Case Study #1: Identifying Problems by Keeping the End in Mind

SITUATION

A start-up gene therapy company with limited internal staff was developing a treatment for a rare but incurable infant disease. In order to attract investors and launch their IPO, Accudata Solutions was hired to develop a 2-5 year Clinical Development Plan encompassing both U.S. and European studies.

ISSUE

Accudata Solutions was asked to review an already-existing protocol of a planned Phase 1b/2a, placebo-controlled, dose-finding study which called for the participation of a DSMB to conduct interim safety reviews between dosing cohorts. At that stage, the client's executive team considered the study design final.

PROBLEM IDENTIFIED & RESOLVED

Because the client believed in the overwhelming superiority of the treatment, the protocol had given the DSMB the authority to recommend termination of the trial *even before collection of enough statistical evidence* of superiority. Had the study terminated prematurely, it is likely that the FDA would have refused to accept it as evidence of efficacy and would have required completion of a whole new study. Since the study was for an incurable disease, children randomized to placebo in the original study would have died unnecessarily. Accudata Solutions redesigned the study to remove interim analysis for efficacy.

CLIENT BENEFIT

By not having to repeat a study, the client realized the following savings:

- $3M-$4M in clinical trial costs
- 9 months of product development time
- the lives of 10-12 infants