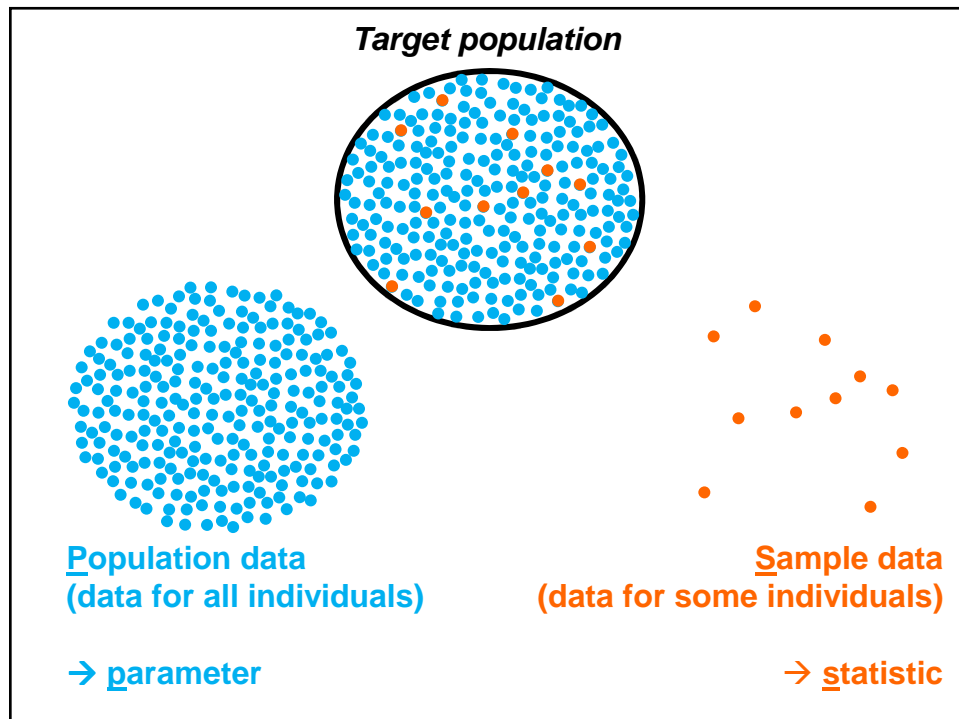


Collecting data

PSLS chapters 6 and 7

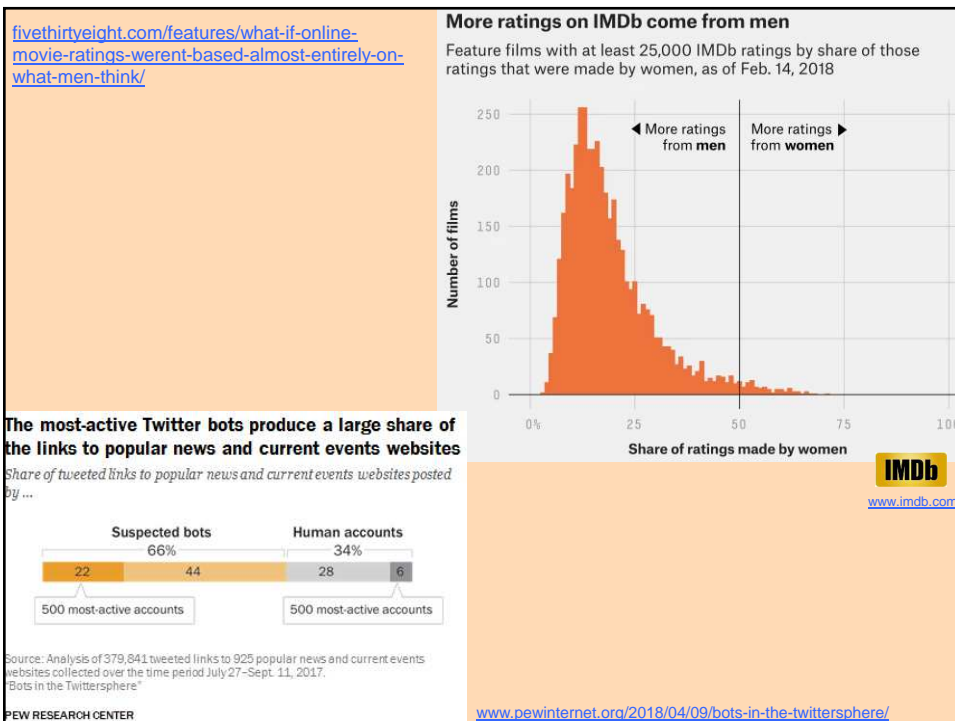
Copyright Brigitte Baldi 2019 ©



The role of randomness

How do you choose the individuals/units to observe for a study?

- ❑ **Anecdotal evidence:** haphazardly selected individual cases
- ❑ **Volunteer/voluntary response sampling:** individuals choose to be involved because they want to be heard (self selected sample)
- ❑ **Convenience sampling:** ask whoever is around (mall, university) or take the next 10 units
- ❑ **Probability sampling:** individuals or units are randomly selected; the sampling process is **unbiased**



Some types of probability samples

Individuals are randomly selected; the sampling *process* is **unbiased**.

▣ A **simple random sample** (SRS) is made of randomly selected individuals. Each individual in the population has the same probability of being in the sample. All possible samples of size n have the same chance of being drawn.

In a class of 220 students, the instructor uses the roster to randomly pick 5 students' midterms to check that they were graded properly.

▣ A **stratified random sample**: make sure your sample has x,y,z% of individuals of certain types.



America's *State of Mind* report was based on a probability sample of Medco's de-identified database of insured members stratified by age group and sex to match its customer demographics.

... many more

Independent vs paired samples

When comparing two "conditions" or two "types," data can be collected as **independent** samples or as **matched pairs/repeated measures** samples.

▣ Independent samples: The individuals compared across conditions/types are UNRELATED

Comparing the concentration of a toxic chemical in a random sample of wells located at an elevation that is either higher or lower than the elevation of a local toxic waste site

▣ Matched pairs, repeated measures, time series: The individuals compared across conditions/types are RELATED (or even identical)

Comparing the concentrations of the herbicide atrazine before and after herbicide application season in a random sample of shallow groundwater sites (*same sites both times*)

Comparing 2 “conditions/types” (A, B):

Data organization is completely different when collecting data with independent vs repeated measures/matched pairs samples

Which data set represents independent samples?

C

Individual ID	Condition or type	Value recorded
01	A	
02	A	
03	B	
04	A	
05	A	
06	B	
07	B	
08	A	
09	B	
10	B	
...		

D

Individual or pair ID	Value for type/ condition A	Value for type/ condition B
01		
02		
03		
04		
05		
06		
07		
08		
09		
10		
...		

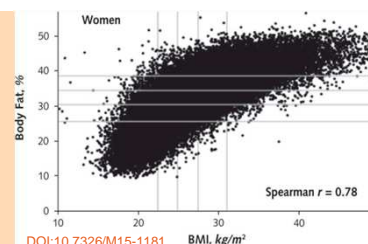
Selecting a variable to study

- What is the study’s ultimate objective, its end goal?
- What aspect(s) of this goal can be recorded?
- Would a quantitative or a categorical assessment be more suitable?
- Is cost, speed, or accuracy more important to the study’s success?

→ Pick a primary variable

→ Pick additional variables needed for a full picture of the problem

There are 10 different methods for assessing **body fat**, which is then used to determine underweight/ healthy/ overweight/ obese status, which is then used to assign various health risks.



Canada To Measure Marijuana Use By Testing Sewage (NPR: April 13, 2018)

www.npr.org/sections/health-shots/2018/04/13/599747395/canada-to-measure-marijuana-use-by-testing-sewage

Canada is working on a bill to legalize recreational marijuana use, so there is strong interest in quantifying cannabis use. [*legalized summer 2018*]

- There are things like surveys where people report frequency of use, but the consumption numbers weren't quite as reliable as we would like them to be.
- Ideally, Statistics Canada would like to estimate how much cannabis Canadians consume, in total, through the sewage measurements.
- The compound measured to detect cannabis use sticks around in fat, not water, and it leaves the body slowly, over days rather than hours.
- The suburban users, are they peeing in the city but consuming in the suburbs?
- Wastewater testing primarily samples liquids, not solids, so it only provides a small window into all the cannabinoids that exit when you use cannabis.



Observational versus experimental studies

Observational study: Record data on individuals without attempting to influence the responses.

Countless confounding variables limit scope of conclusions.

Concluding causation is very difficult.

Experimental study: Deliberately impose different treatments on individuals and record their responses.

Influential factors can be controlled.

Concluding causation is often possible.

#1. In 1992, several major medical organizations said that women should take hormones such as estrogen after menopause, because a study found a lower risk of heart attack for women who took replacement hormones in a large random sample of postmenopausal women.

#2. By 2002, several studies concluded that hormone replacement does not reduce the risk of a heart attack. These studies had randomly assigned women to take either hormone replacement pills or dummy pills.

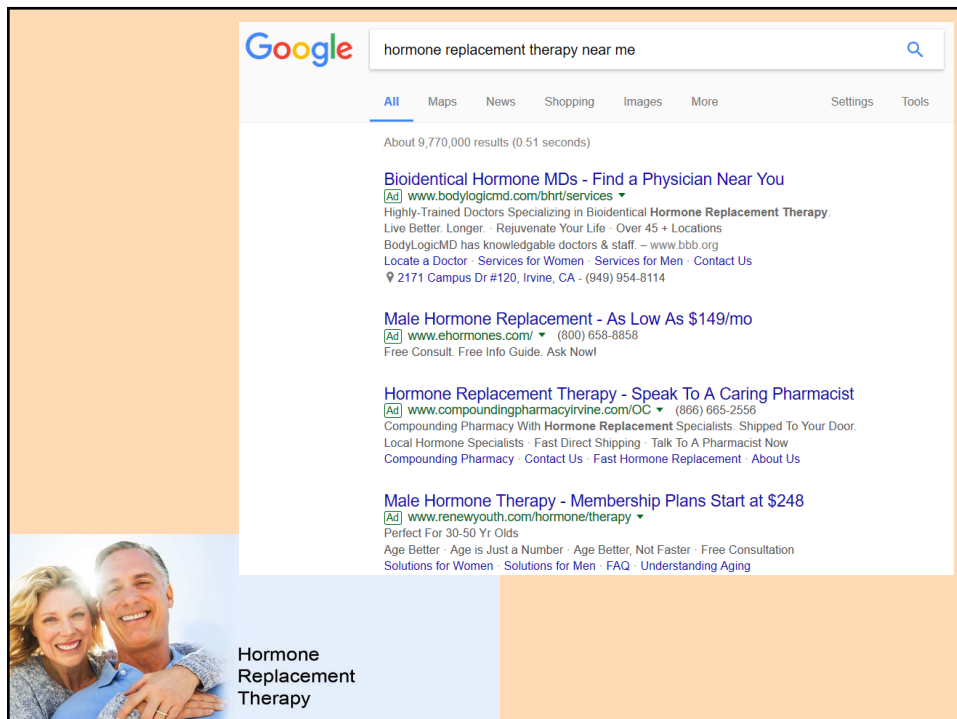
- A) both observational B) both experimental C) both anecdotal
D) #1 observational, #2 experimental E) #1 anecdotal, #2 experimental

Some studies were terminated early because adverse health events were more common and more serious among the women assigned to hormonal treatment. Other studies didn't see find a difference.

→The effects of HRT on the body vary by age and time since last physiologic exposure to hormones.

[Climacteric. 2017 Apr;20\(2\):91-96.](#)

www.cancer.gov/about-cancer/causes-prevention/risk/hormones/mht-fact-sheet#g3



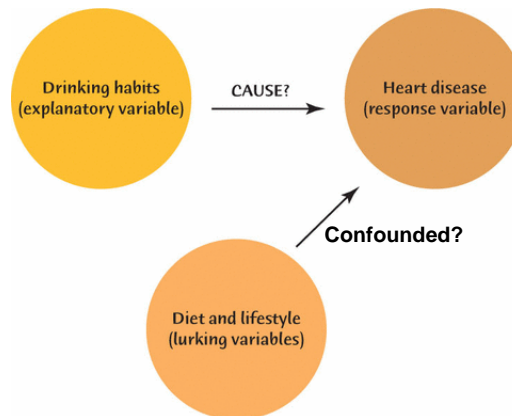
The screenshot shows a Google search interface with the query "hormone replacement therapy near me". Below the search bar, there are tabs for "All", "Maps", "News", "Shopping", "Images", "More", "Settings", and "Tools". The search results indicate "About 9,770,000 results (0.51 seconds)". Four paid advertisements are visible:

- Bioidentical Hormone MDs - Find a Physician Near You**
Ad | www.bodylogicmd.com/bhrt/services | (866) 665-2556
Highly-Trained Doctors Specializing in Bioidentical Hormone Replacement Therapy.
Live Better. Longer. · Rejuvenate Your Life · Over 45+ Locations
BodyLogicMD has knowledgeable doctors & staff. — www.bbb.org
Locate a Doctor · Services for Women · Services for Men · Contact Us
2171 Campus Dr #120, Irvine, CA · (949) 954-8114
- Male Hormone Replacement - As Low As \$149/mo**
Ad | www.ehormones.com/ | (800) 658-8858
Free Consult. Free Info Guide. Ask Now!
- Hormone Replacement Therapy - Speak To A Caring Pharmacist**
Ad | www.compoundingpharmacyirvine.com/OC | (866) 665-2556
Compounding Pharmacy With Hormone Replacement Specialists. Shipped To Your Door.
Local Hormone Specialists · Fast Direct Shipping · Talk To A Pharmacist Now
Compounding Pharmacy · Contact Us · Fast Hormone Replacement · About Us
- Male Hormone Therapy - Membership Plans Start at \$248**
Ad | www.renewyouth.com/hormone/therapy | (866) 665-2556
Perfect For 30-50 Yr Olds
Age Better · Age is Just a Number · Age Better, Not Faster · Free Consultation
Solutions for Women · Solutions for Men · FAQ · Understanding Aging

In the bottom left corner of the screenshot, there is a small image of a smiling couple and the text "Hormone Replacement Therapy".

Reminder: **Confounding**

Two variables are **confounded** when their effects on a third variable cannot be distinguished from each other. This is a major issue with observational studies, making it extremely difficult to conclude causation.



Review of 87 research studies on alcohol's effect on death from any cause:

[*Journal of Studies on Alcohol and Drugs*, 77\(2\), 185–198 \(2016\)](#)

Such studies typically compare health outcomes for non-drinkers, occasional, moderate, and heavy drinkers. Most find that moderate drinkers (1-3 drinks a day) are less likely to die early from health problems (heart disease, cancer ...).

But moderate drinkers tend to be very socially advantaged.

And people in poor health tend to quit drinking eventually.

So moderate drinkers tend to be in better health, while non drinkers are actually a mix people who never drank and people who stopped for health reasons.



Common types of observational studies

- A **sample survey** is an observational study that relies on 1 random sample drawn at one time from a population (“**cross-sectional**”)
 - Opinion polls (typically using voter registries or telephone numbers)
 - Economic indicators (unemployment, consumer price index, ...) monitored through extensive and closely guarded monthly surveys
 - Epidemiologic surveys to establish the rate of medical conditions, diseases, and lifestyles
 - Surveys are also used extensively in social sciences

Don't confuse scientific surveys/polls and quick polls using voluntary samples.



**WATCH
AT
HOME**

Video Explainer: Understanding random sampling for public opinion surveys

The first video in our “Methods 101” series is about random sampling, a concept that undergirds all probability-based survey research. Here’s how it works.



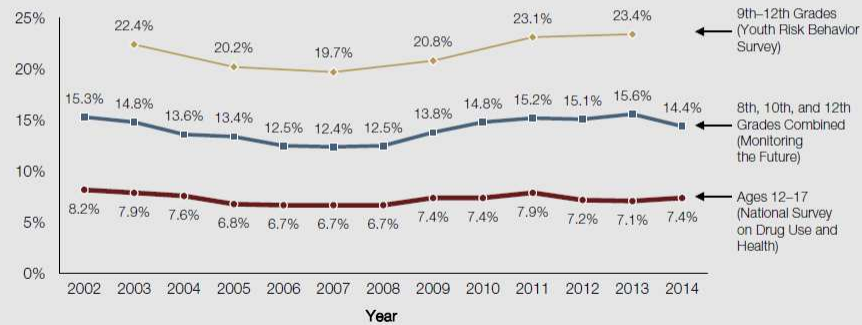
www.pewresearch.org/2017/05/12/video-explainer-understanding-random-sampling-for-public-opinion-surveys/

<https://www.youtube.com/watch?v=sonXfzE1hvo>

Why such drastic differences in findings?

Past Month Marijuana Use Among Adolescents in the United States, by National Survey (2002–2014)

Despite differences in methods between surveys, data from the National Survey on Drug Use and Health (NSDUH), Monitoring the Future (MTF), and the Youth Risk Behavior Survey (YRBS) generally show that the percentage of adolescents in the United States who used marijuana decreased from the early 2000s to the mid- to late-2000s and then increased gradually in more recent years. The percentage for MTF decreased significantly from 2013 to 2014.



Sources: SAMHSA, Center for Behavioral Health Statistics and Quality, National Surveys on Drug Use and Health, 2002–2014; National Institute on Drug Abuse, Monitoring the Future, University of Michigan, 2002–2014; Centers for Disease Control and Prevention, Youth Risk Behavior Surveys, 2003, 2005, 2007, 2009, 2011, and 2013. www.samhsa.gov/data/sites/default/files/2015_National_Barometer.pdf

YRBSS and Monitoring the Future are school-based surveys
NSDUH is a survey of households conducted in-person

Some survey challenges

- **Undercoverage:** Parts of the population are systematically left out.
www.pewresearch.org/2015/11/18/advances-in-telephone-survey-sampling
www.pewresearch.org/2015/09/22/coverage-error-in-internet-surveys
- **Nonresponse:** Some people choose not to answer/participate.
- **Wording effects:** Biased or leading questions, complicated/confusing statements can influence survey results.
- **Response bias:** Fancy term for lying or forgetting (especially on sensitive/personal issues). Can be exacerbated by survey method (in person vs. by phone or online).

The Census Bureau's American Community Survey (used for federal funding etc.) is mailed monthly to ~300,000 U.S. addresses. Response to the survey is mandatory. Response rate is consistently well above 95%.

The CDC's National Health Interview Survey interviews participants in person and has a response rate of ~70%.

Table 2. Response Rates (AAPOR study of 15 large and prestigious polling research firms)

www.aapor.org/Education-Resources/Reports/The-Future-Of-U-S-General-Population-Telephone-Sur.aspx

	Landline	Cell
2008	15.7%	11.7%
2009	13.7%	10.3%
2010	13.0%	11.2%
2011	13.6%	10.4%
2012	10.9%	7.2%
2013	9.8%	6.9%
2014	8.2%	6.5%
2015	9.3%	7.0%

Table 1. Mean Difference Between Self-Reported and Measured Height, Weight, and Body Mass Index, by Sex, National Health and Nutrition Examination Survey, 2001-2006^a

Characteristic	Self-Reported	Measured	Mean Difference (95% CI)
Men			
Standing height, cm	177.67	176.45	1.22 (1.15, 1.28)
Weight, kg	87.55	87.25	0.30 (0.20, 0.40)
Body mass index (kg/m ²)	27.62	27.96	-0.34 (-0.38, -0.30)
Women			
Standing height, cm	162.99	162.31	0.68 (0.62, 0.74)
Weight, kg	72.46	73.85	-1.39 (-1.48, -1.30)
Body mass index (kg/m ²)	27.20	28.02	-0.82 (-0.85, -0.77)

**WATCH
AT
HOME**

MARCH 21, 2018



Video Explainer: Understanding survey question wording

The second video in Pew Research Center's "Methods 101" series helps explain question wording – a concept at the center of sound public opinion survey research – and why it's important.



www.pewresearch.org/2018/03/21/video-explainer-understanding-survey-question-wording/

www.youtube.com/watch?v=eFzGdQrr2K8

Some other types of observational studies

▣ **Case-control studies** start with 2 random samples of individuals with different outcomes. Individuals with the condition are cases, and those without are controls.

▣ **Cohort studies** enlist individuals sharing a common demographic and study them over a long period of time to examine the compounded effect of various factors over time ("**longitudinal**").

-**Retrospective** studies look for exposure factors in the subjects' past.

-**Prospective** studies collect data over time waiting until some participants eventually develop a condition.

Researchers studied the 2010 epidemic of pertussis in California.

- They selected a random sample of 682 medical records of California children ages 4 to 10 who had been diagnosed with pertussis.
- They also selected a random sample of 2016 medical records of California children in the same age group who had received care from the same clinicians on the same day but were not diagnosed with pertussis.

They found that children diagnosed with pertussis were much more likely to have not received any pertussis vaccine, to have not received all recommended doses of the vaccine, or to have had a longer interval of time since their last vaccination.

- A: cross-sectional survey**
B. longitudinal cohort
C. case-control study
D. unscientific study with voluntary sample
E: anecdotal evidence

[doi:10.1001/jama.2012.14939](https://doi.org/10.1001/jama.2012.14939)



Comparative, randomized experiments

Experiments **compare** the response to a treatment versus to:

- another treatment,
- the absence of treatment (a “control”)
- a placebo
- or any combination of the above

Experiments **randomize** the assignment of individuals to treatment.

Experiments use **replication**: several or many individuals are studied.

About the “placebo effect”

Improvement in health or perceived condition due, not to any active treatment, but only to the patient's belief of being cared for or helped.

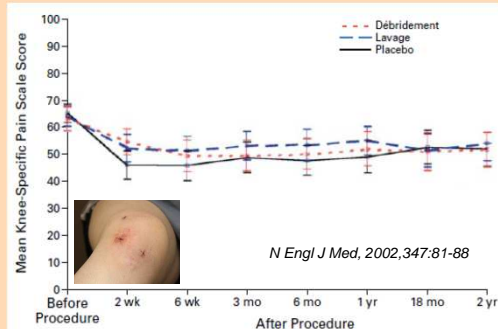
- therapeutic results observed (nocebo also exists)
- neural response to the placebo effect recorded even in spinal chord

Direct Evidence for Spinal Cord Involvement in Placebo Analgesia

Falk Eippert,^{1*} Jürgen Finsterbusch,² Ulrike Bingel,² Christian Büchel¹
Science (2009), DOI:10.1126/science.1180142

When medical/physical therapy fails to relieve the pain of osteoarthritis of the knee, arthroscopic lavage or débridement is often recommended.

- Over 650,000 procedures per year, roughly \$5,000 each.
- In uncontrolled studies, ~half the patients report some pain relief.



Some experimental design issues

Bias, conscious or unconscious, may arise from the placebo effect (subjects) or the experimenter. "**Blinding**" can help against bias.

A **double-blind** experiment is one in which neither the subjects nor the experimenter(s) know which individuals received which treatment until the experiment is completed.

However, subjects must be informed that they will get one of a number of treatments, and must consent to that condition (it would be unethical otherwise).

How do you account for the placebo effect in the treatment of migraine when one treatment is acupuncture (needle-based) and the other treatment is a medication (taken by pill)?

Efficacy of acupuncture for migraine prophylaxis: A single-blinded, double-dummy, randomized controlled trial

doi: 10.1016/j.pain.2011.04.006

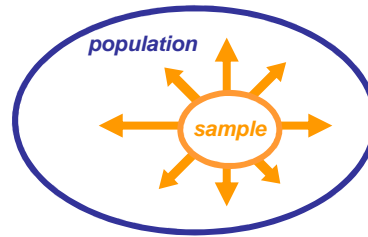
Blinding was implemented by means of **double-dummy** with verum acupuncture plus placebo medication in the acupuncture group and flunarizine plus sham acupuncture in the control group. The follow-up assessors and statisticians, who were uninvolved in clinical management, were blinded throughout the study. Due to the procedure of the acupuncture technique, acupuncture practitioners in this trial were unable to be blinded.

The sham points were chosen to be unrelated to headache treatment but to have otherwise the same number and same needle insertion method as the acupoints intended for the treatment of headache.

The appearance of the placebo medication was exactly the same as that of flunarizine.

Lack of realism

Conclusions apply only to individuals like the ones studied in the specific conditions of the study.



Carcinogenicity studies administer high doses of a potential carcinogen to lab rats. Results don't always apply to humans (e.g. saccharin delisted in 2000).

Drugs That Work In Mice Often Fail When Tried In People, NPR April 10, 2017
www.npr.org/sections/health-shots/2017/04/10/522775456/drugs-that-work-in-mice-often-fail-when-tried-in-people

- Mice/rats are not people
- Lab mice/rats are highly inbred
- Lab mice/rats are kept in unrealistically identical conditions



FDA requiring lower recommended dose for certain sleep drugs containing zolpidem (Jan. 10, 2013): "Since women eliminate zolpidem from their bodies more slowly than men, the FDA has notified the manufacturers that the recommended dose should be lowered for women."



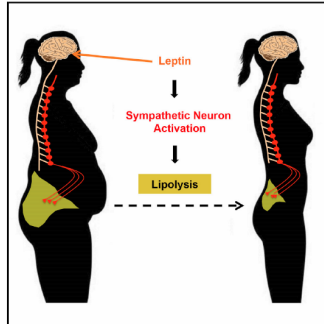
Eighty percent of drug studies that are done in mice are done in male mice. This is true even for diseases such as multiple sclerosis that affect mostly women. Starting January 25, 2016, as a condition of NIH funding, researchers will now have to include female and male animals in their biomedical studies.

www.npr.org/sections/health-shots/2016/02/10/464697905/a-fix-for-gender-bias-in-animal-research-could-help-humans

Sympathetic Neuro-adipose Connections Mediate Leptin-Driven Lipolysis

<http://dx.doi.org/10.1016/j.cell.2015.08.055>

Graphical Abstract



Authors

Wenwen Zeng, Roksana M. Pirzgalska, Mafalda M.A. Pereira, ..., Gabriel G. Martins, Jeffrey M. Friedman, Ana I. Domingos

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dominan@igc.gulbenkian.pt

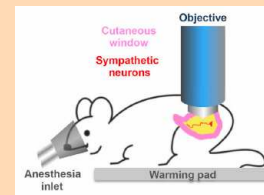
In Brief

The lipolytic effect of leptin is mediated by sympathetic neurons that innervate adipocytes, forming neuro-adipose junctions that directly mediate fat breakdown. Anti-obesity strategies targeting the sympathetic neurons in fat have the potential to circumvent central leptin resistance.

This study found experimental evidence of a lipolytic (fat burning) effect of leptin in mice.

Do you think that the “graphical abstract”:

- A. provides an accurate summary.
- B. isn't detailed but it is reasonable.
- C. is grossly misleading.



Mice

DBH KO mice were kindly provided by Steve Thomas at the University of Pennsylvania. *Adip1^{-/-}2^{-/-}* and *Adip3^{-/-}* were kindly provided by Bruce Spiegelman at Harvard Medical School. *TH-Cre, Rosa26-LSL-ChR2-YFP* (Stock No. 012-569; Daou et al., 2013), *Rosa26-LSL-DTR*, and C57BL/6J mice at 6–10 weeks old were purchased from The Jackson Laboratory. Animal procedures were approved by the ethics committee of Instituto Gulbenkian de Ciência and the Institutional Animal Care and Use Committee of Rockefeller University.

Some common experimental designs

In a **completely randomized experimental design** individuals are randomly assigned to groups, then the groups are assigned to treatments completely at random.

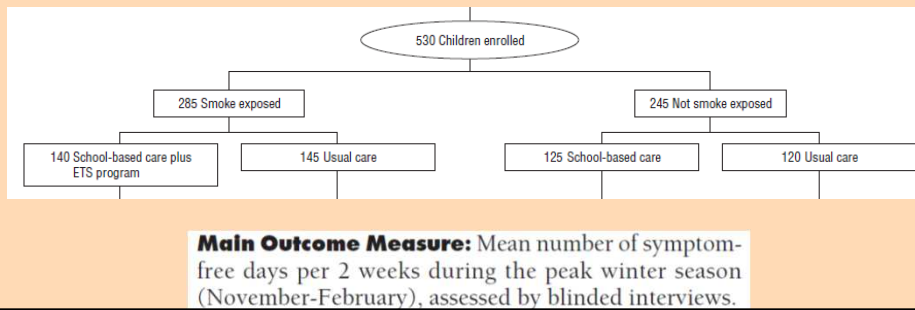


In a **block design**, subjects are divided into groups (blocks) before randomization to study possible differences between the blocks.

The assignment into blocks is NOT random.

The randomization occurs inside each block, offering greater control over the random process.

Study of asthma treatment in school-aged children



Repeated measures (cross-over) and matched pairs designs:



- Choose **pairs of subjects** that are closely matched (like twins). Within each pair, **randomly assign who** will receive which treatment.

- Or give the two (or more) treatments to **each subject over time**, in *random* order, so we have repeated measures for each subject.

Each subject is given two chili bowls: version A and version B. Subjects eat and rate both versions.



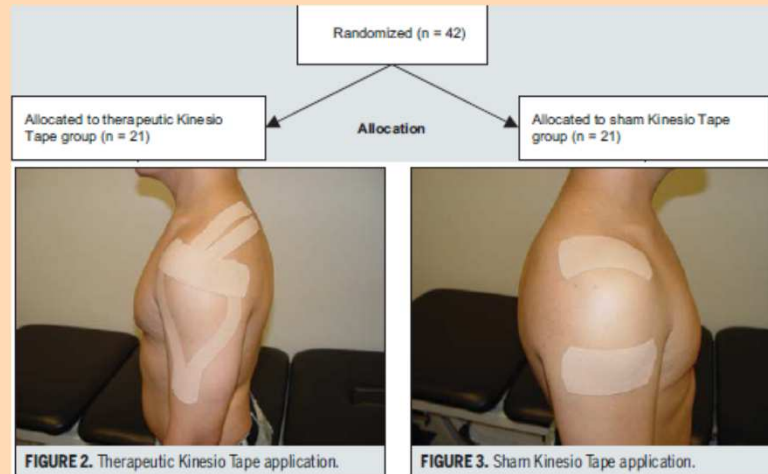
- Half of all subjects are given version A first then version B.
- The other half are given version B first then version A.



The Clinical Efficacy of Kinesio Tape for Shoulder Pain: A Randomized, Double-Blinded, Clinical Trial

doi:10.2519/jospt.2008.2791

A: cross-sectional survey
B: case-control study
C: completely randomized experiment
D: matched pairs experiment
E: anecdotal evidence



Does oligofructose consumption stimulates calcium absorption?

Healthy adolescent males took a pill for nine days and had their calcium absorption tested on the ninth day. The procedure was repeated three weeks later. Participants received either an oligofructose pill first or a control sucrose pill first. The order was randomized and the study was double-blind.



Fractional calcium absorption data
 (in percent of intake) for 11 subjects:

Control	Oligofructose
78.4	62.0
76.6	95.1
57.4	46.5
51.5	49.4
49.0	89.7
46.6	43.8
44.2	50.3
42.9	51.6
37.2	66.6
34.1	52.7
24.6	54.0

A: cross-sectional survey
B: longitudinal cohort
C: case-control study
D: completely randomized experiment
E: matched pairs experiment



- Wakefield 1998 Lancet paper
 - 12 children, all personally referred
 - alleges a temporal association between MMR vaccination and autism
 - suggests a possible causal effect

- No other scientific evidence in support since
- Lengthy journalistic investigation revealed fraud
 - Financial and legal conflicts of interest for author and several participants
 - Manipulation of the symptoms timeline to suit the argument
 - Modification of lab results to suit the argument

BMJ 2011; www.bmj.com/content/342/bmj.c5347.full

As Measles Surges In Europe, Officials Brace For A Rough Year

April 3, 2017 - 3:37 PM ET

MICHAEL LEON DOUGLEFF

Measles In 2017: Suspected And Confirmed Cases By Country

