In the last 12 years, The FARA Energy Ball has...

- Funded >$15 Million in Drug Discovery and Development and Clinical Research; including research leading to >8 potential therapies being tested in clinical trials
- Expanded the research capacity of the Collaborative Clinical Research Network (CCRN) CCRN in FA:
  - >1,200 individuals with FA enrolled in clinical research including site at USF
  - Biobank with DNA, RNA, serum, and plasma samples available for collaborative research with pharmaceutical companies
- Raised unparalleled awareness of FA within the Tampa Bay community and beyond

2021 Initiatives focused on advancing drug candidates in a deep and diverse treatment pipeline - FARA needs to raise >$7 million

- Grow the scientific community to bring new ideas, technologies and expertise to advance the field
- Develop the genetic based research findings to therapeutic agents for people living with FA
- Fund research to understand the cell/tissue-type specific vulnerability in FA
- Expand the clinical research and care centers for FA to a worldwide network
- Identify early markers that are predictive of cardiac risk to reduce mortality caused by heart failure
- Fund the International FA Neuroimaging Consortium, TRACK-FA study, conducting detailed structural and functional imaging of the brain and spinal cord to identify markers that can be used to measure response to treatment in next generation clinical trials
- Invest in clinical trial readiness for genetic therapies that are advancing toward clinical trials, ie, education for FA community

“Acting alone there is very little any of us can accomplish. Acting together there is very little we will not accomplish.”

-Ron Bartek, FARA Founder and President
Because of supporters like you...
FARA has been able to dramatically increase the number of potential treatments for FA in just a few years (see graph below). To learn more about the progress of these additional approaches, please visit our website at: http://www.curefa.org/pipeline

Friedreich’s Ataxia Treatment Pipeline

- Omaveloxozone (RTA-408) - Nr12 Activator
- PTC-743
- RT001 (4Pufes)
- NAD+ and Exercise in FA (ExRx in FA)
- MIB-626
- MIN-162
- IMF & Dimethyl fumarate
- Frataxin replacement CTI-1601
- Etravirine
- Resveratrol
- Gene-TACs
- XCUR-FXN
- Oligonucleotides
- Gene Replacement and Editing
- CNS & Systemic, AAV approaches

BREAKTHROUGHS AND MILESTONES...

- 1996 The disease-causing gene mutation was identified
- 1998 FARA was founded
- 2001 The first animal models of FA were created
- 2006 Number of researchers working in FA doubled and FARA Patient Registry was launched
- 2007 International Collaborative Clinical Research Network for FA was established
- 2009-2011 Number of new drug candidates and pharmaceutical companies interested in FA doubled
- 2012 Gene therapy shown to reverse FA cardiac disease in a mouse model
- 2013 5 new clinical trials initiated and 3 new candidates added to treatment pipeline
- 2014 FARA established Penn Medicine / CHOP Friedreich’s Ataxia Center of Excellence; 3 new pharmaceutical companies formed to advance gene therapy in FA
- 2015-2017 FARA implemented collaborative FA Biomarker initiative with industry & academic partners
- 2018 Gene therapy shown to reverse FA neurological disease in mouse model
- 2019 First drug trial to demonstrate positive treatment effect
- 2020 International Consortium Launches to Study Neuroimaging Biomarkers in FA
- 2021 First drug trial to show elevation of the frataxin protein (low frataxin causes FA)

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