

FA pipeline update: a new ERA in FA



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FRIEDREICH'S ATAXIA TREATMENT PIPELINE

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

Tune Therapeutics

CRISPR, Lacerta, Neurocrine/Voyager, PTC, Prime Medicine, Solid Bioscience/AavantiBio, Takeda/StrideBio,

and Editing

CNS & Systemic,

AAV approaches



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FIRST MEDICATION TO TREAT FA APPROVED ON RARE DISEASE DAY!





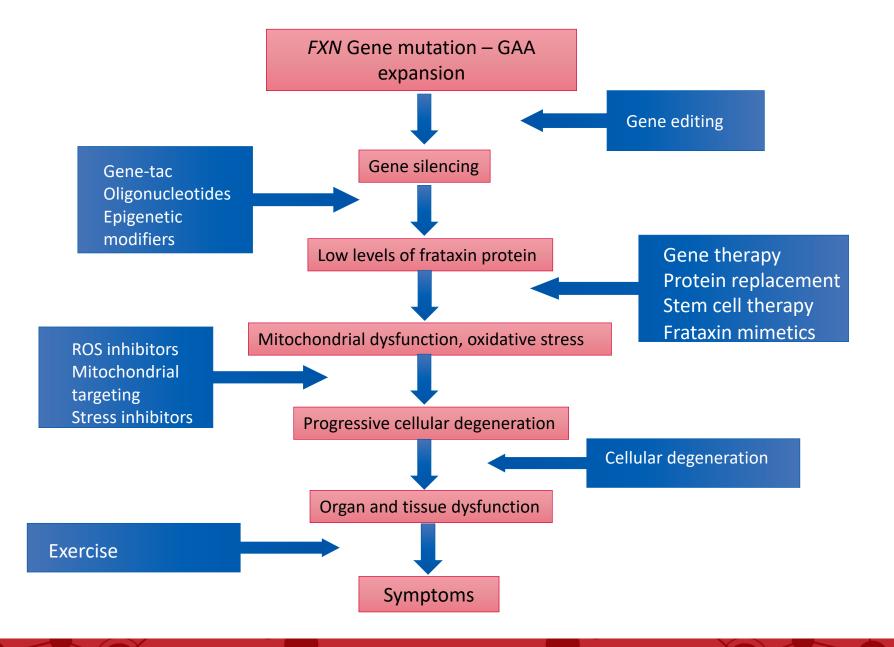
FARA continues to advocate for Reata to:

address the access questions and needs of the GLOBAL community

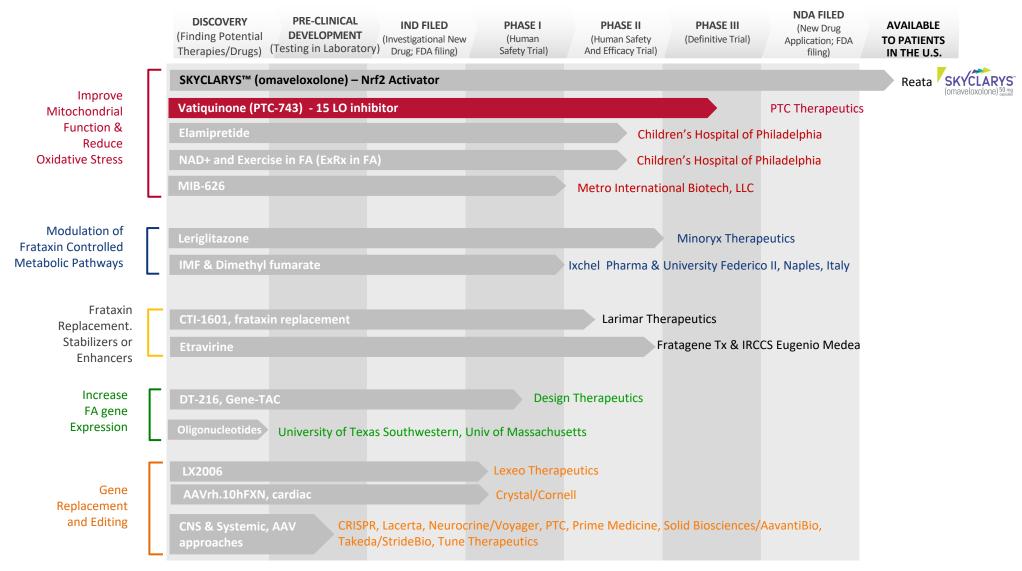
initiate paediatric studies as soon as possible

provide updates to the FA community directly (in lay language) and address the recurring questions and concerns





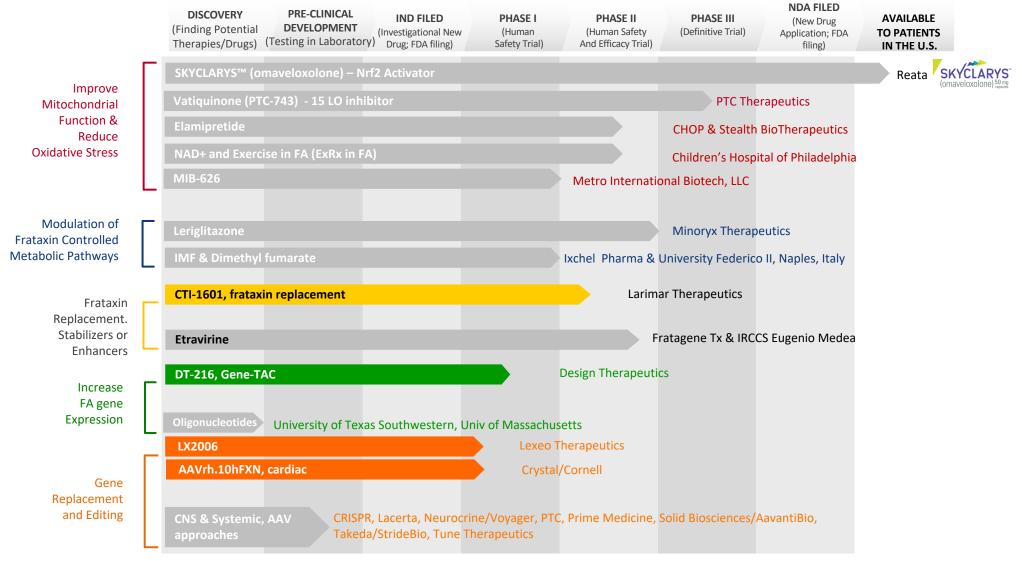
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Global clinical trials horizon for FA



Need to support global trials



More education to the FA community



Promote clinical sites with FA expertise



FA GCC GLOBAL CLINICAL CONSORTIUM





CLINICAL TRIALS 101: A GUIDE FOR PARTICIPANTS



ARE YOU CONSIDERING ENROLLING IN A

CLINICAL TRIAL FOR FA?

THIS GUIDE WILL HELP!



Guide to patient engagement

Resource for industry partners

<u>Timeline:</u> Start >1 year before entering clinic



Follow Through

Design your Trial with

Recruit!



THANK YOU!







