

Navigating Research Opportunities

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

Presentation Agenda

- I. Welcome & Introduction
- II. Why Research in Down Syndrome Matters
- III. Understanding Clinical Research Basics
- IV. After research: Academic Papers
- V. Becoming Comfortable with Research
- VI. ABC-DS Overview
- VII. Question & Answer



Purpose of Today's Presentation

This session will help us:

- Understand the value of scientific research
- Explore how to interpret and evaluate research
- 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**

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.

Understanding Clinical Research & Clinical Trials



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**, and to
- 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

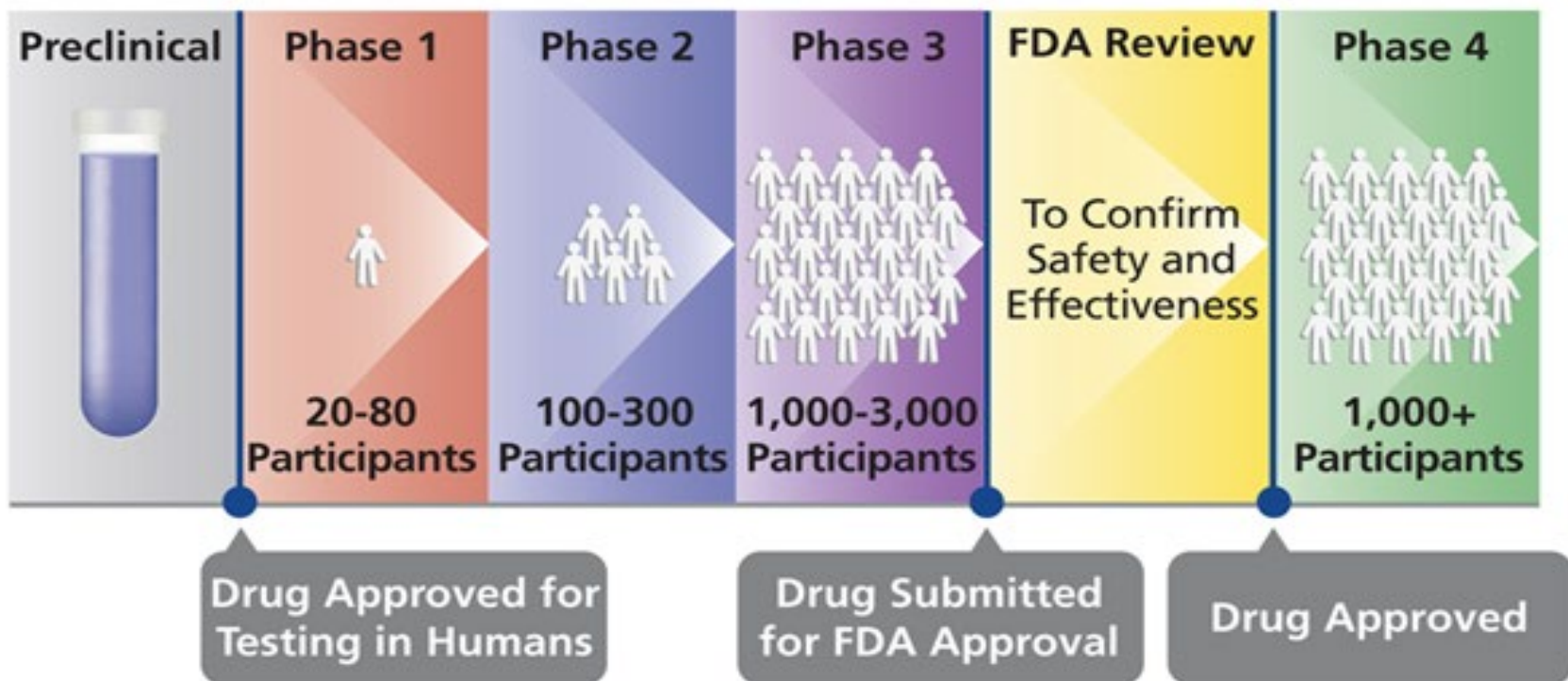
- Important data are collected over time to be used to develop future research
- No experimental medicines or treatments are involved
- Individuals who may be already taking a medicine may be tracked to see long-term health outcomes without changing their treatment plan

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

Clinical Trials





**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.

Patient-focused Drug Development Meeting (PFDD)

Patient-Focused Drug Development (PFDD)

helps ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation.

In June, NDSS will produce a scheduled meeting with members of the FDA to hear directly from self-advocates with Down syndrome, their families, and caregivers on their lived experience with Down syndrome-associated Alzheimer's Disease.



www.NDSS.org/pfdd

Two important dates:

- **Monday, April 6:** community webinar to prepare for PFDD meeting
- **Tuesday, June 2:** Externally-led Patient-focused Drug Development Meeting, streaming online, all day

MinDSet – training researchers to work with individuals with Down syndrome



The MinDSet training is designed for researchers who do not have familiarity working with individuals with Down syndrome.

By taking the MinDSet training, researchers can feel more confident and competent in welcoming individuals with Down syndrome to their clinical research site.

Verbal Communication & Demeanor

Changes in expressive communication and communicative efforts may indicate stress and provide clues to the participant's underlying emotional state. It's important to uncover what they aren't saying.

- Were they chatty and engaged when they first arrived, and are now giving one-word answers to questions?
- Has the pitch and tone of their voice changed, or their pace of speaking picked up or slowed?
- Have more space-filler words, such as "um," and "like" started to appear in their conversation?

These can all be indications of anxiety, fatigue, or simply the need to move on to another task.



Tips for Better Procedures from Experienced Study Coordinators

When working with individuals with Down syndrome: Setting expectations, communicating clearly, and deliberate planning can make medical procedures go more smoothly.

Download a **printable guide** that includes experienced advice on administering procedures to individuals with Down syndrome:



BLOOD DRAWS



BRAIN IMAGING - MRI & PET



LUMBAR PUNCTURES



CONTINUE

After research... academic papers!

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



START WITH THE ABSTRACT

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

DISCUSSION

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?

INTRODUCTION

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?

METHODS

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!

RESULTS

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.

DISCUSSION/ CONCLUSIONS

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?

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

Search

Down Syndrome, Physical Activity and Sleep Apnea

STATUS: RECRUITING

[View Full Study](#)

Down Syndrome Obstructive Sleep Apnea

STATUS: RECRUITING

[View Full Study](#)

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

STATUS: RECRUITING

[View Full Study](#)

CONDITION/DISEASE

GENDER

AGE RANGE

STUDY TYPE

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?

FOR IRB USE ONLY
IRB ID #: 202110138
APPROVAL DATE: 04/04/22
RELEASED DATE: 04/04/22
EXPIRATION DATE: N/A

BEAU ANCES, MD, PHD
WASHINGTON UNIVERSITY IN ST. LOUIS

DOWN SYNDROME UMBRELLA STUDY



Dr. Ances is conducting a research study to look at the effects of aging in adults with Down syndrome.

Do you know an adult with Down syndrome?

We're looking for individuals with Down syndrome who meet the following criteria:

1. Males and Females
2. Ages 18 and older
3. Have a knowledgeable caregiver willing to participate.

Study procedures include:

1. Blood Draw
2. Stool Sample
3. Cognitive Testing
4. Caregiver Questionnaires

STUDY CONTACT:
Brittany Nelson
Phone: 314-747-8425
Email:
bmnelson@wustl.edu



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.

Are you interested?

Scan the QR code and join us!



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

Contact us!

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



ILLINOIS
Speech & Hearing Science
COLLEGE OF APPLIED HEALTH SCIENCES

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 study activities are a lot to handle.

It's a decision that will take **information, education, and conversation**.

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 CISCPR.
- [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.

Research in Action: ABC-DS Overview



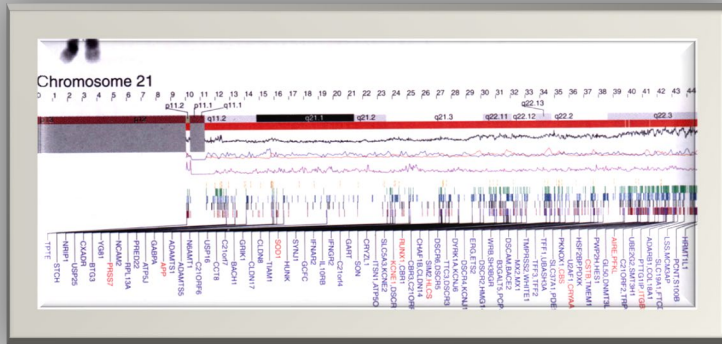


***THE DEVELOPMENT & PROGRESSION
OF ALZHEIMER'S DISEASE
IN DOWN SYNDROME***

Ann D. Cohen

University of Pittsburgh School of Medicine

WHY ARE PEOPLE WITH DOWN SYNDROME VULNERABLE TO ALZHEIMER'S DISEASE?

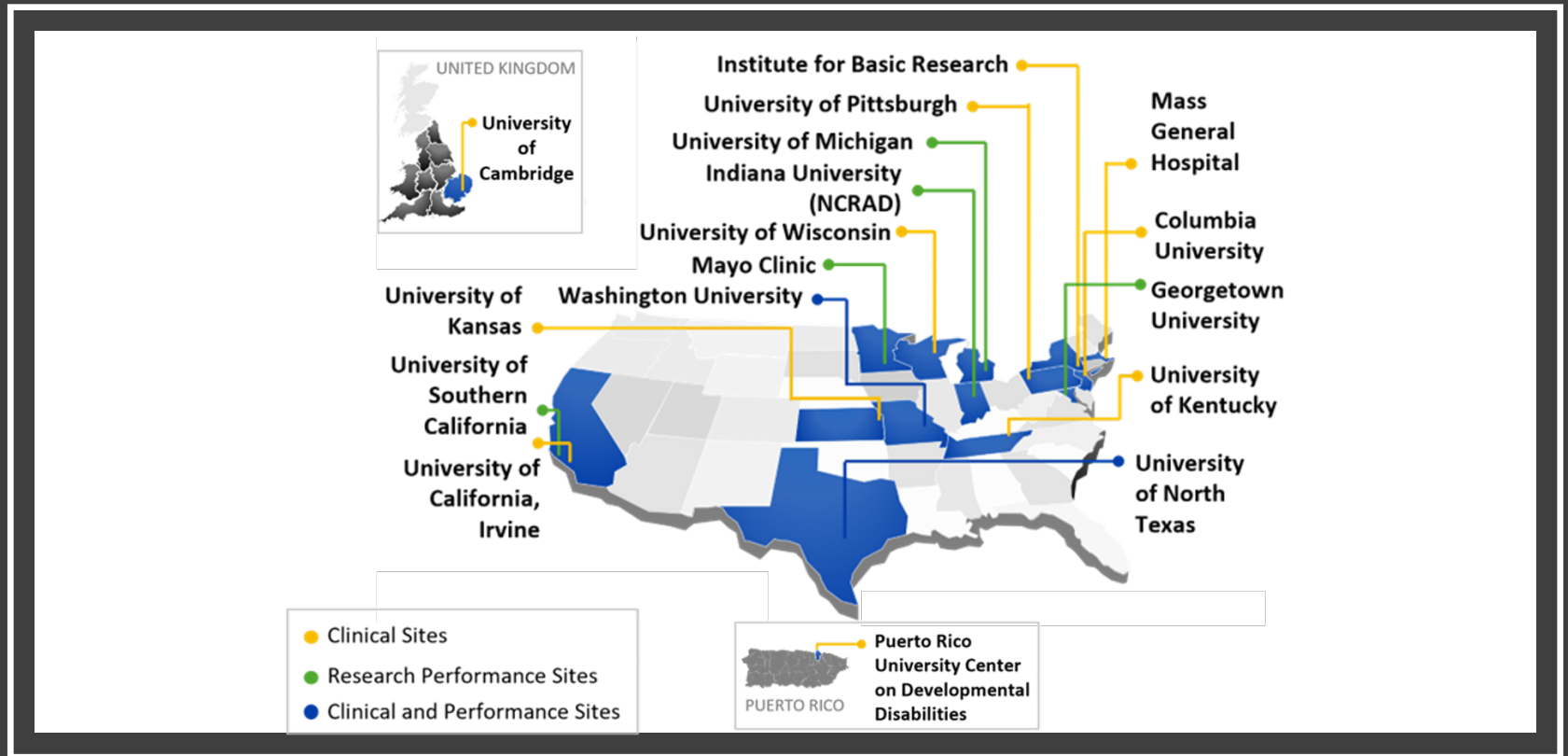


Amyloid precursor protein is present at 1.5 higher levels – overproduction of A β



ABC-DS BACKGROUND

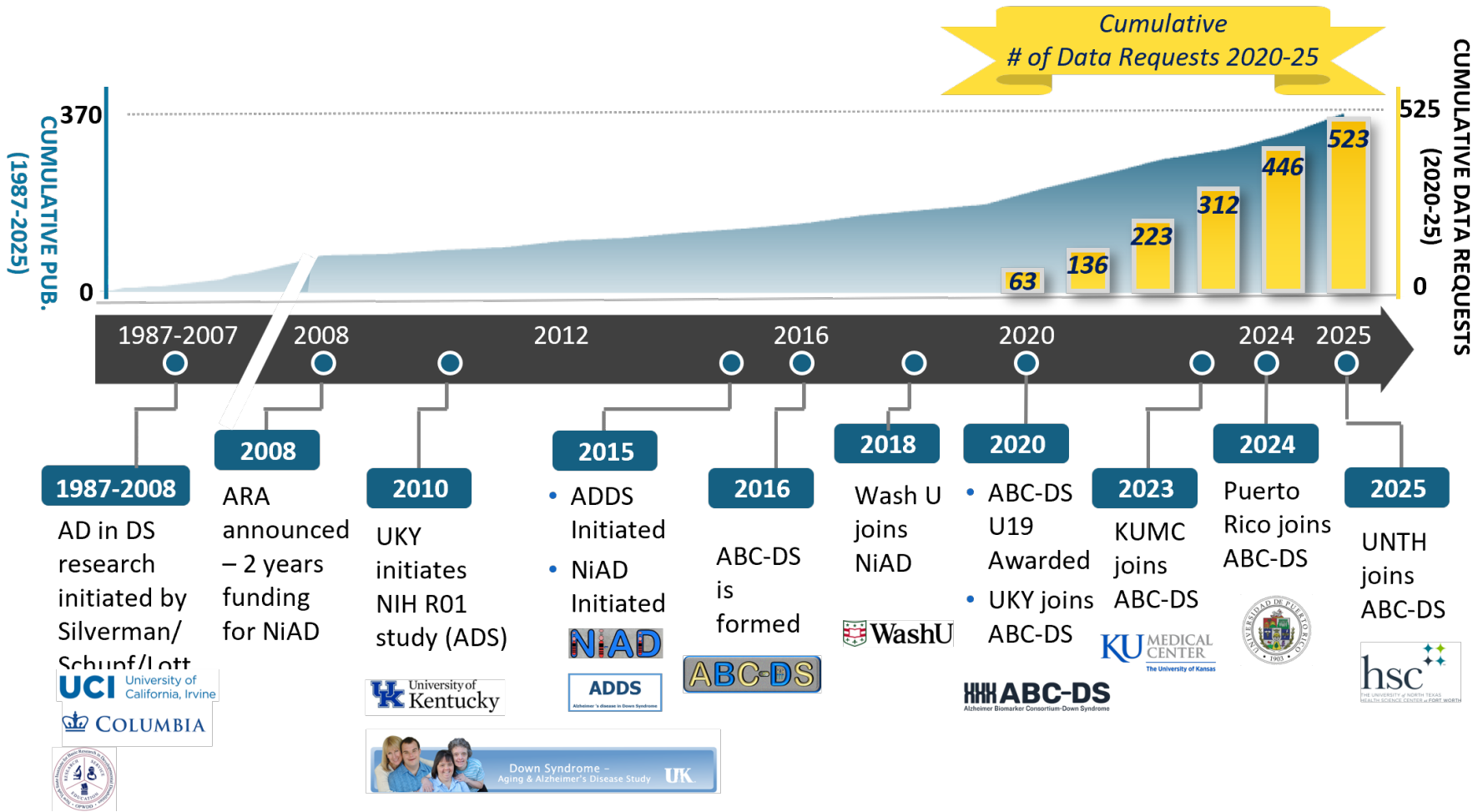
- The Alzheimer Biomarker Consortium- Down Syndrome (ABC-DS) is a five-year longitudinal study to examine biomarkers of Alzheimer's disease (AD) in adults with Down syndrome (ages 25 and older).
- The overall goal of ABC-DS is to understand biological changes underlying AD in people with DS and to develop 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.



COHORT CHARACTERISTICS

Sex N (%)		Age Group N (%)					Diagnosis at Cycle I N (%)				
Male	Female	25-34	35-44	45-54	55-64	65+	CS	MCI	Possible Dementi a	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)



OUR MODEL



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

OUR COMMUNITY PARTNERSHIPS





COMMUNITY
ADVISORS



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





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



THANK YOU!



Questions?