

**ICORD  
Super Bowl Sunday in U.S.  
Super ICORD Sunday in Tokyo**

**FARA**

**Friedreich's  
Ataxia  
Research  
Alliance**

**Joint-Venture Philanthropy and Public-Private Partnership**

**Ron Bartek  
President, Co-Founder**

# Conflicts of Interest

---

- **Shareholder -- Not personally, but FARA holds \$1.1M in Series A preferred stock in Edison Pharmaceuticals.**
- **Grant/Research Support -- Not personally, but FARA has received a few thousand dollars from each of a number of pharmas to support international conferences and a few fundraising events. On the other hand, FARA has awarded to pharmas about \$4.5M in research grants and invested \$1.1M.**
- **Consultant -- No.**
- **Employee -- of FARA.**
- **Paid Instructor -- No.**
- **Speakers Bureau -- No.**

# What are Friedreich's Ataxia & FARA?

---

- Rare, hereditary, life-shortening, neuromuscular disorder usually diagnosed in childhood; causes loss of coordination and strength in the arms and legs; diminished vision, hearing and speech; scoliosis, diabetes; life-threatening heart condition; full-time wheel-chair use by the mid teens or twenties; currently no treatment. Death in early adulthood.
- ~ 1 in 50,000 in populations of European origin
- ~ 1 in 100 are carriers; recessive trait.
- FARA is non-profit advocacy org. that funds research. Began in 1998; were all volunteer until 2006; raised only \$100,000 in first year; knew we could not do it alone; needed to collaborate and partner rather than confront and compete.
- Had to overcome the hurdles of rare diseases. Don't have "the answers" but will share our experiences. How has FARA tried to help advance development?



# Had to Assemble & Grow the Field

- Assemble partners; insist on & support collaboration
  - 1999, 6 months after FARA began, world's first FA scientific conference; 80 scientists; zero pharmas; one other advocacy org
  - 2011, 4<sup>th</sup> international conference in Strasbourg, turned away many to hold to 200 scientists, dozen pharmas, 10 other advocacy orgs.
- Public-Private Partners
  - NIH (ORDR, Neuro Institute, etc.): conferences, grants
  - FDA: meetings to seek approval of trials (Pre-IND), etc.
  - U.S. Congress: >\$ for NIH, FDA; rising tide lifts all boats
  - HHS: New Born Screening
  - Academic scientists, other advocacy orgs, pharmas, patient families, donors, supporters
  - FARA ~ \$17M + similar amount from partners (excluding pharma), so >\$30M to FA research + \$Ms from pharmas



## Try to Grow Low-Hanging Fruit

- Increasing interest in rare diseases. Pharmas looking for low-hanging fruit. Advocacy orgs can grow it by:
  - Funding, maturing the basic, discovery science (pharmas almost twice as successful developing discoveries from outside sources vs. inside)
  - Developing translational tools (bio samples, tissue & organ bank, cell lines & models, animal models, high- and low-throughput screening assays, biomarkers)
  - Developing (unsexy) clinical infrastructure (natural history studies, clinical outcome measures, patient registry & communications, clinical network). Good news - can partner for all.
  - Appealing to the “ripple effect” (benefits for other diseases). For PD, HD, AD, ALS, MELAS, LHON, Leigh, DMD, stroke, diabetes, etc.



## The Fruit is Being Picked

- **Matchmaking:**
  - Companies with discovery scientists
  - Companies with Government agencies
  - Companies with other advocacy organizations
- **Contractual Arrangements (varied, some evergreen)**
  - Research grants with some rights & return
  - Investments with some rights and potential ROI
  - Intellectual Property (we do not hold, some do)
- **In sum, Companies are coming to pick the fruit because advocacy organizations can save them time and money.**
- **Preclinical Valley of Death, Pricing, reimbursement, expanded access, remain difficult issues.**
- **Times are tough for small and mid-size pharma. Very few drugs being approved. They need us, too, especially for R&D. Increasing pressure from investors for return on investment – commercialize, sell products, end R&D.**



## The Fruit is Being Picked

- Advocacy orgs doing all above, awarding grants to & investing in pharmas, we call Joint-Venture Philanthropy.
- Case study – small, start-up pharma visited; had small molecules to improve mitochondrial function in FA; asked which FA scientists should test them; we introduced to our best cell assay; scientist reported one molecule performed many fold better than any on his assay; pharma needed money for pre-clinical development; we submitted joint application to NIH RAID; first rare disease successful application; FARA awarded \$3.5M research grant & invested \$1.1M; Phase I in healthies; Phase II in FA patients showed clinically significant benefit in just 28 days; eager to begin Phase III. Introduced to PRIP (Yukiko); MELAS study soon.
- Case study – We worked with Takeda & Santhera to do Phase I and II at NIH; for Phase III, Santhera spends \$1M to recruit, FARA recruits all but one patient.
- Results to date are good. No treatment yet, but close.

ViroPharma

Intellect  
Neurosciences

edison

Penwest

Takeda

santhera  
Pharmaceuticals

FARA | Friedrich's  
Ataxia  
Research  
Alliance

# FA Treatment Pipeline - 2004

Available to  
Patients

**1 Clinical Trial**  
**3 Potential Treatments/Approaches**

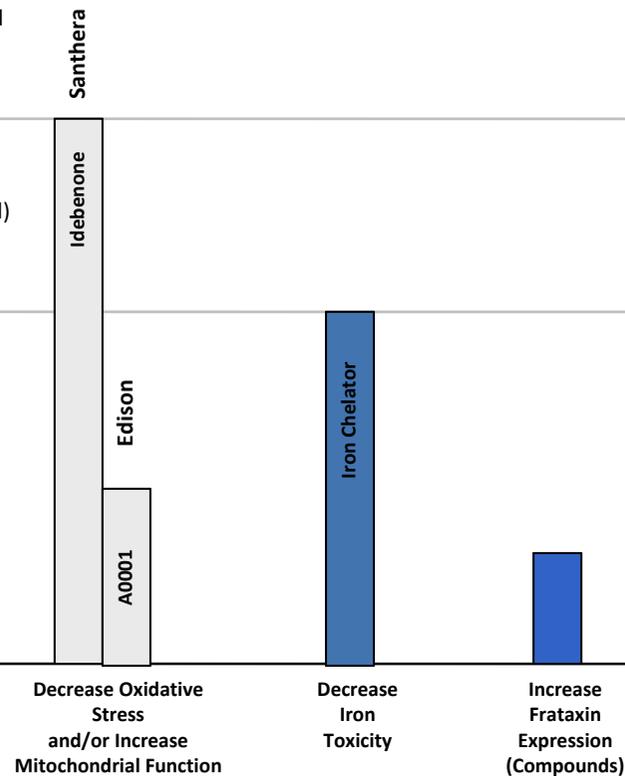
**Phase III**  
(Definitive Trial)

**Phase II**  
(Human Safety and  
Efficacy Trial)

**Phase I**  
(Human Safety Trial)

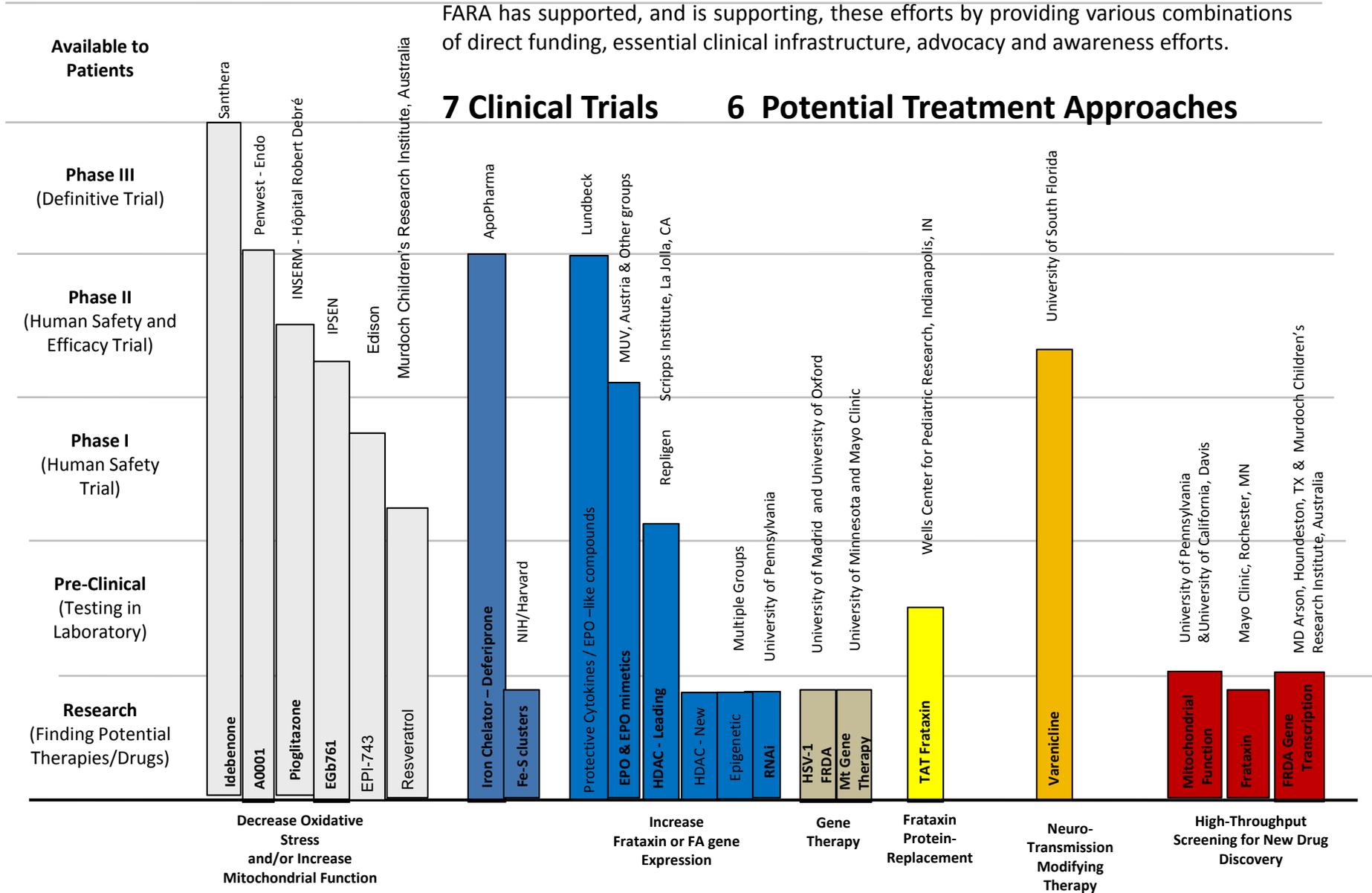
**Pre-Clinical**  
(Testing in  
Laboratory)

**Research**  
(Finding Potential  
Therapies/Drugs)



# FA Treatment Pipeline - 2012

FARA has supported, and is supporting, these efforts by providing various combinations of direct funding, essential clinical infrastructure, advocacy and awareness efforts.



# We are all in this together!

---

- Together, we are not rare.
- We are all partners in a joint venture to treat and cure our patients. Together, we are making a difference.
- Thank you, ICORD and PRIP, for bringing us all together to advance that mission.



**PRIP** *Tokyo*

**FARA** | Friedrich's  
Ataxia  
Research  
Alliance