

# ATAXIA TELANGIECTASIA RESEARCH - DRUGS AND CLINICAL TRIALS (JUNE 2023)



Description	Drugs	Preclinical	Phase 1	Phase 2	Phase 3	Available to patients	Contact
Improve Mitochondrial Function & Reduce Oxidative Stress	N-Acetyl L-Leucine >>						Intrabio, UK >>
	Nicotinamide Riboside (NAD+) >>						Oslo University Hospital, Norway >>
	Triheptanoin >>						Queensland Children's Hospital Brisbane, Australia >>
Inflammation	Intra-Erythrocyte Dexamethasone >>						Erydel, Italy >>
Gene Replacement And Editing	Hematopoietic stem cell transplant & FiCAT >>						CUN (Navarra) & UPF (Barcelona), Spain >>
	Nanoparticle chemistry with gene augmentation >>						University of Nottingham, UK >>
	Transposons method >>						University of Granada, Spain >>
Immunology	T-Car >>						Sheba Medical Centre, Israel >>
ASO Therapies	Splice site mutations >>						Harvard and Boston Children's Hospital, USA >>