The Association for
Frontotemporal Degeneration
Opening the gateway to help and a cure

Understanding FTD Clinical Trials

Continuing Education Training

FTD Support Group Facilitators

May 13, 2013

Our Shared Vision

We envision a world where frontotemporal degeneration is understood, effectively diagnosed, treated, cured and ultimately prevented.

Understanding FTD Clinical Trials

Susan Dickinson, MS, CGC
Executive Director
AFTD

Jill Shapira, PhD, RN
Nurse Practitioner
UCLA Neurobehavior Program

Disclosures

Relevant financial and non-financial relationships

Susan Dickinson, MS, CGC – no disclosures

Jill Shapira, PhD, RN – no disclosures

The Association for
Frontotemporal Degeneration

Opening the gateway to help and a cure

Emerging Focus:
FTD Clinical Trials

Drug Development

Only one out of every 10,000 compounds investigated by American pharma companies is approved for patient use by the FDA.

Average investment:

- **15 years**
- **> $1 billion**

--PhaRMA

Challenges

- **Drug Development** is expensive with low prospect of success
- **Rare disease** = Small market for a drug
- **Neurodegenerative diseases** --Currently NO approved disease-modifying treatments.
- **FTD**
  - Various clinical presentations
  - 3 different pathologies
  - No biomarkers (definitive dx only at autopsy)

But…we DO have some Assets!

- Explosion in FTD research
- Overlap with Alzheimer's and ALS
- Orphan Drug Act
- Passionate and dedicated community

Our Goal Today

To Educate about clinical trials and research participation

To Empower patients and families to be full partners in the research process

Participating in FTD Clinical Trials

AFTD Continuing Education
May 13, 2013

Jill Shapira, RN, PhD

Appreciation

For all our patients and families

Who shared their research experiences with us

Experiences with FTD Clinical Trials

- *FTD: A Basis for Clinical Trials*
  - Knopman, Mayo Minnesota (2004-2008)
  - Developed methods for clinical trials
- *Comparing Namenda to Placebo*
  - Boxer, UCSF and Forest Pharm (2008-2012)
  - Namenda not helpful in bvFTD or SD
- *TauRx0237: Safety and Efficacy*
  - TauRx Therapeutics International (2013)

Evaluating Research Projects:
Is this a good study for "us"?

EDUCATE

Obtain information through process of informed consent

EMPOWER

Consider *your* unique situation

- Rewards and challenges
- Wishes of patients *and* families

Informed Consent

- Provides information about study
- Details potential risks and benefits
- Describes research procedures
- States research is voluntary
- Includes consent of study partner
- Encourages discussion with others

Capacity to Consent
Who signs the consent form?

- Assess FTD participant's understanding
- Evaluate decision-making ability
- Obtain surrogate consent as necessary
  - *Agent in advanced health care directive*
  - *Spouse*
  - *Domestic partner*
  - *Adult son or daughter*
  - *Custodial parent*
  - *Adult brother or sister*
  - *Other relative*

Study Introduction

Why is this study being done?

- Support future research
- Better understand FTD
- Discover if drug is safe and useful for FTD

How is a drug study designed?

- Placebo-controlled: receive study drug or placebo
- Randomized: assigned by chance
- Double-blind: for research team, patient & families
- Continuation study?

Screening:
Do "we" match the study requirements?

- Establish type and stage of FTD
- Exclude other medical conditions
  - Prevent masking effectiveness of drug
  - Protect from side effects
- Assess ability to complete procedures
- Evaluate study partner readiness
  - Monitor drug intake and side effects
  - Keep study appointments

Description of Procedures
Is the study right for you?

- Cognitive testing
  - Anxiety, boredom, fatigue, inability to sit still
- Brain scans
  - Noise, enclosed space
- Blood tests
  - Concern about needles
- Transportation issues
  - Traffic, comfort with automobile trips
- Time commitment

Study Risks or Discomforts

- *From study drug*
  - ✓ Likely, less likely, rare but serious
  - ✓ Reproductive risks; contraception required
- *Randomization:* drug not helpful vs. more side effects
- *Placebo:* worsening of condition
- *Identification of elder abuse*: report to authorities
- *Psychological:* feelings of sadness about disease
- *Confidentiality:* identifiable personal information

Are there benefits if you participate?

- Possible benefits to study participants
  - Improvement in FTD symptoms
  - But symptoms could get worse
- Possible benefits to others or society
  - Help researchers learn more about FTD
  - Help future FTD patients

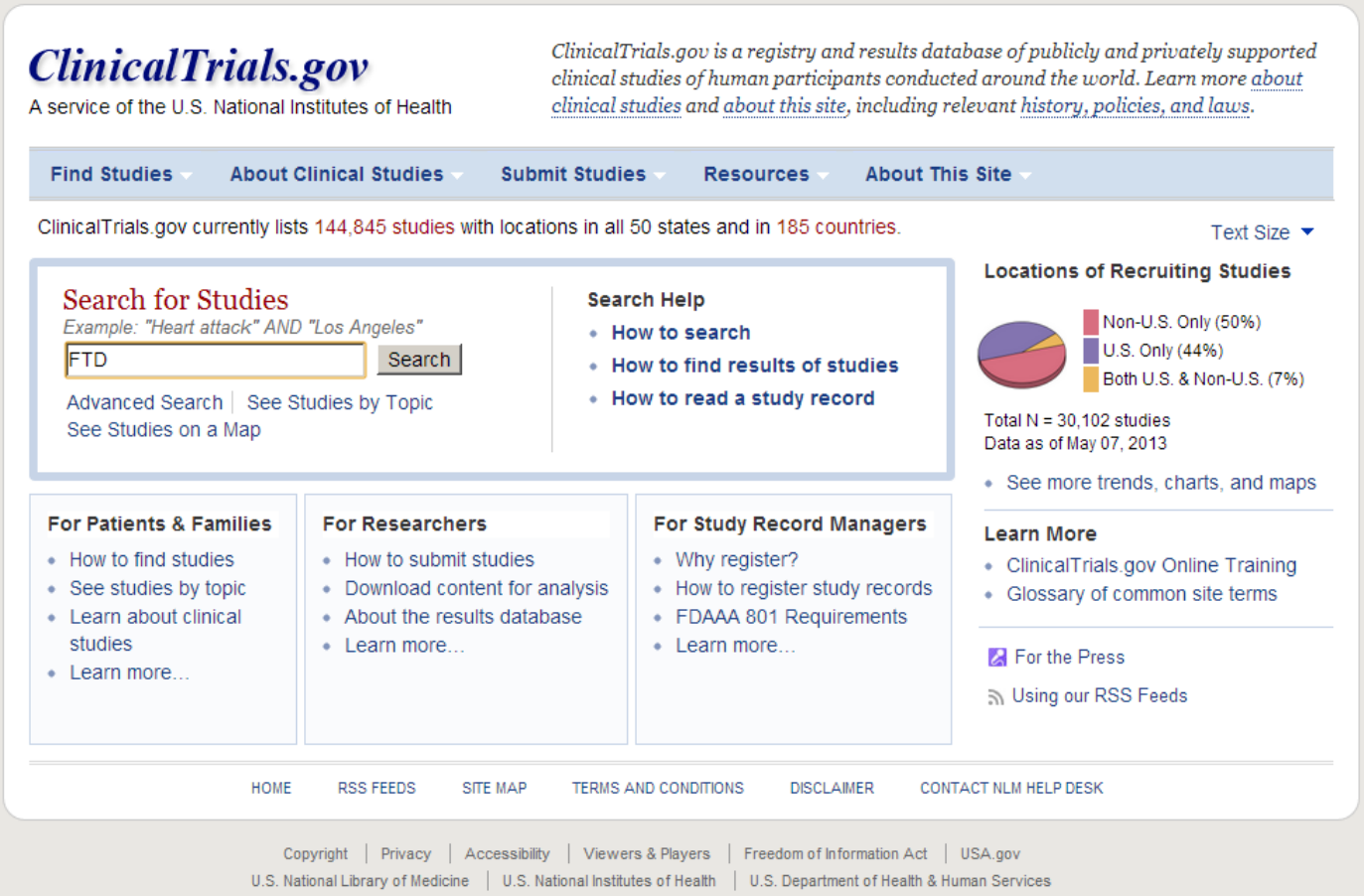
What are your choices if you decide not to participate in the drug study?

- Receive no experimental treatment now
- Receive standard treatment for FTD
  - Currently no available medicines to slow disease
  - Medicines and strategies to control behavior
- Take part in another study when available

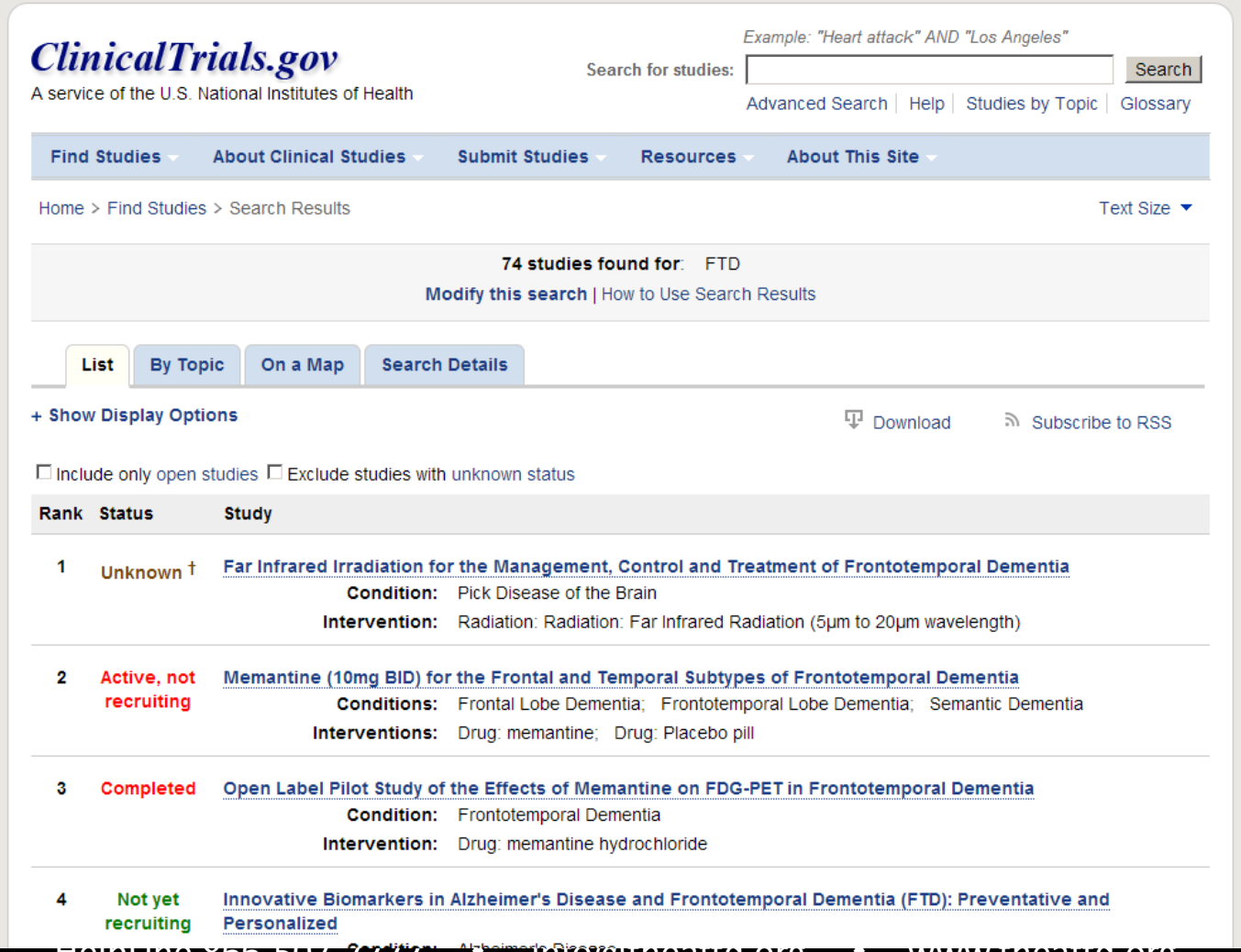
So, is the study right for "us"? Educate and Empower

- Consider *your* situation and challenges
  - Respect wishes of patient and family member
  - Consider risk, effort, emotional response
- Balance with potential rewards
  - Improve symptoms
  - Help others
  - Relationship with FTD research team
- There are multiple ways to further research!
  - We are ALL working together
  - Support AFTD effort to emphasize our numbers to pharmaceutical companies

www.clinicaltrials.gov



www.clinicaltrials.gov Search: "FTD"



ClinicalTrials.gov
A service of the U.S. National Institutes of Health

Example: "Heart attack" AND "Los Angeles"
Search for studies: [ ] Search
Advanced Search | Help | Studies by Topic | Glossary

Find Studies | About Clinical Studies | Submit Studies | Resources | About This Site

Home > Find Studies > Search Results Text Size

74 studies found for: FTD
Modify this search | How to Use Search Results

List | By Topic | On a Map | Search Details

+ Show Display Options Download Subscribe to RSS

☐ Include only open studies ☐ Exclude studies with unknown status

Rank	Status	Study
1	Unknown †	Far Infrared Irradiation for the Management, Control and Treatment of Frontotemporal Dementia **Condition:** Pick Disease of the Brain **Intervention:** Radiation: Radiation: Far Infrared Radiation (5μm to 20μm wavelength)
2	Active, not recruiting	Memantine (10mg BID) for the Frontal and Temporal Subtypes of Frontotemporal Dementia **Conditions:** Frontal Lobe Dementia; Frontotemporal Lobe Dementia; Semantic Dementia **Interventions:** Drug: memantine; Drug: Placebo pill
3	Completed	Open Label Pilot Study of the Effects of Memantine on FDG-PET in Frontotemporal Dementia **Condition:** Frontotemporal Dementia **Intervention:** Drug: memantine hydrochloride
4	Not yet recruiting	Innovative Biomarkers in Alzheimer's Disease and Frontotemporal Dementia (FTD): Preventative and Personalized **Condition:** Alzheimer's Disease

Info on clinicaltrials.gov

- Purpose (Phase I, II, III)
- Study Type (Interventional, Observational)
- Study Design (Blinded, Placebo controlled)
- Endpoints
- Number of enrollees
- Est. Start Date, Est. Completion Date
- Patient Eligibility (age, diagnosis, sex)
  - Inclusion criteria
  - Exclusion criteria
- Most recent update of this trial on clinicaltrials.gov

Q & A Session

Susan Dickinson, MS, CGC
Executive Director
AFTD

Jill Shapira, PhD, RN
Nurse Practitioner
UCLA Neurobehavior Program

Future Web Training

This training will be archived on AFTD's website.

Future training for group leaders, family caregivers and professionals will be offered in webinar format.

Watch your email and AFTD's newsletter for more!

Thank you for all you do to support AFTD's mission!

For More Information

The Association for Frontotemporal Degeneration
Radnor Station Building 2, Suite 320
290 King of Prussia Road
Radnor, PA 19087
267.514.7221 / 866.507.7222

www.theaftd.org
info@theaftd.org