



# New therapies : How do we know what we know and why should we care?

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# Disclosures

- Research funding

- NIH
- Ultragenyx
- Biomarin
- Takeda
- Aeglea
- Alexion
- Moderna
- Mereo
- Stealth
- Kaleido
- Synlogic
- LogicBio
- Arcturus
- Hemoshear

- Consulting

- Biomarin
- LogicBio
- Sangamo
- Orphan Labs
- Moderna
- Applied Therapeutics
- Homology
- Agios
- Sanofi
- Axcella Health



## A quiz

Everyone has been “talking” on the internet about a new supplement. It’s available over the counter and from a variety of suppliers. Some families have tried it and rave that it gives their child more energy. Others have questioned its effect. There doesn’t seem to be much published information in the medical literature on the compound. Which statement best describes your feelings on trying the new supplement?



# Question

- I have to try it because it might make a difference
- I'll do some more reading and make up my mind about trying it after that
- I'll ask my doctor his/her opinion on trying the new supplement
- I can't see how something like this will work so I won't try it
- I would be very interested in participating in a clinical trial







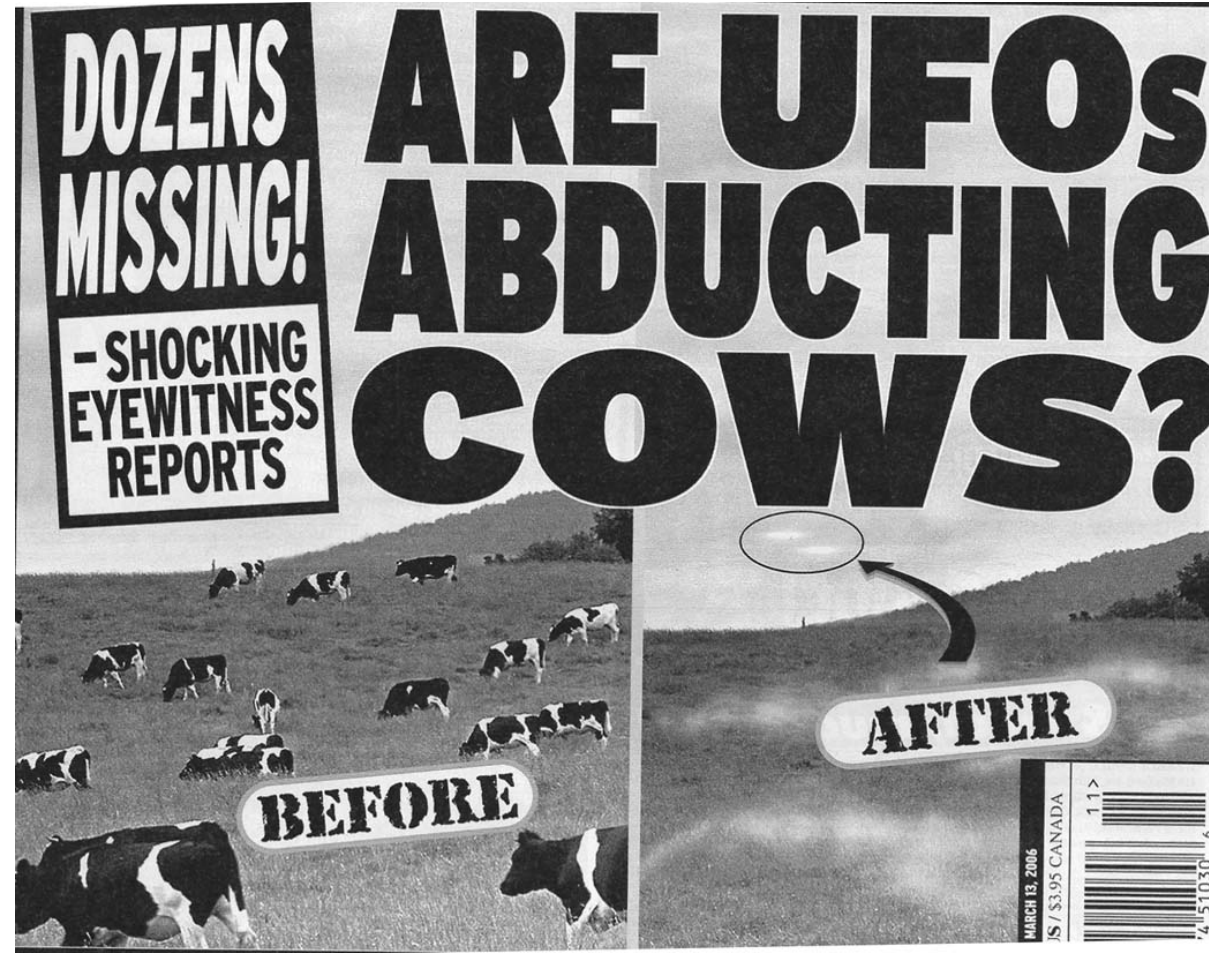
# Answer

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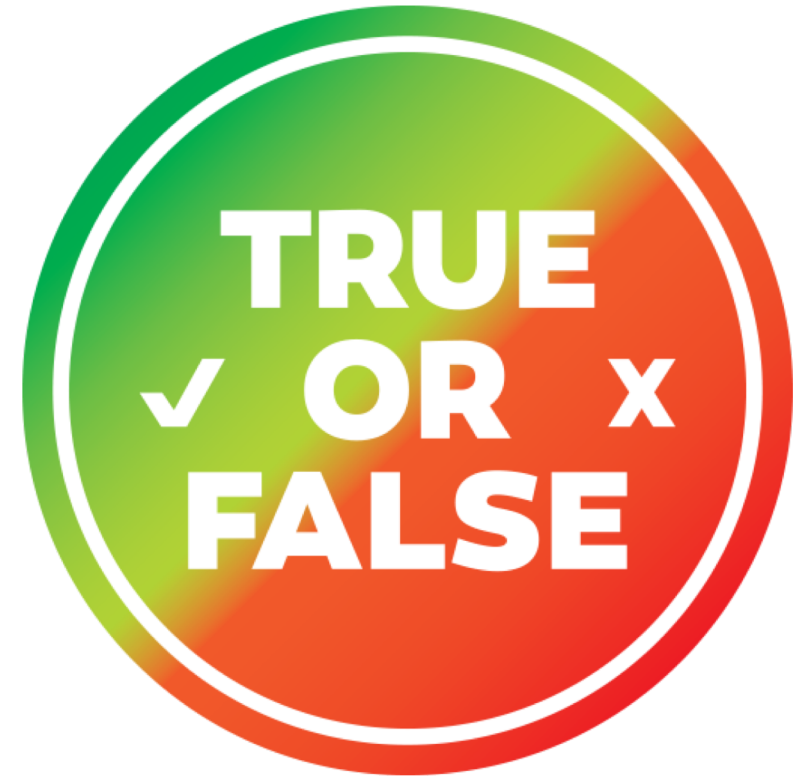
# Evidence based medicine





Anonymous

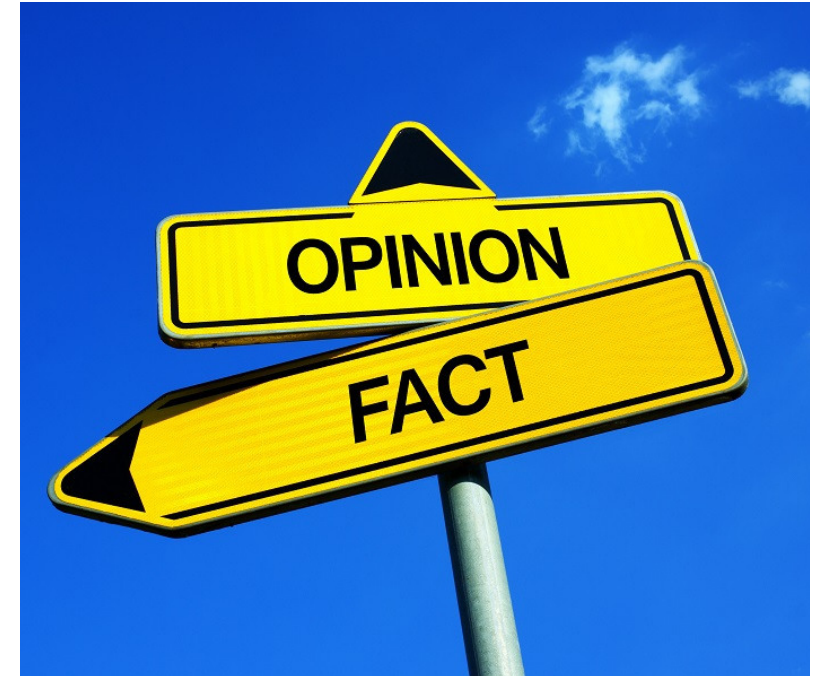
“The plural of ‘anecdote’  
is not ‘data’ ”





# Levels of medical evidence

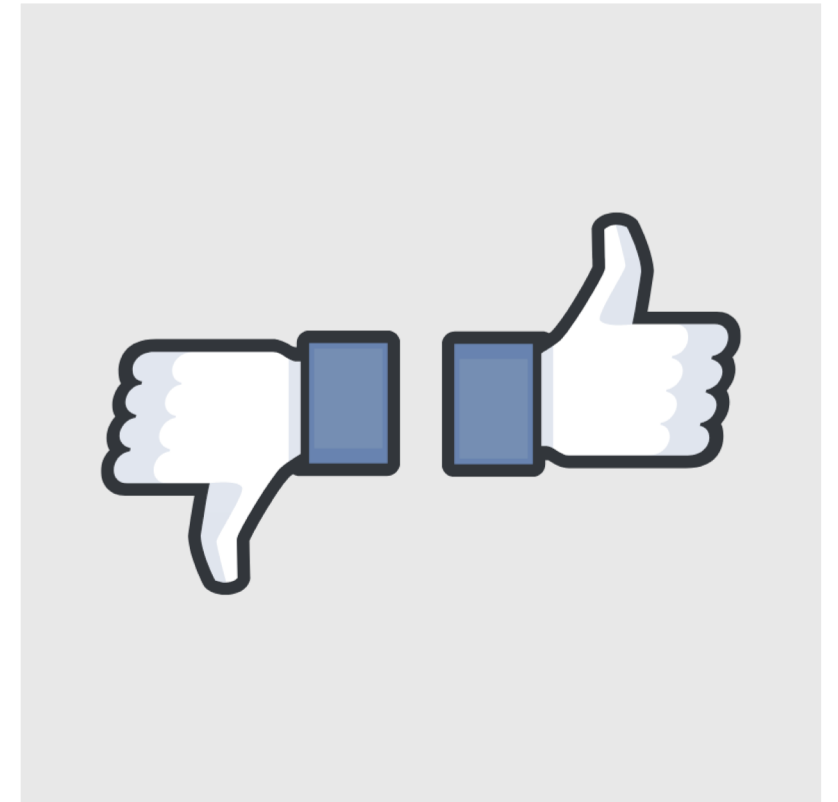
- **Level I:** Randomized control study
- **Level II-1:** Controlled trials without randomization
- **Level II-2:** Cohort or case control analytic studies, preferably from more than one group
- **Level II-3:** Multiple series with or without the intervention. Dramatic results in uncontrolled trials
- **Level III:** opinions of respected authorities, based on clinical experience, descriptive studies, or reports of expert committees





# Recommendation categories

- **Level A:** Strong evidence that benefits substantially outweigh the potential risks.
- **Level B:** Fair scientific evidence that benefits outweigh the potential risks.
- **Level C:** Balance between benefits and risks is too close for making general recommendations.
- **Level D:** Risks outweigh potential benefits..
- **Level I:** Lack of evidence. Risk versus benefits cannot be assessed





# Phases of a Clinical Trial

- **Phase I:** Drug metabolism and dose-ranging studies in healthy volunteers.
- **Phase II:** Blinded or open-label studies in the target population for safety and efficacy.
- **Phase III:** Randomized, double-blind, placebo-controlled study in target population for safety and efficacy.
- **Phase IV:** Post-marketing study in target population at large.







# Childhood cancer survival

- Nearly uniformly fatal in 1960
- Current overall 5 year survival rate 75%
- Some cancers with nearly 100% cure rate





# Children's oncology group

## CureSearch

Children's Oncology Group



### OUR MISSION

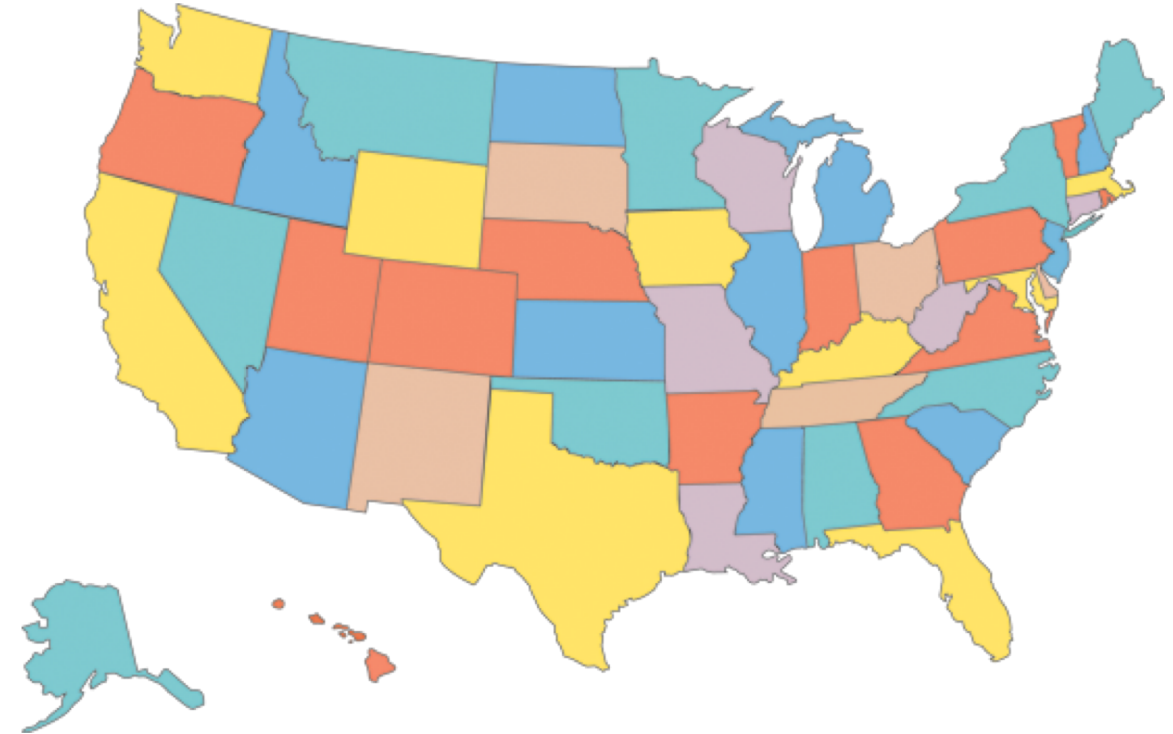
To cure and prevent childhood and adolescent cancer through scientific discovery and compassionate care.





# National studies

- All individuals with rare diseases should have access to standard of care
- All should be enrolled in a clinical study to compare standard of care with one other variable
- Constant feedback to coordinating centers to incorporate successful changes and discard unsuccessful





# National studies: a partnership





# Misplaced beliefs

- “It won’t hurt and it might help.”
- “I don’t want (my child) to be a guinea pig.”
- “I want (my child) to get the real treatment, not the placebo.”





# Why should you participate?

- Clinical trials provide the **ONLY** mechanism for obtaining FDA approval for new treatments
- There are **NO** FDA-approved treatments for most rare/genetic diseases
- **NO** patients = **NO** trials = **NO** proven treatments





# Participating in a clinical trial

- Know its purpose, generally and specifically
- Know the logistics
  - Travel
  - Hospitalizations
  - Procedures
- Know the investigators
- Understand the consent form
  - procedures, risks, benefits
  - right to refuse/withdraw without penalty
  - compensation
- **Participation = Partnership = Mutual trust**







# A bumpy ride







# Making it





Just do it!







Thank you!





# Kelly Jackson

