



Paul Stoffels, MD  
CSO Johnson & Johnson

*need*  
The ~~case~~ for collaboration  
and trial innovation in  
Alzheimer's Disease





# IMAGINE

## What I'll talk about

Our Mission

Why do we need trial innovations such as Adaptive Controlled Trials (ACT's)?

Why do we need to work more collaboratively?





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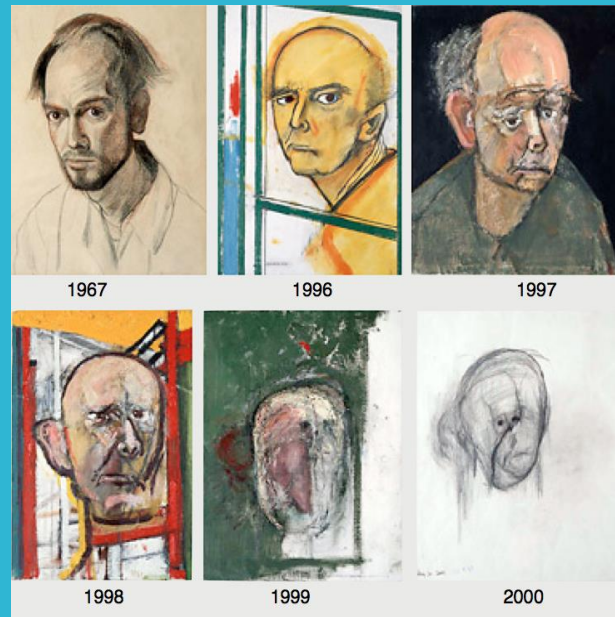
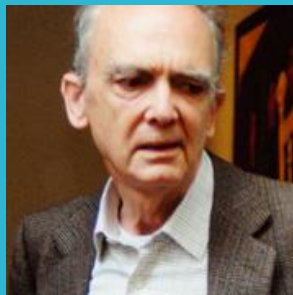
Why do we need to work more collaboratively?



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## William (Bill) Utermohlen

was born in Philadelphia in 1933. The artist settled in London in 1962 and married the art historian Patricia Utermohlen in 1965. When he learned in 1995 that he had Alzheimer's disease he immediately began work on an ambitious series of self-portraits. He died from the consequences of Alzheimer's disease in March 2007.





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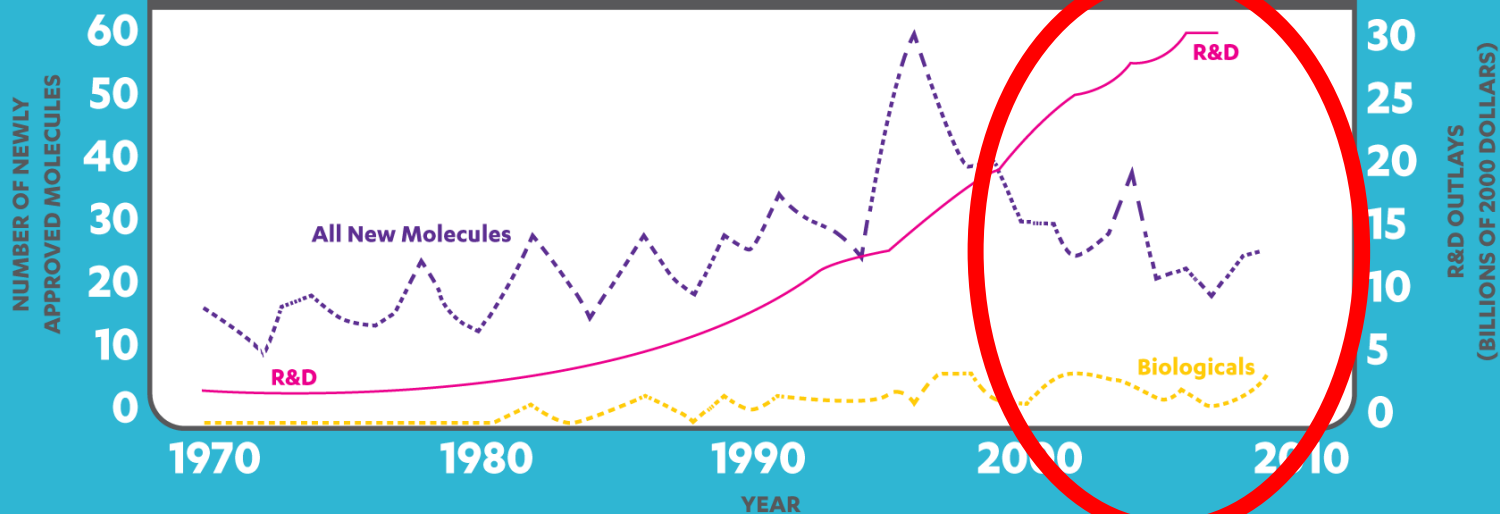
**Why do we need trial innovations such as Adaptive Controlled Trials (ACT's)?**

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**Figure 1**  
**Trends in Newly Approved Molecules and R&D Spending**  
United States, 1970-2009



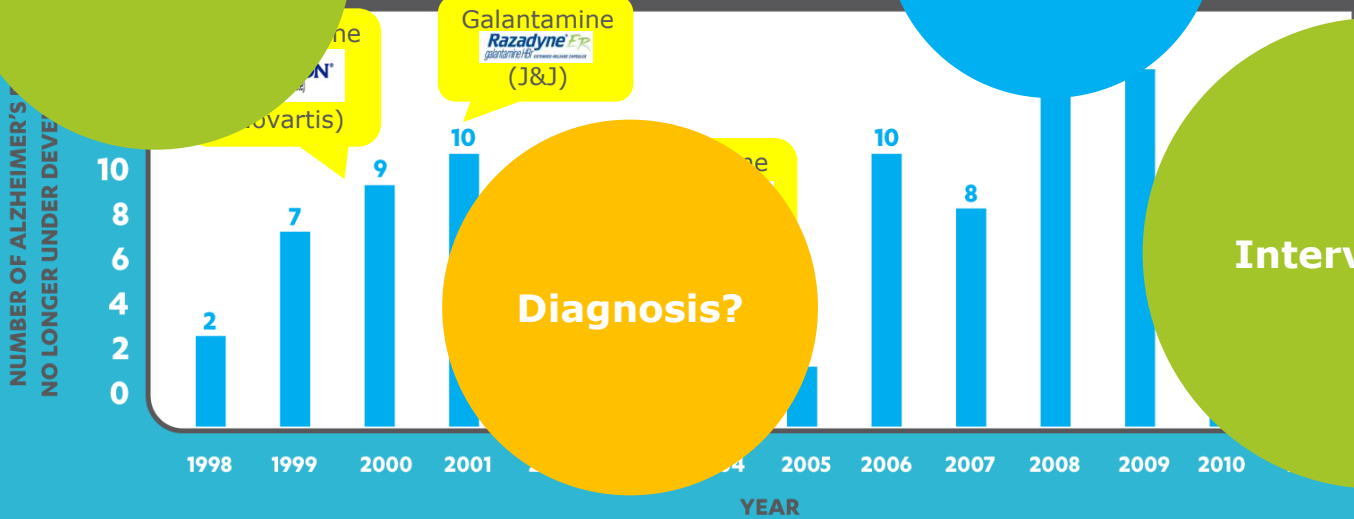
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## The case of AD

Too little  
Too late?

Total unsuccessful drugs 1998-2010  
3 approved = -3% PTRS

Fire and  
Forget  
trials



Diagnosis?

Intervention?



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## What do we need?

**LEARN** faster at  
**optimal**  
time/resource  
cost with **lower**  
**risk** for  
CONFIRMATORY  
PHASE

**Efficiency in trial**

Faster recruitment

Robust standing infrastructure

More data standardization & **data sharing**

**CONFIRM** faster  
with **optimal**  
time/resource  
cost with **higher**  
**probability**  
for achieving  
PATIENT ACCESS



# Types of ACT

TRANSITION ZONE

TRANSITION ZONE

**DISCOVER**

**LEARN**

**CONFIRM**

## Learning/exploratory activities

### Phase I improve exploratory properties:

- Better estimate of safety
- Better understanding of PK-PD

### Phase IIa & IIb possibly combined- the sweet spot of current adaptive design:

- "Quick win/Quick kill"
- Test more doses
- Test more hypotheses

### During the LEARN Phase, study design characteristics can be modified such as:

- Population
- Endpoints
- Dosing regimen

## Confirmation/Confirmatory Activities

### Phase III limited adaptation possible, but useful insurance:

- Start with more doses
- Sample size reassessment

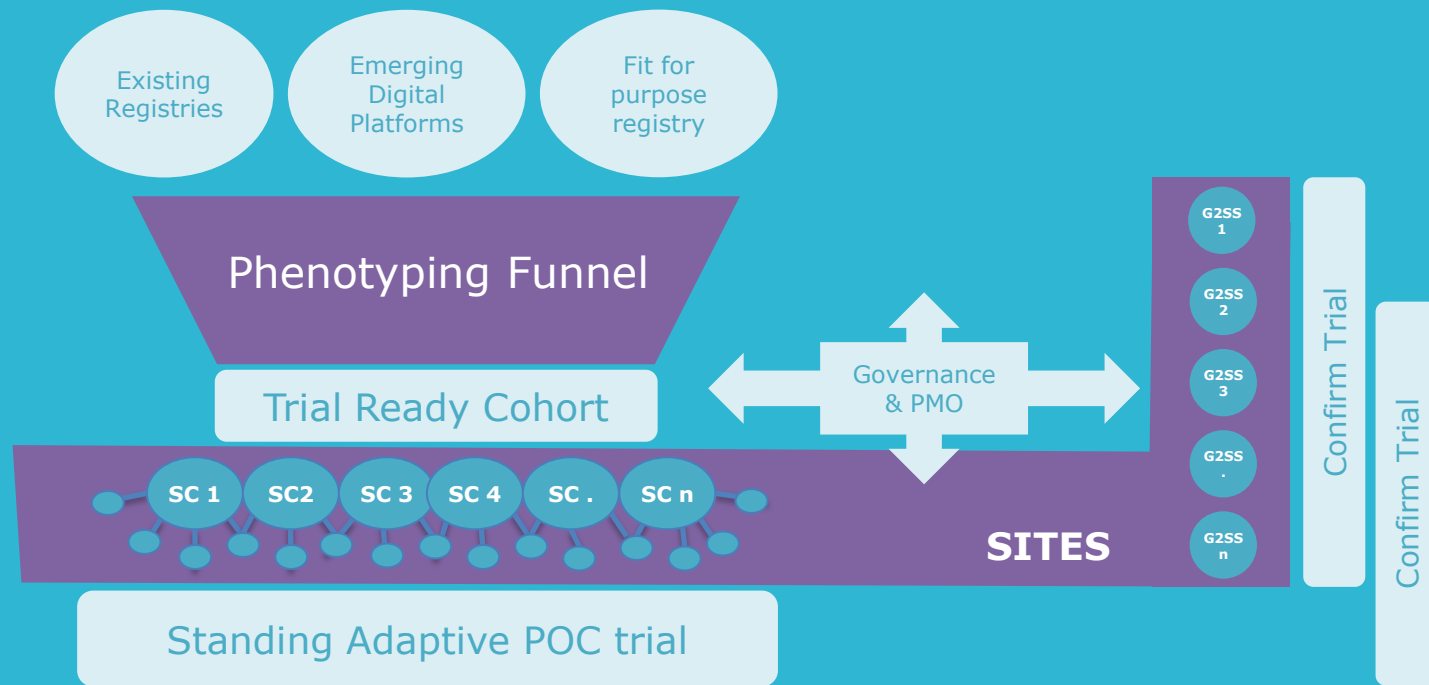
### During the CONFIRM phase study design characteristics can be modified such as:

- Drop doses
- Focus on sub-population
- Re-assess sample size
- Early Stopping



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## Trial platforms- solutions in a box





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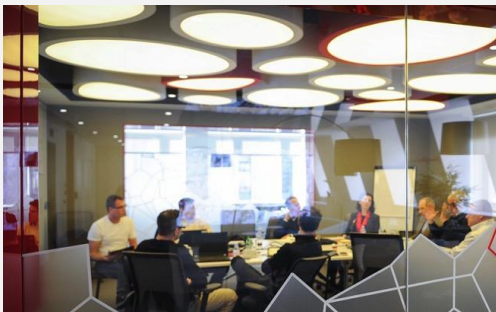
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**0.00002%**  
of brain power

# IMAGINE

## J&J External Innovation Progress: 2013–2015



### Innovation Centers

- 4 Innovation Centers on 3 Continents
- ~200 investments from 2013 - 2015
- Engagement across sectors



### JLABS

- 6 sites in North America
- 120+ companies in residence
- 100% portfolio selected by ICs
- 50% portfolio in discussions
- 27 progressed to deals
- Engagement across all sectors

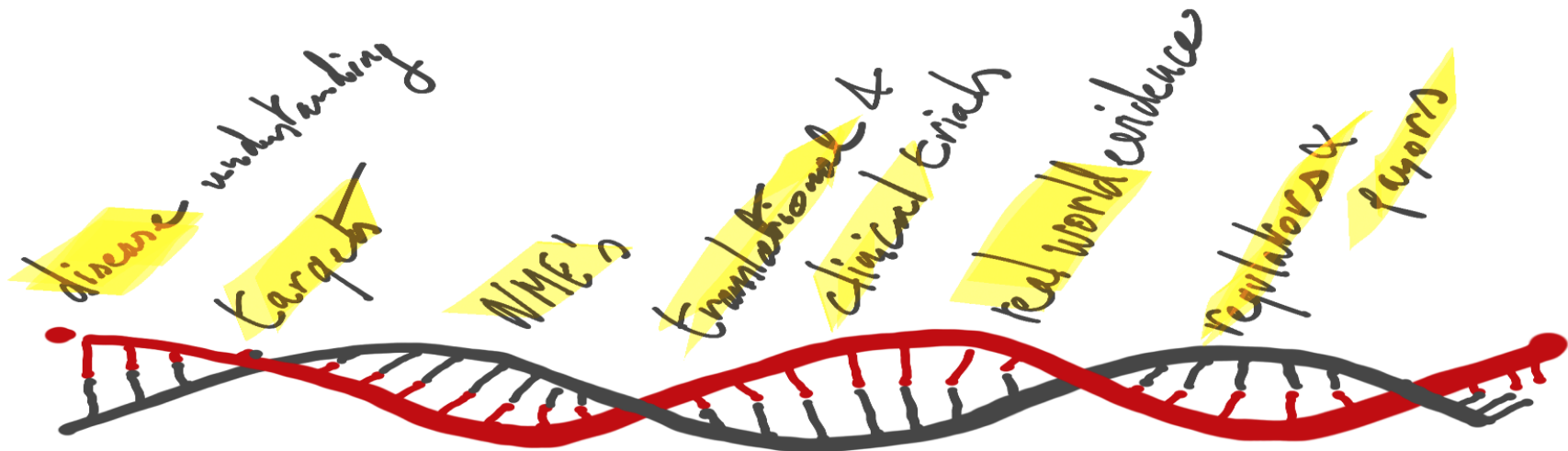


### JJDC

- (40+ years of VC investing)
- 142 direct investments and \$884MM invested 2013 - 2015
  - Partnership w/IC to form newcos on the rise

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## JnJ PPP's: a Continuum of Collaboration



A row of logos for various organizations and initiatives, including:

- Accelerating Medicines Partnership (AMP)
- ADNI (Alzheimer's Disease Neuroimaging Initiative)
- CAMD (Critical Path Institute)
- imi (innovative medicines initiative)
- Dementia Discovery Fund
- Dementias Platform UK (Medical Research Council)
- EPAD (European Prevention of Alzheimer's Dementia Consortium)
- GLOBAL ALZHEIMER'S PLATFORM FOUNDATION
- pcori (Partnership for Public Health)
- Global action against dementia
- World Dementia Council
- OECD (Organisation for Economic Co-operation and Development)

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## Conclusion



We **cannot afford not** to use everything at our disposal **to fight AD**



**Innovative ways to collaborate** provide opportunities to more effectively harness our global brain power and advance the field



**Adaptive trials and standing platforms** are essential tools to accelerate our learning