FA Drug Development

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Disclaimers

The information is for informational use only.

NAF and Reata encourage all attendees to consult with their healthcare provider for medical advice.

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Friedreich’s Ataxia: Ultra-Rare, Progressive, Neuromuscular Disease

• Ultra-rare genetic disease
  An estimated 5,000 patients are diagnosed in the U.S.\(^5\)

• Relentlessly progressive loss of motor function
  - Typically diagnosed in teens\(^6\), requires mobility aids in twenties\(^7\), mean survival is mid-thirties\(^8\)

• No approved therapies before now

FA Community has Worked Diligently to Facilitate Drug Development

- Better understand mechanism and course of disease
- Support research & development through grants and funding
- Develop clinically meaningful endpoints
- Design robust, viable clinical trials
- Consult with FDA on regulatory path
Developing a New Drug May Take ~12-15 Years

Research & Development
- For every 20,000 to 30,000 drug compounds tested, only 1 is eventually approved

Preclinical / Animal Studies
- Initial lab testing to gather information on indicators of activity, safety & dosing

Clinical Trials
- Phase 1, 2 & 3 to evaluate dosing, safety & efficacy
- Only 10-20% of drug compounds that enter trials are approved by FDA

Approval
- Formal New Drug Application (NDA) submission & review are mandatory before FDA approval may be granted
- Review timelines range from 6-12 months

Friedreich’s Ataxia Research Alliance - Participation in Clinical Trials (curefa.org)
Reata’s Journey From Bench to FDA Approval

- **Early R&D**
  - Initiated in 2014
  - Role of Nrf2 in FA proposed by advocacy group & FA caregiver
  - Reata conducts preclinical studies

- **Clinical Trial Endpoint**
  - KOLs, FARA, & Reata partner on mFARS use in clinical trials
  - FDA consult on study design

- **MOXie Clinical Studies**
  - Positive pivotal data!
  - FDA requires more data before New Drug Application (NDA) can be submitted

- **FA Community Petition**
  - 74,000 global signatures advocating for FDA review & approval
  - 35,000 from the US
  - 2,000 from patients

- **FDA Priority Review**
  - Add’t data submitted
  - Priority Review granted
  - Advisory Committee meeting canceled

- **FDA Approved!**
  - Reata received FDA approval for the U.S. on Feb 28, 2023
  - Rare Disease Day
  - 1st & Only Treatment for FA

Reata's Journey From Bench to FDA Approval
Visit Us at Booth #1
Sign Up at www.SKYCLARYS.com to Learn More

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BOOTH #1
Patients’ ROLE in drug development: Be a part of research!

- Participate in clinical trials for investigational drug compounds
- Partake in a natural history study or patient registry
- Engage with patient advocacy groups
- Contact your healthcare provider to learn more
We thank FA patients, their caregivers, advocacy & all