Ataxia-Telangiectasia (A-T): A grant of $70,966 has been made possible by Team Derek's Dreams and the A-T Children's Project to pursue one of three objectives: 1) to demonstrate a technique that uses multiple viral vectors or a non-viral vector to deliver and express the entire 9.2kb coding length ATM gene, potentially making gene replacement therapy possible for children with A-T, 2) to demonstrate a gene editing technique (rather than a whole-gene delivery method) that can correct ATM gene mutations observed in children with A-T by targeting small insertions, deletions and base swapping, and then confirming success with a functional assay, or 3) to identify and validate a clinically useful blood or CSF biomarker, other than NfL and AFP, that correlates with the neurological deterioration seen in A-T patients and that can be used to determine whether a potential therapeutic is having a positive benefit in a clinical trial.