Enrolling Sickle Cell Patients Is Tough – So We Got Busy

Kick off a study of a drug to treat sickle cell disease and you're facing a tough climb. Experimental drugs for sickle cell are few and far between, so there is little in the way of clinical trial infrastructure. The patient population is demographically challenging, too, made up largely of socioeconomically disadvantaged people who find it difficult to comply with the rigors of clinical drug trials.

That's what made it so remarkable when we enrolled the first patient three weeks early in this Phase I open-label, dose-escalation trial of a drug to make hemoglobin more efficiently transport oxygen in sickle cell patients.

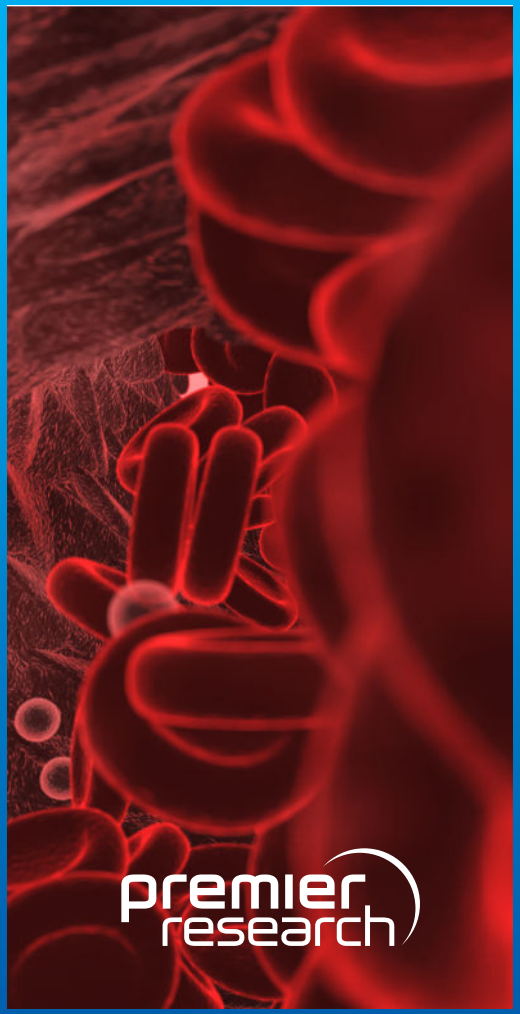
Recruiting for sickle cell trials is difficult not just because of demographics, but because to participate, patients must suspend participation in the only approved treatment regimen. So the sponsor was asking a lot when it imposed a tight timeline: less than four months from delivery of the final protocol to first patient in. Recognizing that subjects would be hard to locate, the sponsor budgeted 15 sites to achieve its target of 30 patients.

Extraordinary Discipline

Beating the ambitious four-month goal by three weeks required an extraordinary level of discipline. We selected a site that accommodated our need for fast start-up with an accelerated contracting process and eagerness to prescreen potential patients. Weekly meetings between the principal investigator and the project team – an uncommon level of intensity so early in a Phase I trial – helped us complete contract negotiations, implement regulatory processes, stand up an institutional review board, assemble lab kits, and put the investigational product in place.

With four of 15 sites now operating (and two more planned before the end of the year), the trial still has a lot of ground to cover over the next two years and is progressing rapidly on the strength of an enthusiastic sponsor and a highly engaged Premier Research project team.

RARE HEMATOLOGY



Our Response to a Short Timeline? We Beat It by Three Weeks

Study Description:
Phase I open-label, dose-escalation study to evaluate the safety and efficacy of a drug that helps hemoglobin more efficiently transport oxygen in sickle cell patients

Therapeutic Area:
Hematology

Geographic Scope:
15 sites in the United States

Patient Population:
30 patients (projected)

Length of Enrollment Period:
Two years (projected)

Outcome:
Despite a dearth of potential subjects – sickle cell trials traditionally are slow to enroll due to demographics and minimal clinical trial infrastructure – we signed up the first patient three weeks ahead of an already ambitious schedule. The trial is ongoing.

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