

Ataxia Research Update - 2009

NAF Annual Meeting, Seattle

Harry Orr, Chair MRAB



Basic Science of Ataxia

- Gene discoveries
- Disease pathways

Translational Research

- Novel therapeutic approaches

Patient/Clinical Research

- Clinical trials
- 

Expectations for Ataxia Research 2009

Expand the Probability of Treatments

Basic research:

Identify additional pathways to disease.

Additional ataxia genes.

Translational Research

More drug targets/approaches for ataxias.

Clinical Research

Drug trials for ataxias.

Basic Science of Ataxia

- **Polyglutamine-based ataxias,
Henry Paulson, Saturday, 1:30 pm**



GO BLUE

Translational Research

- DNA structure & FRA therapy
Marek Napierala, 1:00 pm Saturday**
- RNA-based therapy
Ryan Boudreau, Sunday, 10:30 am**

Patient/Clinical Research

- SCA compound multicenter trial,
Theresa Zesiewicz, 7:00 pm, Friday**
- Update FRA clinical trials,
David Lynch, 9:00 am Sunday**
- Update SCA1 lithium trial,
Grisel Lopez, 11:00 am Sunday**

Ataxia Research Support - NAF Partnerships

FARA

Kyle Bryant FRA Translational Research Awards

Bob Allison Ataxia Center (Minneapolis)

SCA compound trial

Ataxia Research Support - NIH

Last eight years NIH budget was flat, no increases

**Next Two Years NIH - American Recovery
& Reinvestment Act of 2009 (ARRA)**

Additional \$8.2 billion in research funding over two years

\$1 Million - Two Year Challenge Grants

Application Due Date(s): April 27, 2009

Peer Review Date(s): June/July 2009

Earliest Anticipated Start Date(s): September 30, 2009

(01) Behavior, Behavioral Change and Prevention

(02) Bioethics

(03) Biomarker Discovery and Validation

(04) Clinical Research

(05) Comparative Effectiveness Research

(06) Enabling Technologies

(07) Enhancing Clinical Trials

(08) Genomics

(09) Health Disparities

(10) Information Technology for Processing Health Care Data

(11) Regenerative Medicine

(12) Science, Technology, Engineering and Mathematics Education

(13) Smart Biomaterials – Theranostics

(14) Stem Cells

(15) Translational Science

(15) Translational Science

**15-OD(ORDR)-101* Pilot projects for prevention,
Early detection & treatment of rare diseases.**

Research projects in rare diseases could include:

**identification of molecular targets for rare diseases;
development of models of disease;
screening or detection of rare diseases;
development of tools for drug discovery;
and clinical trials.**

Ataxia Research Support - NAF

NAF distributed for ataxia research -

in 2007 - \$844,310

in 2008 - \$960,862

Committed to date in 2009 - \$484,000

Ataxia Research Support - NAF

Committed in 2009 - \$484,000

48 Applications Received

28 Expert Reviewers

9 Applications Funded by the NAF Board

If you are able to donate for Ataxia research consider donating to the general ataxia research fund.

**Thank You
Ataxia Patients, Families
and Friends**