

**BLANCHETTE ROCKEFELLER**  

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**NEUROSCIENCES**  
**INSTITUTE**  
at West Virginia University.

**Fifth Annual West Virginia Biosciences Summit**  
**April 21, 2015**  
**Shana Kay Phares, CEO**

# In Memory

- Founded in 1999 by the Rockefeller Family in memory of Blanchette Hooker Ferry Rockefeller
- We are a unique, independent, non-profit institute dedicated to the study of memory and memory disorders



# BRNI's Efforts to Address Alzheimer's

- Alzheimer's Outreach and Registry Program (AORP)
- Diagnostics
- Therapeutics



# AORP CME Sessions

- Alzheimer's Disease Update for Physicians
- Diagnosing and Treating Alzheimer's Disease: The Latest Developments
- Behavioral and Psychological Symptoms in Dementia
- Palliative Care in Dementia
- Certification in Alzheimer's/Dementia Care



# BRNI is a hybrid organization

- Rockville lab is testing an Alzheimer's diagnostic
- Morgantown lab has two distinct units:
  - NIH-funded basic research lab
  - Institute-funded translational research lab



# Licensure Agreement

- October 2013 BRNI entered into a licensing agreement with Neurotrope Biosciences (OTCQB: NTRP), a public company
- License agreement covers diagnostic and therapeutic patents
- License agreement provided funding required to conduct human clinical trials
- BRNI involved in trial design and provides research services in support of the BRNI-licensed technology



# Therapeutic Clinical Trials

- Phase 2a safety study completed December 2014
- Phase 2b multi-site, double-blind, placebo controlled trial with 150 patients planned
- Compassionate use treatment ongoing – 1 active, 3 in the pipeline



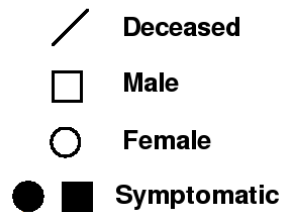
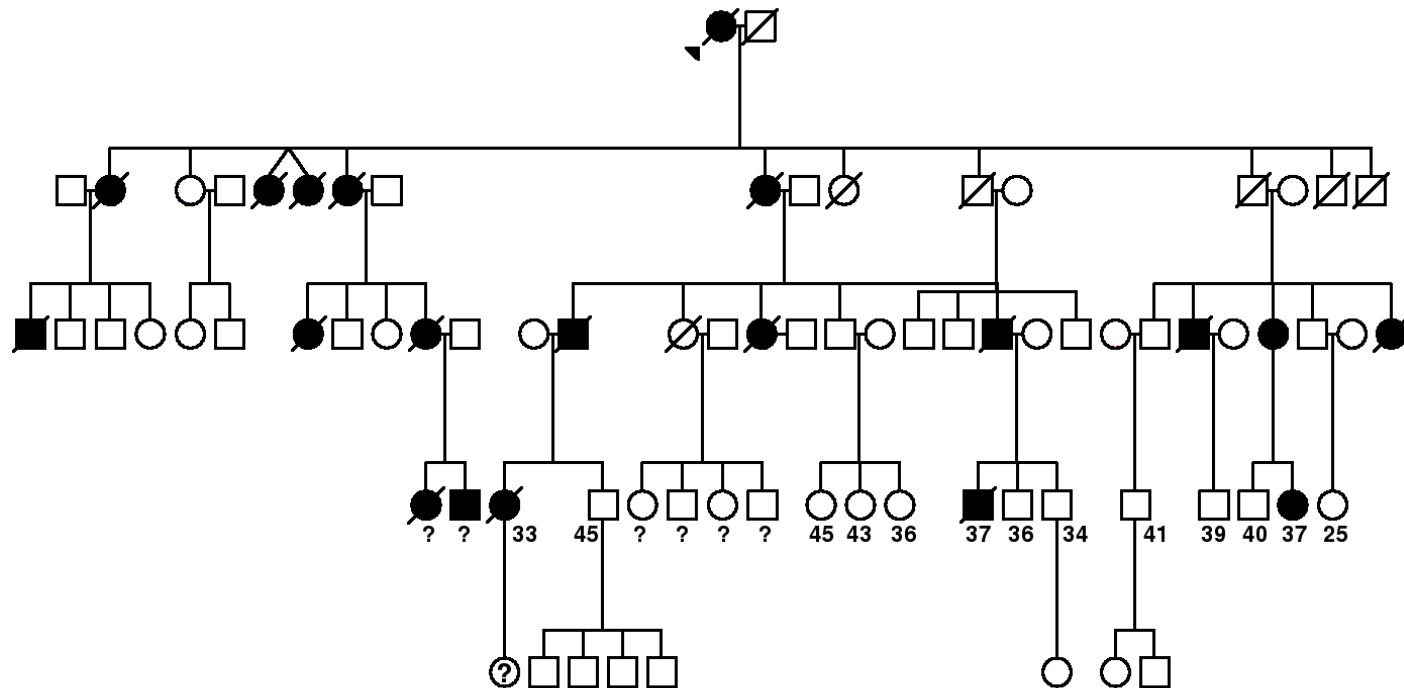
# Update on Compassionate Use Trial

- Family contacts BRNI and initial conversations reveal an unusual form of dementia
- Pedigree reveals a genetic dementia which is autosomal dominant.
- Treatment from April to September 2013 showed promising results
- "PSEN1 Variant in a Family with Atypical AD" appears in the Journal of Alzheimer's Disease March 2015





# Family Pedigree



# Blood-Brain Barrier Update

- BRNI devised a novel carrier system to transport molecules such as adriamycin and tetracycline – into the brain
- Hired Senior Commercial Advisor
- Submitted business plan to venture capital organization



# A Word about Growing Pains

- Licensing agreements require the melding of business practices and objectives with research practices and objectives
- Patent prosecution must add value to your organization – just because it is patentable doesn't mean its saleable or defensible



# Further conversation

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