# IHE

Amy Leval| PhD medical epidemiology| Sept, 2017

Cliff Enright, *Sunburst*
Artwork from The Creative Center at University Settlement, a non-profit organization dedicated to bringing creative arts to people living with and beyond cancer and other chronic illnesses.

janssen

The NEW ENGLAND JOURNAL of MEDICINE

REVIEW ARTICLE

THE CHANGING FACE OF CLINICAL TRIALS

Jeffrey M. Drazen, M.D., David P. Harrington, Ph.D., John J.V. McMurray, M.D., James H. Ware, Ph.D., and Janet Woodcock, M.D., *Editors*

# Evidence for Health Decision Making — Beyond Randomized, Controlled Trials

Thomas R. Frieden, M.D., M.P.H.

# Paradigm shift

Numerous examples of when real world evidence (RWE) had been superior to RCT results in terms of accuracy. Discussed the limitations of RCTs in terms of ethics, long term follow-up and feasibilty.

Article advice on using non-RCTs for decision making:

1) promote transparency in study methods
2) ensure standardized data collection for key outcomes
3) use new approaches to improve data synthesis (a.k.a. harmonization)

# Medical science transformation

1906 FDA false labeling- not claims

1938 Safety

1948 1st RCT

1953 DNA Watson and Crick

1966 FDA efficacy

1980s computer capacity to describe molecular structure

Rational drug development


1990 Human Genom Project

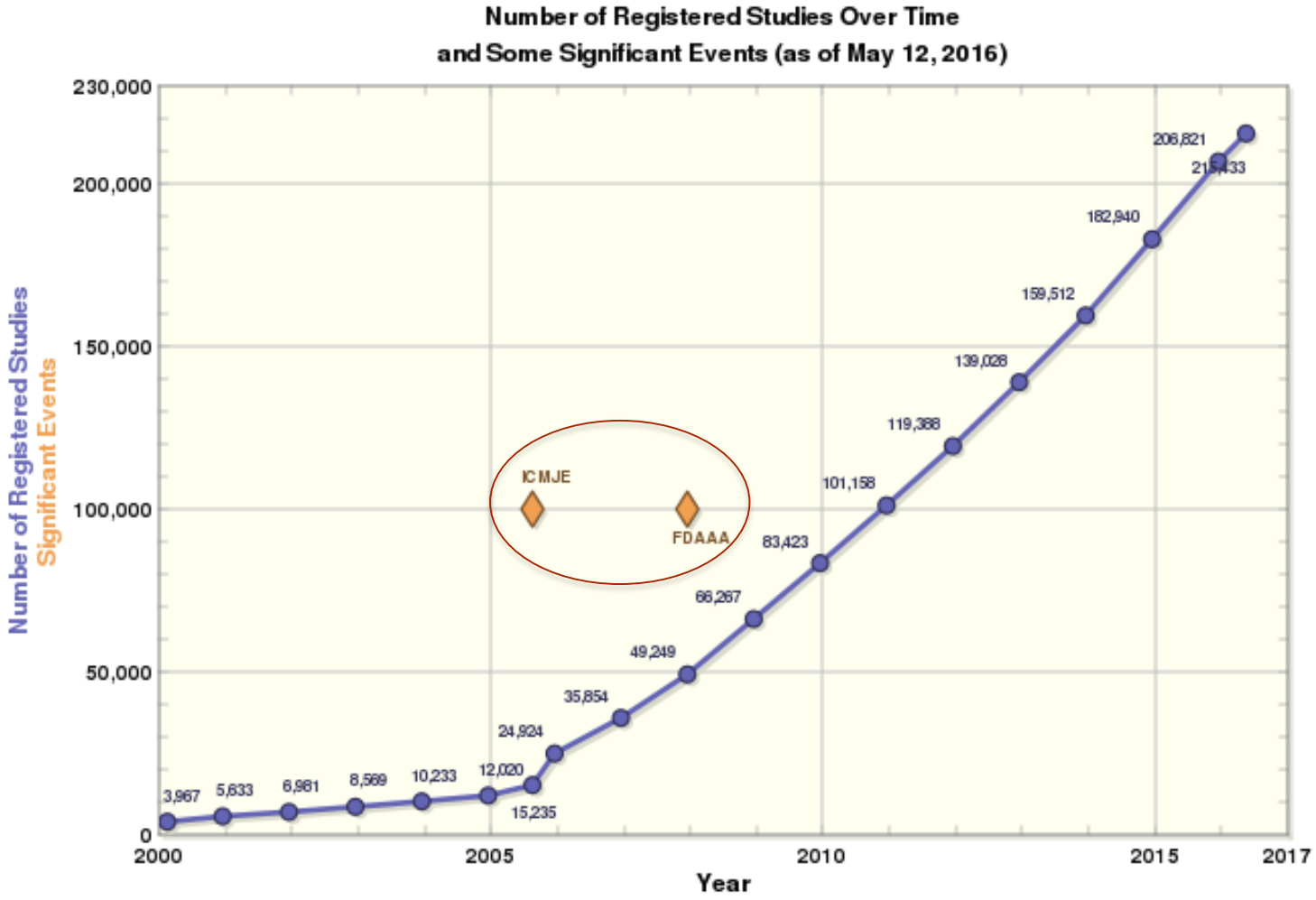
1991 www

2000 clincialtrials.gov

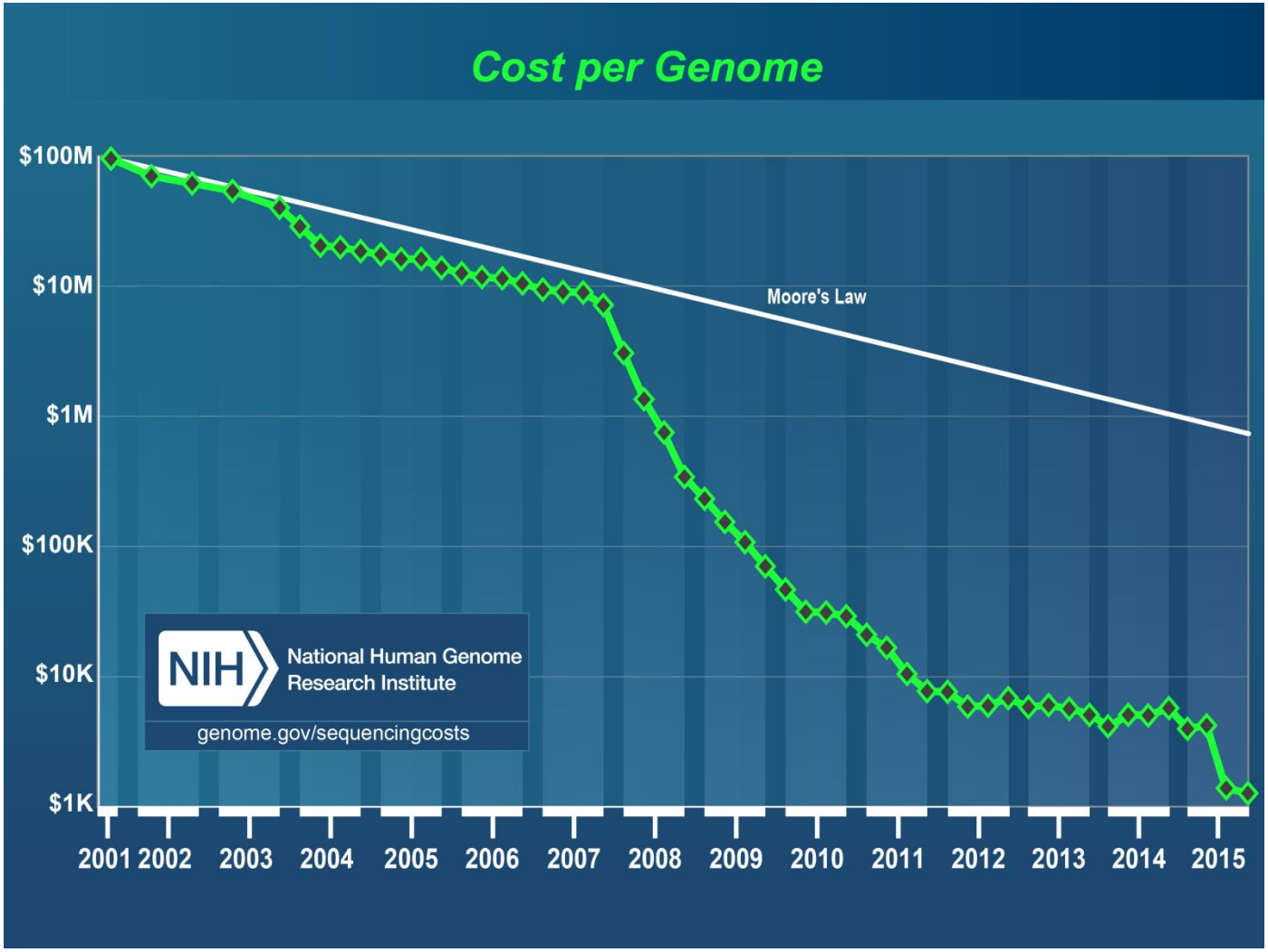
2008 Next gen sequencing

2017 A.I./machine learning

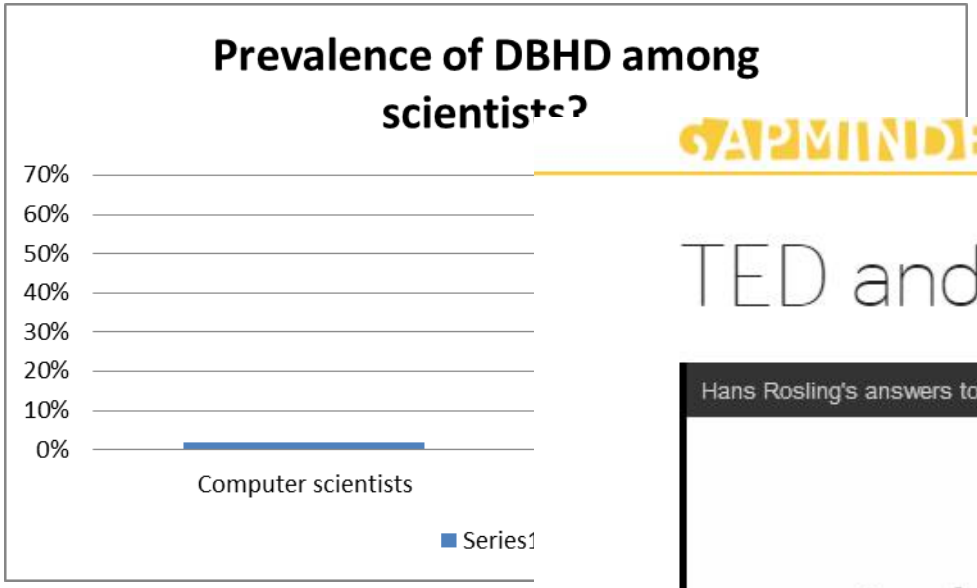
# Clinical Trials.gov




Source: https://ClinicalTrials.gov


Cost per Genome
$100M
$10M
$1M
$100K
$10K
$1K
Moore's Law
NIH
National Human Genome Research Institute
genome.gov/sequencingcosts
2001 2002 2003 2004 2005 2006 2007 2008 2009 2010 2011 2012 2013 2014 2015

# DBHD epidemic




GAPMINDER WORLD VIDEOS DOWNLOADS TEACH

## TED and Reddit's 10 questions

DATA.GOV

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# The home of the U.S. Government's open data

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ACTGATGGTATGGGGCCAAGAGATATATCT
CAGGTACGGCTGTCATCACTTAGACCTCAC
CAGGGCTGGGCATAAAAGTCAGGGCAGAGC
CCATGGTGCATCTGACTCCTGAGGAGAAGT
GCAGGTTGGTATCAAGGTTACAAGACAGGT
GGCACTGACTCTCTCTGCCTATTGGTCTAT

ClinV

ClinVar a

DN Debatt

# *DN Debatt. "Regeringen vill snabba på arbetet med öppna data"*

Usir

Abou

PUBLICERAD 2017-05-14

**Sverige har halkat efter i ar offentligt producerad data s för effektivisering och nya tjänster. Regeringen snabbar nu på arbetet med en rad insatser. Öppna data hjälper offentlig sektor och ger svenska företag möjligheter att skapa innovativa tjänster, skriver civilminister Ardalan Shekarabi (S).**

The New York Times



BUSINESS DAY

# *Johnson & Johnson Will Make Clinical Data Available to Outside Researchers*

By KATIE THOMAS JAN. 14, 2015

the YODA PROJECT

Forging a unified scientific community

ABOUT REQUEST TRIALS FAQS LOG IN

## Johnson & Johnson

### Partnership with Johnson & Johnson Family of Companies

As of October 2014, the Yale University Open Data Access (YODA) Project is enabling scientists across the world to gain access to the company's clinical trial data assets. The goal is to balance data availability with an intention to promote strong scientific research and serve the public good. This effort will accelerate the movement of the clinical research enterprise toward more cooperative learning and sharing.

The YODA Project performs independent scientific reviews of investigator requests for Johnson & Johnson's pharmaceutical and medical device clinical trial data, including both full Clinical Study Reports and participant-level data. Johnson & Johnson has conferred on the YODA Project the authority to make decisions about the release of their clinical trial data. This

# Present

Open data movements but prevalence of DBHD

Precision medicine: targeted therapies gaining early regulatory approval

PPPs like IMI and genomic consortiums driving data harmonization and collaborative analytics

Expanding use of real world evidence to inform trial design – entire lifecycle

# Why is this region unique?

Link, link, link
genotype with
phenotypes.
**Longitudinal cohorts**

# Unique opportunity for Nordic life science

**Real world evidence will impact all phases of medical research and therapeutic development.**

Thanks to its data infrastructure, the Nordic region can become a hot-spot for advanced medical research by enabling uniquely comprehensive and long term studies.

Janssen is partnering with Karolinska Institutet in a major collaborative research initiative that focuses on:

1. Treatment resistant depression
2. Prostate cancer
3. Psoriasis
4. B-Cell malignancies
5. Methodology development

Janssen's real world evidence network also facilitates collaboration in other disease areas, across Nordic institutions.

# ” promote transparency in study methods”

1. EMA’s ENCePP (Guide on Methodological Standards in Pharmacoepidemiology)

2. SBU - TLV

*** Specify method requirements and sensitivity analyses for comparisons (be they direct or indirect)**

# ” ensure standardized data collection for key outcomes”

1. Paucity of data for oncology treatment outcomes!
2. HCPs cannot be expected to double and triple document.

- National registries, but missing key treatment exposure
- ICHOM for some disease areas (otherwise define)

***Requirements for e-journals to structure data and open APIs**

*Introduce clinical decision support tools (InfCare, PC, SRQ)

# ” use new approaches to improve data synthesis ”

- OHDSI – collaborative analytics
- Swedish Cohort Consortium
- BD4BO



***Think national and international harmonization**

# Future

1. Specify method requirements and sensitivity analyses for comparisons
2. Requirements for e-journals to structure data and open APIs
3. Promote national and international harmonization
4. Find DBHD cure and prevention

Thank you