SAFETY AND EFFECTIVENESS MATTERS

IMAGINE THIS:

An ultra-rare, fatal childhood condition exists with no effective treatment

Condition affects only 36 infants per year in the U.S.

THE SOLUTION:

Regulatory and Quality Consulting consultants submit an Orphan Drug and Pre-IND/End of Phase 2 (EOP2) Briefing Document to FDA

Secure meeting with the FDA

RESULTING IN:

FDA approves small clinical trial (25 patients)

Orphan Designation granted

Improved chances of therapy development