Introduction to Clinical Research & Navigating Research Opportunities

A family-friendly perspective for learning about scientific research in Down syndrome

national down syndrome society

ndss®

WELCOME

Purpose of Today's Presentation

This session will help us:

- Understand the value of scientific research
- Learn about research directly from people who have participated in studies
- Get more comfortable with the format and tone of scientific articles
- Develop critical thinking skills around research & research participation
- Learn about some research opportunities in Down syndrome

Mission

NDSS is creating a world where individuals with Down syndrome thrive.

NDSS Core Pillars

NDSS supports and advocates for the Down syndrome community by focusing on four key pillars:

Resources & Support

Research

Advocacy & Policy

Community Engagement

Community
Engagement

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Why research in Down syndrome matters

Clinical Trials and Research in Down Syndrome

- Every drug, vaccine, and diagnostic test we take undergoes strict clinical testing for safety, quality, and effectiveness before it can be approved by the U.S. Food and Drug Administration (FDA) for use in humans.
- Clinical trials are how we get our next generation of new cures.
- We need clinical trials to see if discoveries that work in the lab, actually do what they say they're going to do in people.

What are Clinical Trials?

Clinical trials are the engine of medical progress

The primary goals are to:

- Determine whether a treatment is **safe and effective**
- Gather data on **dosage, side effects, and overall impact on health**

Clinical trials are **essential for advancing medical knowledge** and **improving patient care.**

Types of Clinical Trials

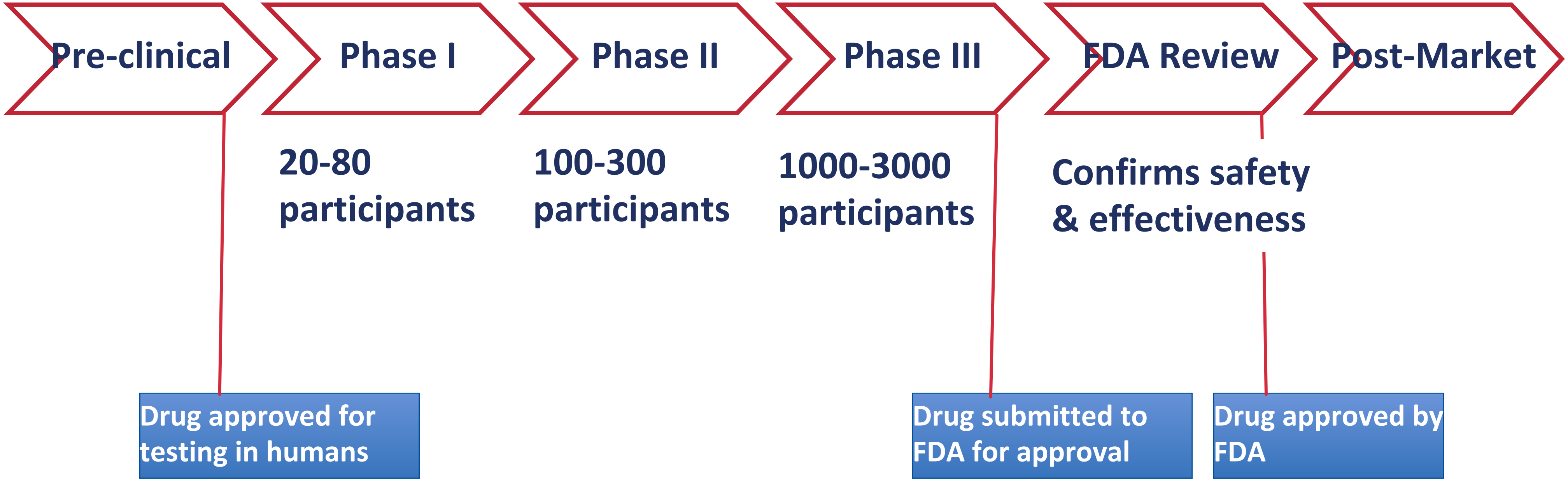
Observational

- Important data are collected over time to be used to develop future research
- No experimental medicines or treatments are involved
- Individuals who may be already taking a medicine may be tracked to see long-term health outcomes without changing their treatment plan

Interventional

- An experimental medicine ("study drug"), or in some clinical trials, a placebo, is given to the study participant to evaluate its effectiveness in treating a specific condition.

Phases of Clinical Trials



- **Pre-clinical**
- **Phase I** – 20-80 participants
- **Phase II** – 100-300 participants
- **Phase III** – 1000-3000 participants
- **FDA Review** – Confirms safety & effectiveness
- **Post-Market**

Drug approved for testing in humans

Drug submitted to FDA for approval

Drug approved by FDA

Is a clinical research study right for me?

Being an informed participant or caregiver/study partner

- **Understand the study**
- **Ask questions**
- **Make informed decisions**
- **Leave the study at any time**
- **Seek second opinions**
- **Understand the costs**
- **Understand safety measures**
- The **Informed Consent** will be a great resource for you.

Becoming Comfortable with Research

Searching for Down Syndrome Clinical Research

Clinical Trials.gov

- Website and digital database of clinical studies and results
- Maintained by the National Library of Medicine https://clinicaltrials.gov/

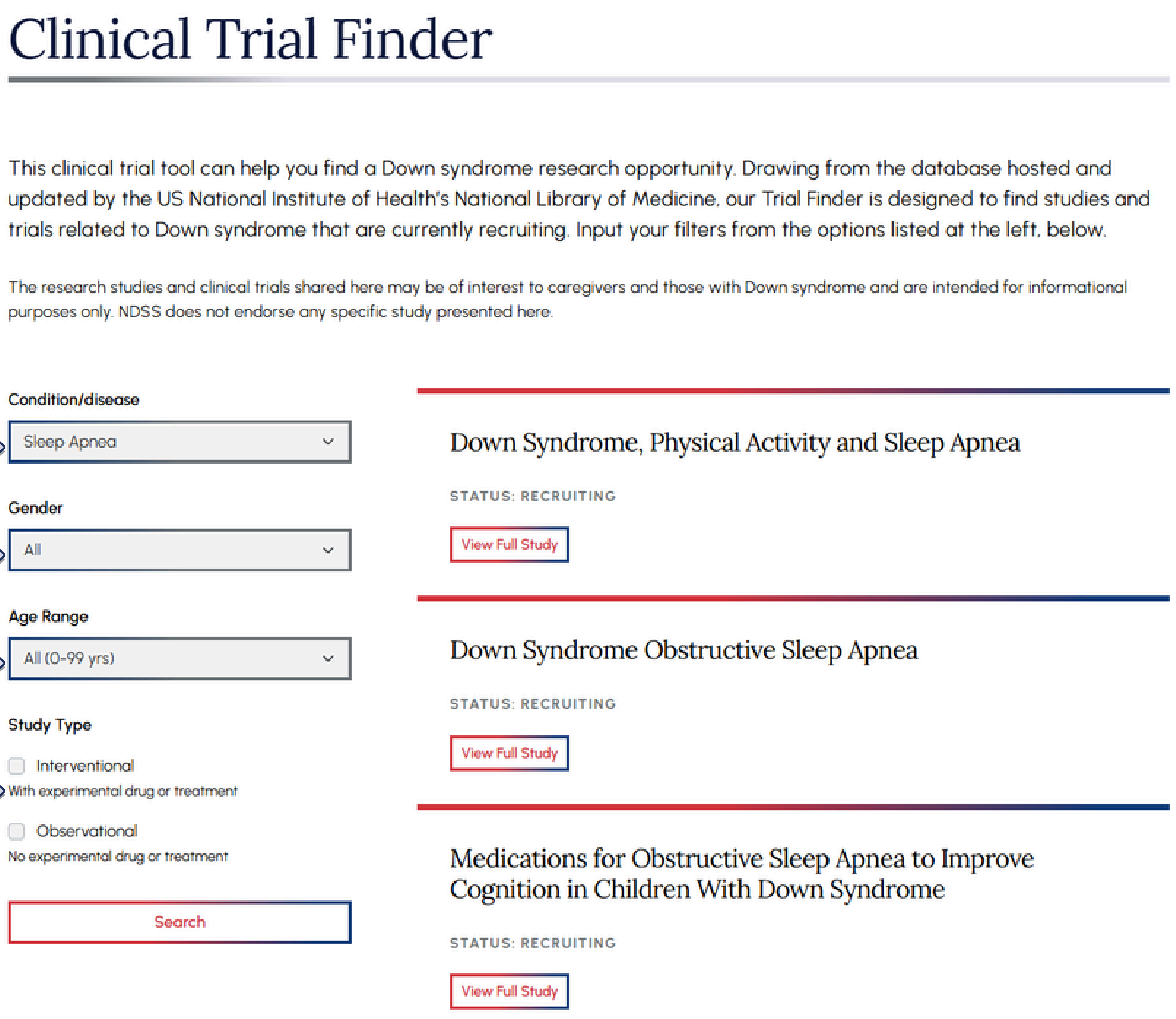
DS Connect

- NIH-funded, Down syndrome-specific registry for individuals with Down syndrome of all ages interested in participating in clinical research https://ds-connect.org/

Trial-Ready Cohort-Down Syndrome (TRC-DS)

- Program specifically for adults with Down syndrome interested in participating in research related to Alzheimer's disease. https://www.trcds.org/

Clinical Trial Finder



Clinical Trial Finder

This clinical trial tool can help you find a Down syndrome research opportunity. Drawing from the database hosted and updated by the US National Institute of Health's National Library of Medicine, our Trial Finder is designed to find studies and trials related to Down syndrome that are currently recruiting. Input your filters from the options listed at the left, below.

The research studies and clinical trials shared here may be of interest to caregivers and those with Down syndrome and are intended for informational purposes only. NDSS does not endorse any specific study presented here.

Condition/disease
Sleep Apnea

Gender
All

Age Range
All (0-99 yrs)

Study Type
☐ Interventional
With experimental drug or treatment
☐ Observational
No experimental drug or treatment

Down Syndrome, Physical Activity and Sleep Apnea

STATUS: RECRUITING

Down Syndrome Obstructive Sleep Apnea

STATUS: RECRUITING

Medications for Obstructive Sleep Apnea to Improve Cognition in Children With Down Syndrome

STATUS: RECRUITING

CONDITION/DISEASE

GENDER

AGE RANGE

STUDY TYPE

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NDSS List of Research Opportunities

Our web site lists opportunities for research participation, reviewed and listed by NDSS' Scientific and Clinical Advisory Board.

https://ndss.org/participate-research-studies

ON THIS PAGE:

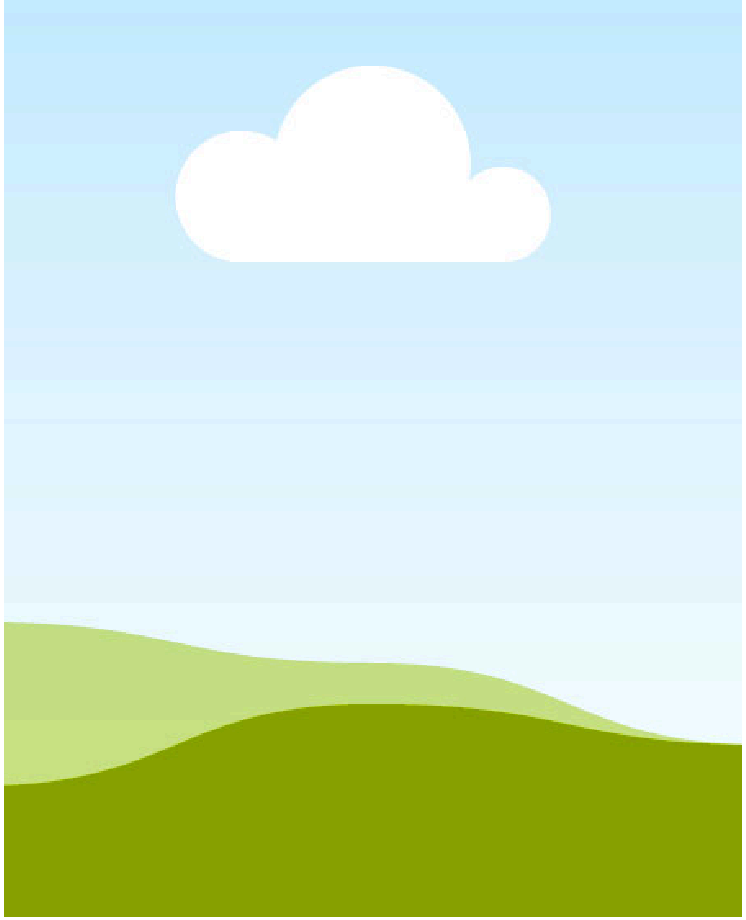
- Research Opportunities Interest Form
- Caregivers
- Birth-4 year olds
- 5-18 year olds
- 19-29 year olds
- 30+ year olds
- Submit a study

How Do I Know if a Study is “Good”?

When considering a research participation opportunity, make sure the recruitment flyer, web page, contain clear, family-friendly language and:

- The contact information and credentials of the individual who will be the main point of contact for questions
- A thorough description of the study/trial/survey
- Proof of regulatory oversight (IRB, ERB, etc.)
- Outline of participant inclusion parameters
- Timeline components of the request and any other relevant information

What Do “Family-Friendly” Materials Look Like?



Multilingualism and Down Syndrome Online Survey

IRB25-0152

We are looking for Spanish-speaking caregivers of children with Down syndrome 5 years old and younger who want to share their perspectives and experiences about raising a child in a multilingual context

What will we do?

Primary caregivers will complete a survey that will take about 30 minutes.
This can be completed over the phone, on a Zoom call, or online on your own.

Contact us!

✉ dcf2@illinois.edu
☎ (217) 265-8043
🌐 dndlab.shs.illinois.edu

The DND LAB
Development in Neurogenetic Disabilities Lab

ILLINOIS
Speech & Hearing Science
COLLEGE OF APPLIED HEALTH SCIENCES

Are you interested?

Scan the QR code and join us!

Your input will contribute to research on multilingualism and language development in Down syndrome

Connecting With Your Loved One's Doctor About Research

Come to the appointment prepared with:

- A list of your key concerns about a condition or behavior
- Recent journal or academic articles
- Research study flyers
- Your understanding of the study, its parameters and any other info you've compiled

Have you heard about this new treatment/approach for…?

Do you know anything about this research lab, clinic, researcher?

Looking at this, do you think it might be a good fit for Jane?

Can you suggest any questions I should ask when I connect with the study coordinator?

Can I leave this with you to review, and then follow up in a few days, or by email?

Talking With Self-Advocates About Research Participation

Participating in clinical research is a **complicated and personal decision**.
It can be **empowering to contribute to research**, but the activities can be a lot to handle.
It's a decision that will take **information, education**, and **conversation**.

Caregivers can start preparing for those conversations:

- Review information that has been provided and be ready to break it down
- Try to be informed about all major aspects of the study
- Use the IRB-approved tools that have been provided to you: study overview, plain language explainers, guides, etc.

Talking With Self-Advocates About Research Participation

Seek out supplemental tools if necessary. Some useful sources:

- Web page: Clinical Research Glossary from Brigham and Women's Hospital and Harvard offers clear definitions of clinical research terms.
- General Clinical Research Overview – 8-minute video: (not Down Syndrome-specific) animated overview of Clinical Research from CISCPR, a non-profit organization.
- Series of 2-minute videos: Pediatric, live-action version of "What is a Clinical Trial?" from CISCRP.
- Explaining the Concept of Placebo – 3-minute, animated video from J&J.
- NDSS "What to Expect?" Videos to prepare for medical procedures: MRI, PET, and lumbar puncture – featuring individuals with Down syndrome.

What is it like to participate in research?

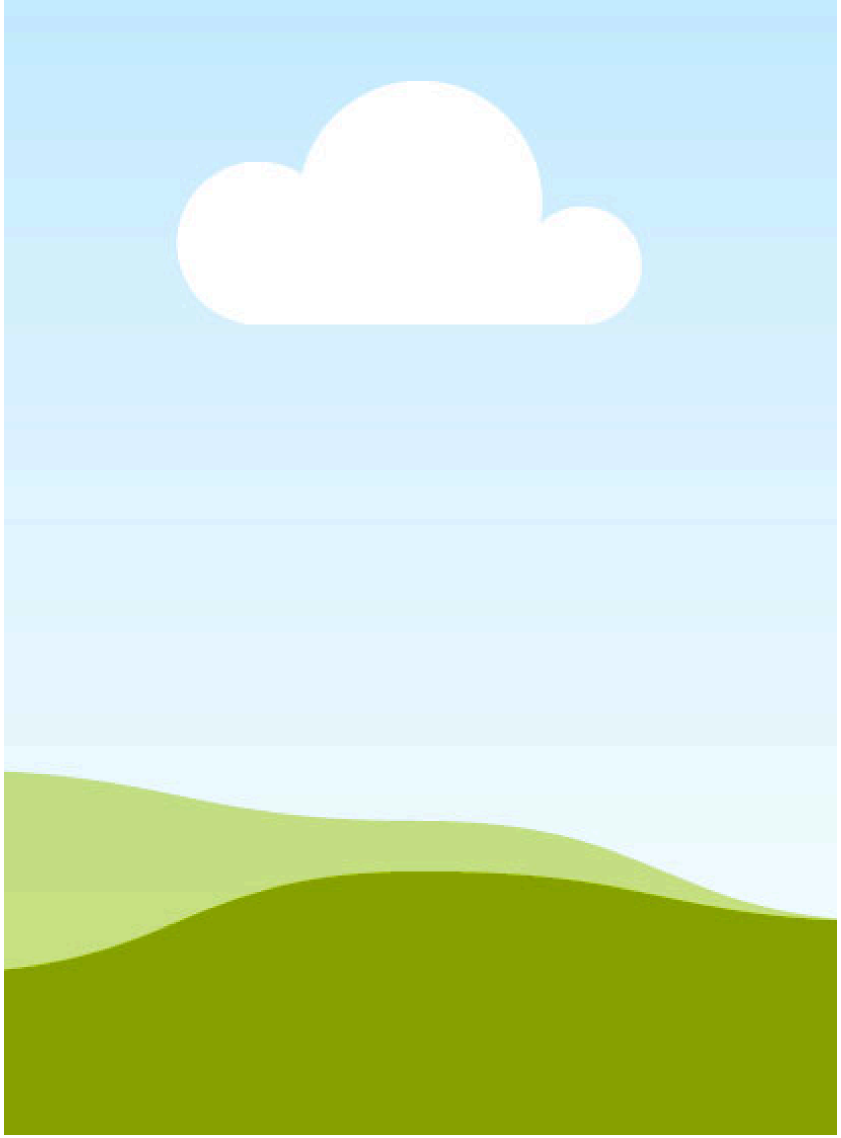
Research Voices: Susie & Dee Stufflebam

How to read a scholarly article

Where to start?

https://pubmed.ncbi.nlm.nih.gov
https://scholar.google.com

How do I know what is an "academic paper"



https://youtu.be/rOCQZ7QnoN0

Different types of articles

- Research Reports
- Literature reviews
- Theoretical articles

Anatomy of an article

- https://www.lib.ncsu.edu/tutorials/scholarly-articles

How do I read it?

DON'T READ
STRAIGHT THROUGH

- **Abstract:** Tells you briefly **what** experiment **was done** and **what was found.**
  - **Question:** What *specific results* are mentioned? Are they *relevant*?

Discussion: Summarizes important results, gives reasons for conclusions based on results.

Question: Do you *agree* with the logic of the conclusions?

Are these results *useful* to you?

If you have reviewed the abstract and discussion and you think it applies to your interests its time to dig into the introduction.

Explains motivation and importance of research, provides background information.

Question: Do you *understand* background info? Do you need to *look up references* for more info?

What and how they performed the study.

Should explain well enough that **you could replicate** the experiment yourself, if you wanted to.

Often the most complex to understand, that’s OK!

When reading, skim and **try to pick out basic methods** used. Don’t worry that much about the details!

This section provides the **data the authors use** to reach their conclusions.

Figures are often included to **make the data more compact** and intuitive, and **Tables organize data** in one place for easier reading.

Understanding Figures and Tables is **EXTREMELY** important in understanding a paper.

For figures, make sure you understand what quantities are on the axes.

Do you agree with the conclusions?

Are they supported by the results?

Are they overgeneralized?

What other factors may have influenced the findings?

What questions would you ask next? What are the next steps?

Research in Action: ABC-DS Overview

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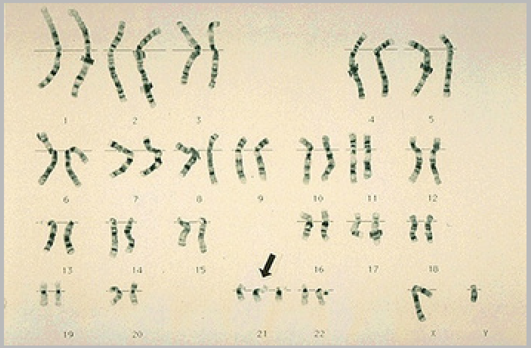
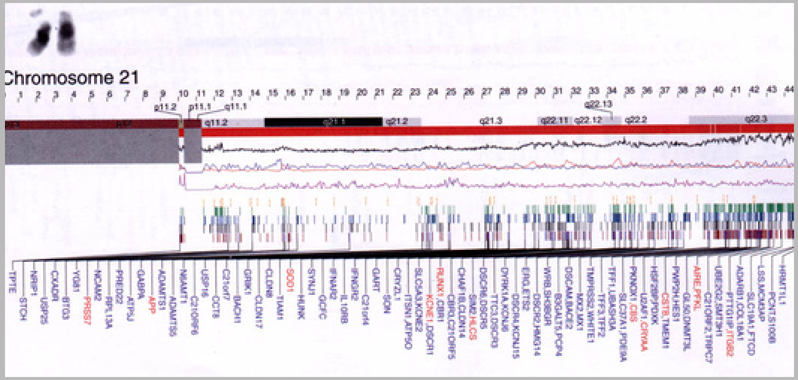
The development & progression of Alzheimer’s disease in Down syndrome

Ann D. Cohen
University of Pittsburgh School of Medicine

ABC-DS
lzheimer Biomarker Consortium-Down Syndrom

Why are people with Down syndrome vulnerable to Alzheimer’S disease?



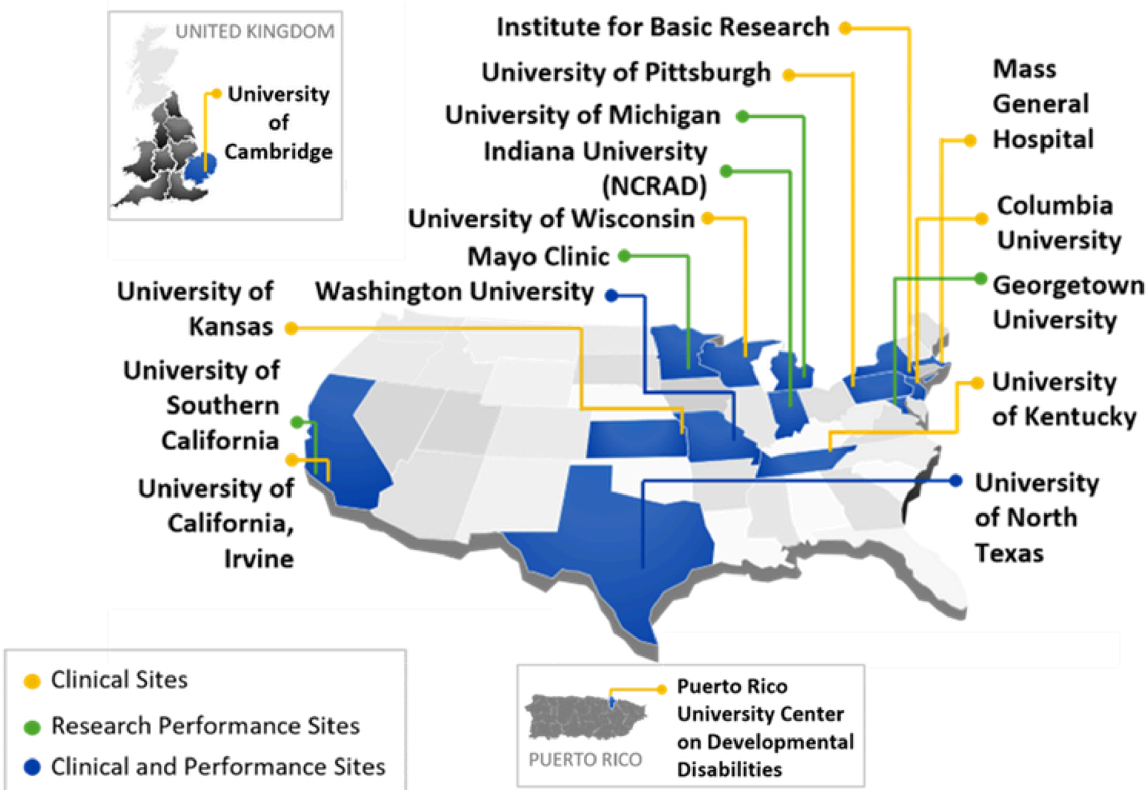


Amyloid precursor protein is present at 1.5 higher levels – overproduction of Aβ

ABC-DS Background

- The Alzheimer Biomarker Consortium- Down Syndrome (ABC-DS) is a five-year longitudinal study to examine biomarkers of Alzheimer's disease (AD) in adults with Down syndrome (ages 25 and older).
- The overall goal of ABC-DS is to understand biological changes underlying AD in people with DS and to develop biomarkers for future clinical trials.
- We wish to maintain a stable cohort of approximately 550 adults with DS and 50 sibling controls.
- Participants undergo a wide range of assessments, including neuropsychological evaluations, blood draws for genetics, proteomics and metabolomics, CSF samples, and neuroimaging (e.g., MRI, amyloid, tau, & FDG PET), and neuropathology.
- Participants are offered the opportunity to co-enroll in a trial ready cohort (TRC-DS) for clinical trials.

ABC-DS
lzheimer Biomarker Consortium-Down Syndrom



ABC-DS

lzheimer Biomarker Consortium-Down Syndrom

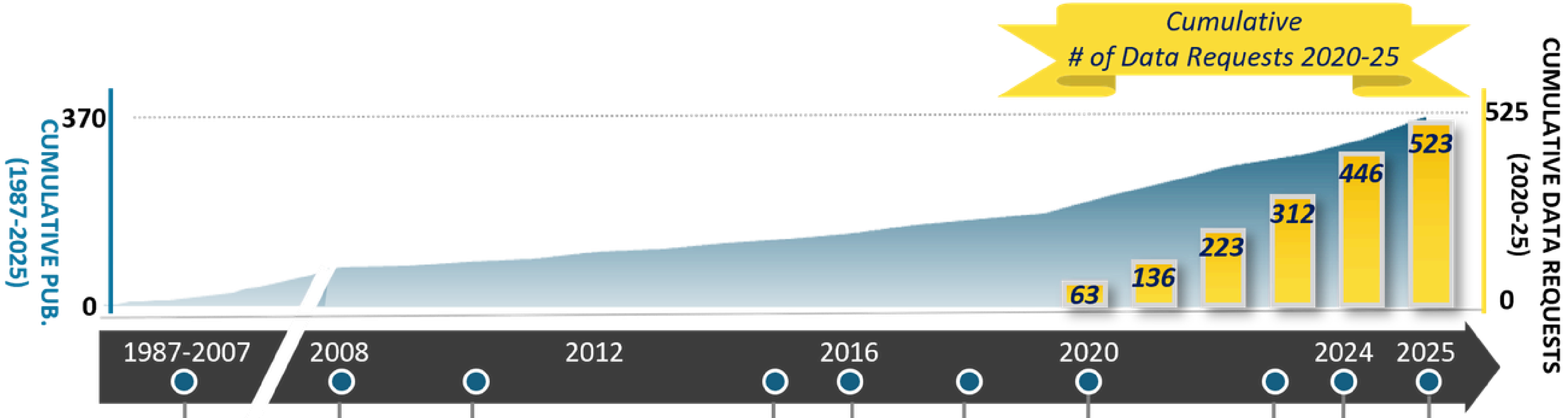
Cohort Characteristics

Sex N (%)		Age Group N (%)					Diagnosis at Cycle 1 N (%)				
Male	Female	25-34	35-44	45-54	55-64	65+	CS	MCI	Possible Dementia	Definite Dementia	UTD
288 (55.0)	236 (45.0)	140 (26.8)	195 (37.3)	123 (23.5)	58 (11.1)	7 (1.3)	421 (80.3)	37 (7.6)	12 (2.5)	33 (6.8)	18 (4.3)

Race N (%)					Ethnicity N (%)		
White	Black/AA	Am Indian/ Alaska Ntv	Asian	More than One Race	Hispanic or Latino	No Hispanic Latino	Unknown
482 (94.3)	12 (2.3)	0 (0.0)	14 (2.7)	3 (0.6)	38 (7.3)	486 (92.7)	0 (0.0)

ABC-DS

lzheimer Biomarker Consortium-Down Syndrom



1987-2008
AD in DS research initiated by Silverman/ Schupf/Lott

2008
ARA announced – 2 years funding for NiAD

2010
UKY initiates NIH R01 study (ADS)

2015
- ADDS Initiated
- NiAD Initiated

2016
ABC-DS is formed

2018
Wash U joins NiAD

2020
- ABC-DS U19 Awarded
- UKY joins ABC-DS

2023
KUMC joins ABC-DS

2024
Puerto Rico joins ABC-DS

2025
UNTH joins ABC-DS

Our model

Traditional Model

Our Approach

Our Goal

1 **INFORM**
Provide the community with relevant information

2 **CONSULT**
Gather input from the community

3 **INVOLVE**
Ensure community needs and assets are integrated into process and inform planning

4 **COLLABORATE**
Ensure community capacity to play a leadership role in implementation of decisions

5 **DEFER TO**
Foster democratic participation and equity by bridging the divide between community and governance, through community-driven decision-making

Facilitating Power

From: The Spectrum of Community Engagement to Ownership, Rosa González of Facilitating Power

Our Community Partnerships

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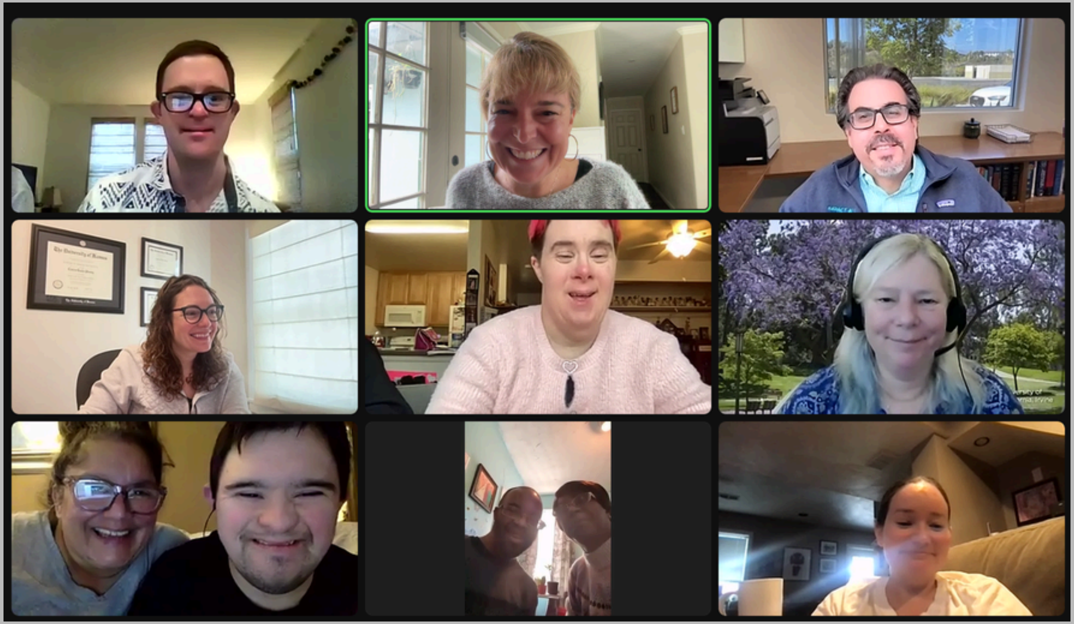

ABC-DS

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What You Need to Know Before Participating:

PARTICIPANT BILL OF RIGHTS

Anyone who is thinking about giving consent to participate in clinical research or on behalf of someone else has the following rights:

- To be told the purpose of the clinical trial or study
- To be told all the potential risks, side effects, or discomforts
- To be told of any potential benefits
- To be told what will happen in the trial, including treatments they might get and what procedures might happen
- To be told if any procedures, treatments, or devices are different than those that are used as standard medical treatment
- To know how long a trial is expected to last
- To be told about all options available and how they may be better or worse than being in a clinical trial
- To ask any questions about the trial before giving consent and at any time during the trial
- To be told of any medical treatments available if medical problems occur during the trial
- To be told about any potential costs or payments
- To bring a trusted friend or family member to meetings with trial staff
- To receive materials in the language they prefer and to ask for an interpreter
- To receive a signed and dated copy of the informed consent form
- To refuse to participate, for any reason and at any time, before and after the trial starts
- To take their time deciding whether or not to participate

CISCRP

Thank you!!

On behalf of the entire ABC-DS team
Our participants and families
For more information about us:
Go to www.abc-ds.org for more information about data available and data request form

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THANK YOU!

Questions?