FRIEDREICH'S ATAXIA TREATMENT PIPELINE

	DISCOVERY (Finding Potential Therapies/Drugs)	PRE-CLINICAL DEVELOPMENT (Testing in Laboratory)	IND FILED (Investigational New Drug; FDA filing)	PHASE I (Human Safety Trial)	PHASE II (Human Safety And Efficacy Trial)	PHASE III (Definitive Trial)	NDA FILED (New Drug Application; FDA filing)	AVAILABLE TO PATIENTS
Improve Mitochondrial Function & Reduce Oxidative Stress								
	SKYCLARYS™ (omaveloxolone) – Nrf2 Activator							**Biogen**
	Vatiquinone (PTC-743) - 15 LO inhibitor							PTC Therapeutics
	Elamipretide							Children's Hospital of Philadelphia
	NAD+ and Exercise in FA (ExRx in FA)							Children's Hospital of Philadelphia
	MIB-626							Metro International Biotech, LLC
Modulation of Frataxin Controlled Metabolic Pathways								
	Leriglitazone							Minoryx Therapeutics
	IMF & Dimethyl fumarate							Ixchel Pharma & University Federico II, Naples, Italy
Frataxin Replacement. Stabilizers or Enhancers								
	CTI-1601, frataxin replacement							Larimar Therapeutics
	Etravirine							Fratagene Tx & IRCCS Eugenio Medea
Increase FA gene Expression								
	DT-216, Gene-TAC							Design Therapeutics
	Oligonucleotides							University of Texas Southwestern, Univ of Massachusetts
Gene Replacement and Editing								
	LX2006							Lexeo Therapeutics
	AAVrh.10hFXN, cardiac							Crystal/Cornell
	CNS & Systemic, AAV approaches							CRISPR, Lacerta, Neurocrine/Voyager, Prime Medicine, Solid Biosciences, Tune Therapeutics

FARA Friedreich's Ataxia Research Alliance

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