



Research Focus Areas:

Ataxia-Telangiectasia (A-T): A grant of \$53,240 has been provided by Team Derek's Dreams and the A-T Children's Project to support a crucial research endeavor aimed at investigating the functional effects and potential therapeutic benefits of exon skipping in specific regions of the ATM gene for the treatment of ataxia telangiectasia (A-T). Targeted exon-skipping therapies, which have had success in other diseases, hold promise for children with A-T as they could potentially enable disease-causing mutations to be ignored. However, the application of this approach to A-T necessitates a more comprehensive understanding of the structure, function, and consequences of exon skipping within the ATM gene. By employing a combination of in silico and in vitro techniques to systematically examine each exon, the recipient of this grant will make groundbreaking contributions to the field, not only enlightening the A-T research community but providing actionable insights to developers of genetic medicines regarding regions within the ATM gene that could be omitted.

