

Patient Focused Drug Development

The Journey of the National PKU Alliance



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National PKU Alliance

- Formed in 2008
- Improve the lives of individuals with PKU and pursue a cure
- Created by 18 state and regional support groups across the country
- Central repository for research funds and create a unified voice for PKU in public policy

NPKUA Vision

- Help change the future of PKU – how it will be known, how it will be experienced and how it will be cured



Initial Success

- \$1.3 million in research grants
- Three sold out patient conferences – largest gathering of PKU patients in the world
- Voice at the national table
- Education materials, mentoring programs, emergency assistance to pregnant patients

NPKUA Strategic Thinking

- Go beyond funding open research
- Active participant in development of next generation of therapies



Strategic Thinking – Understanding the Science

- Science and Status of PKU
- Phone interviews with 10-15 key medical leaders to gain a comprehensive view
- 10 Questions
- Identify existing gaps and consensus
- End result: white paper/scientific poster



Strategic Thinking – Understanding the Patient Community

- Web based survey
- Current treatment status
- What's the most important in new treatments – symptoms and lifestyle changes
- Risks willing to take
- Distributed to researchers and industry
- Inform our research priorities



International Scientific Exchange Session – July 2014

- 13 lead investigators
- Liver cell transplantation, gene therapy, and PKU brain function
- In and outside of PKU
- Share research, discuss challenges, encourage collaboration
- Increase knowledge base
- Held in conjunction with patient conference

Crowd Sourcing – International Research Challenge to Develop Home Blood Test for Optimal Management



- 128 submissions
- Expert review panels
- Initially funded 8 solvers
- 3 Finalists
- Benchmark based
- Testing of prototypes April/ May

FDA Public Meetings

- FDA commitment to PFDD
- 20 disease specific meetings 2013-2017
- Impact of disease on patients, spectrum of severity, measures of benefit that matter most to patients, adequacy of existing treatments
- June 2014 meeting



Industry Partnerships



- Patient focus groups before our conference
- Patient tour of facilities
- Clinical trial info
- Annual meeting
- Meeting challenges together
- Board representation

Research Program

- Impact of funding
- Ownership of intellectual property
- Open ended RFP vs. specific RFP
- New treatment in the FDA development pipeline in the next five years



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