Welcome to today’s webinar

- We will begin the webinar at 2:00 p.m. Eastern
- All attendee lines are automatically muted.
- This webinar is being recorded and will be posted on LBDA’s Youtube page.
- Throughout the event, please submit your questions using the “Q&A” option.
How to Utilize the LBDA Research Centers of Excellence (RCOE) Program

Dave Irwin, MD
University of Pennsylvania
Co-Director, LBDA Research Center of Excellence
Webinar Outline

• Review goals, structure and progress of LBDA Research Centers of Excellence Program

• Discuss general aspects to participation in clinical research and clinical trials.
LBDA Research Centers of Excellence (RCOE)

• 25 Centers Across the US
  • Established through an application and peer-review chosen by committee and announced in October of 2017.

• GOALS
  • Increase access to high quality clinical care over the course of LBD.
  • Increase knowledge of LBD among medical and allied (or other) professionals.
  • Establish a clinical trials-ready network of LBD centers.
  • Create infrastructure and resources to advance LBD research toward the overall goal of development of new symptomatic and disease-modifying therapeutics.
Goal: Increase access to high quality clinical care over the course of LBD.

• It is not uncommon for LBD diagnosis to take several years to establish with multiple visits across different specialists and subspecialists despite being a common cause of dementia.

• Delay in diagnosis results in increased caregiver/patient burden and morbidity.

Galvin et al, Alzheimer Dis Assoc Disord. 2010
Goal: Increase access to high quality clinical care over the course of LBD.

- LBD IS A MULTI-SYSTEM DISEASE
- Coordinated care among health care professionals (e.g. PCP, neurologist, physical therapist).

- REM Sleep Behavior Disorder
- Orthostatic Hypotension
- Constipation
- Agitation
- Motor Parkinsonism
- Dementia
- Cognitive Fluctuations/Excessive Daytime Sleepiness
- Delusions
- Depression/Anxiety
- Visual Hallucinations
Goal: Increase knowledge of LBD among medical and allied (or other) professionals.

• More efficient referral of LBD patients to neurologist for more rapid diagnosis.
• Improve care for LBD patients in emergency settings and other settings where treatment is from non-specialist.
• Improve symptom management of LBD by all treating health care providers.
Goal: Establish a clinical trials-ready network of LBD centers.

• Improve access for patients seeking therapeutic trials.
• Stimulate industry to invest in LBD therapeutic development by demonstrating feasibility of conducting trial.
• Improve quality of LBD therapeutic trials conducted by centers with experience in patient population.
Goal: Create infrastructure and resources to advance LBD research toward the overall goal of development of new symptomatic and disease-modifying therapeutics.

- Researchers and institutions *combing efforts to accelerate discovery*.
- Engage with industry to *stimulate interest in LBD trials* and advise trial design for higher-likelihood for success.
- Allow data/biospecimens collected for therapeutic trials to be used by research community for *further knowledge outside of trial goals*. 
The RCOE Network

25 centers were selected

https://www.lbda.org/rcoe
Penn LBDA Research Center of Excellence

Penn FTDC
David Irwin, MD
Cognitive specialists, clinical nurses, genetic counselor, neuropsychologist, researchers, social workers and research coordinators.

Penn PD&MDC
Andrew Siderowf, MD
Movement Disorders specialists, clinical nurses, research assistants, social workers, counselling program and The Dan Aaron Center Parkinson Rehabilitation Center.

Padrecc
John Duda, MD
Comprehensive multi-disciplinary clinic with a neuropsychologist, a geriatric psychiatrist and a social worker to help care for Veterans with LBD.
LBDA RCOE Investigator Meeting

First Annual Meeting on December 14th 2017 with 4 work-groups:

1. Clinical Care and Professional Education
2. Community Education and Support
3. Clinical Trial Design & Optimization
4. Industry Engagement
LBDA RCOE Survey
Clinical Patients

<table>
<thead>
<tr>
<th></th>
<th>DLB</th>
<th>PDD</th>
</tr>
</thead>
<tbody>
<tr>
<td>Annual New</td>
<td>1361</td>
<td>1288</td>
</tr>
<tr>
<td>Annual Return</td>
<td>~3,000</td>
<td>~4,500</td>
</tr>
</tbody>
</table>
LBDA RCOE Survey
Clinical Trials Experience

• 18/24 (~80%) sites active in ≥ 5 clinical trials in last year
• 21/24 (88%) sites active in ≥ 2 clinical trials in past year
Clinical Care and Professional Education WG

• Important **clinical milestones in LBD management** identified
  • Referrals to allied health therapies and utilization of community resources.

• **Increasing awareness** and disseminating LBD knowledge to healthcare professionals remains a critical priority.
  • “Toolkit” for PCPs and other health care professionals for LBD care.

• Encouraging **LBD education events at RCOE sites**.
Clinical Care and Professional Education

Early Diagnosis:
The Road to Better Therapies and LBD Care

Friday, May 4, 2018 • Philadelphia, PA

• Videos of presentations will be available at: www.LBDA.org
Community Education and Support WG

• Improving access and quality for caregiver support groups at RCOE sites based on caregiver feedback.
• Most sites favored targeting caregivers and/or early-stage patients with LBD.
• Trained facilitators were described as essential.
• Sources of patient/family education for LBDA RCOE sites
  • LBA-produced materials
  • LBD booklet from the National Institutes of Health
  • Materials produced by the individual sites.
Clinical Trial Design / Optimization WG

- Engage Caregivers *(surveys, networks)*
- Comprehensively address current care challenges
- Develop clinically meaningful LBD *outcome measures*
- Account for unique aspects of LBD
- Facilitate academic and industry partnerships for biomarker development / validation
Biomarkers: Overall Goals

1) **Diagnostic Use**
   1) Early Diagnosis (MCI-DLB/prodromal DLB)
   2) Differentiation from AD, FTLD, VASC
   3) DLB subgroup stratification

2) **Prognostic Use**
   1) Track disease progression
   2) Target engagement (disease modifying therapies)
Industry Engagement WG

• **Central resource** for exploring collaborations between the RCOE and industry on LBD clinical trials.
  • Determine requirements of data sharing and publication plans
  • Optimization of protocols

• **Centralized data resource** on the clinical trial capabilities of all RCOE sites.
  • Facilitate clinical research collaboration among RCOEs and expedite site selection for industry-sponsored trials.

• Guidance on **establishing expert panels to advise clinical trial sponsors.**
Webinar Outline

• Review goals, structure and progress of LBDA Research Centers of Excellence Program

• Discuss general aspects to participation in clinical research and clinical trials.
What is research?

• **Research**—A study done to answer a question.

• **Clinical Research**—Studies in which people participate as patients or healthy volunteers.

• Scientists do research when they’re not sure what will work best to help people with an illness.

• Other terms for clinical research are “clinical trial,” “protocol,” “study,” and “experiment.”

From: fda.gov
Definitions: Types of Research Studies

• Observational Research (Diagnostic/ Natural History Studies)
  • No intervention
  • Collection of data (Cross-Sectional vs. Longitudinal)
    • Cognitive testing
    • Blood/CSF
    • Imaging
    • Genetics
Observational Research (Natural History Studies)

Dementia with Lewy Bodies Consortium (U01)

• Longitudinal study using DLB-specific clinical assessments, blood, CSF, MRI and DATSCAN across 9 centers in US.

• Goal is to facilitate clinical trials and advance science of LBD clinical care.
Observational Research (Natural History Studies)

Targeting Lewy Body Specific Pathology Using Biomarkers (U01)

- Recruiting ethnically diverse cohort of DLB patients for annual biofluid and clinical assessments.
- Performing RNA expression experiments from DLB brain tissue to develop new biomarkers.
Observational Research (Natural History Studies)

Longitudinal Imaging Biomarkers of Disease Progression in DLB (U01)

- Enroll and follow DLB patients and perform MRI, DATSCAN, PET amyloid, PET Tau imaging.
- Provide critical tissue validation of imaging studies.
Observational Research (Natural History Studies)

- Recruiting PDD and DLB for 3 year annual cognitive follow up and PET imaging of amyloid, tau and dopamine transport system to classify patients based on biomarker profiles.
Definitions: Types of Research Studies

• Therapeutic Trials
  • Pharmacologic agents (medications)
  • Non-pharmacologic agents
    • Behavioral interventions
    • Devices
Clinical Trial Strategies for DLB

• **Potential disease modifying therapies**
  • Target biology of disease

• **Symptomatic Treatments**
  • Target symptoms of disease
    • Behavioral strategies/interventions
    • Psychiatric medications
    • Devices (e.g. non-invasive brain stimulation)
Stages of Clinical Trials

- **Lab Studies**: Several Years
- **Human Safety**: Days or Weeks
  - Tens
- **Expanded Safety**: Weeks or Months
  - Hundreds
- **Efficacy & Safety**: Several Years
  - Thousands
  - Phase III

FDA Designation for Accelerated Drug Development
National Alzheimer’s disease Prevention Act (NAPA)

Image from: http://neurosciences.ucsd.edu/
Important aspects of therapeutic trials for patients

• Most therapeutic trials are \textit{placebo-controlled} and \textit{blinded}
  • Ensure we can objectively detect a positive effect
  • Maximize the number of patients to receive active drug

• \textbf{There is a screening visit to determine eligibility}
  • Criteria to help ensure participation is safe

• \textbf{Patients may not benefit directly from some trials}
  • Important meaningful contribution which many patients/families value

• \textbf{It is important to follow closely with your physician}
  • Safety monitoring and outcome measures

[Source: www.clinicaltrials.gov/ct2/about-studies/learn]
Questions to Consider

ALL RESEARCH
• What is the purpose of the study?
• What tests and treatments (or procedures) will be involved?
• What are the risks? What are the potential benefits for participation?
• How long will the study last and how frequent are visits?
• Will I learn the results of my tests?
• What steps ensure my privacy?
• Will my expenses be reimbursed? Will I be paid?

CLINICAL TRIALS
• Can I continue my current medications?
• What are the chances that I will receive a placebo?
• Is there an open-label period? When can we expect trial results?

Adapted from: National Institute on Aging/National Institutes of Health, Participating in Alzheimer’s Research and www.LBDA.org
Where to find clinical trial information

• Your physician
• Clinical trials.gov (www.clinicaltrials.gov)
• LBDA (www.lbda.org)
• Parkinson’s Disease biomarker Program (PDBP) (https://pdbp.ninds.nih.gov)
• Alzforum (www.alzforum.org)
• Alzheimer’s Association Trial Match (https://trialmatch.alz.org/find-clinical-trials)
• Michael J. Fox Foundation (https://foxtrialfinder.michaeljfox.org/find-trials)
• EDUCATION- Disease pathogenesis, symptoms and treatment.
• SUPPORT- RCOE centers, support groups, caregiver information.
• RESEARCH- Clinical research/trials listing.
• EVENTS- Webinars, Talks, Fundraisers.
Interested in Learning More?

Visit www.lbda.org/RCOE
CONTACT US

www.LBDA.org
(404) 935-6444
lbda@lbda.org

LBDA is a 501(c)3 organization