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The Case of the Elusive Protocol

A customer asked us to do a chart review of patients with hypophosphatasia, an extremely rare metabolic bone disease with a live birth incidence of about one in 100,000. The perinatal variant is rapidly fatal.

A proliferation of protocols

Initially informed consent was required if local regulatory authorities and/or IRBs required it. Then the Premier Research regulatory team advised that consent was needed only for living patients.

The sponsor's EU regulatory team said they required it for all patients outside North America.

The Canadian Research Ethics Board (REB) stated that they would not require consent from parents of deceased patients, because reminding them of their tragedy would do more harm than good. IRBs at some sites with only deceased patients said it was not a human study, and that the requirements for human studies did not apply. Which way to turn?

Finding the right page – and keeping everyone on it

Ultimately, we ended up trying six different protocols, before agreeing with the sponsor – and regulators – on the right approach.

We spent 10 months working out the protocols. After that, everything went smoothly. Screening charts and collecting data took seven months, and we delivered the final clinical study report four months later.

There wasn't any magic to our success. It was a matter of patiently adapting to changes, communicating clearly and frequently with all concerned, and maintaining our focus throughout. It also helped that we had regulatory specialists in each country to handle submissions on their home ground. As a result, we were able to balance the need for unusual flexibility with steadfast adherence to high ethical and scientific standards. It all goes to show: Good problem solvers are good at solving problems that are very different from the norm.

RARE DISEASE

Good Problem Solvers Are Good At Solving Problems

Study Description:
A chart review to evaluate survivability of pediatric patients with a rare metabolic bone disorder.

Therapeutic Area:
Pediatric rare disease.

Indication:
Hypophosphatasia.

Services Provided:
Chart review.

Geographic Scope:
Submitted in U.S., Canada, Spain, Germany, Switzerland, U.K. and France, with sites initiated and patient charts studied in all countries except UK and France.

Patient Population:
49 unique pediatric patients, mostly deceased, with HPP. (The initial goal was 50, but we discovered that one patient's chart had been submitted by two different sites.)

Outcome:
Unusually complex issues of informed consent had to be resolved before the study was successfully completed.

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North America: +1 919 627 9069 | Europe: +44 118 936 4000 | info@premier-research.com premier-research.com