increased the number of patients to be enrolled in the study midway through the study

Patient Support

- Sponsor contracted with travel reimbursement organization (e.g., SCOUT Clinical) who helped provide travel reimbursement and support. Sponsor also provided a childcare allowance that eased the burden for families with multiple children
- Sponsor allowed for patients to enroll at sites closer to families home even if site was not where they had routine care or would allow for reasonable long-term temporary housing accommodations
- Protocol design allowed for siblings both diagnosed with DMD to enroll together
- Patient-facing materials were developed to provide supplemental information to allow families to learn and understand the study. Once enrolled, additional materials were provided to help provide instructions to families on how to compliantly participate in study, easing the burden on the family from having to remember all the at home procedures