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Clinical Trials

The Good, the Bad and the Ugly



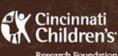
The Good

- May not be good in an absolute sense, but at least definitions are a start
- Standard definitions help create a level playing field that allows discussion of other issues
- This is not meant to be inclusive, but practical in its application



Many Words, More Definitions

• Investigator	• Initiator
• Scientist	• Sponsor
• Researcher	• Funder
• Inventor	• Regulator
• Owner	• Research
• Independent	• Industry
• Collaborator	• Monitor



The Idea

- Research is the process of answering a specific question
- The person/team that develops a specific way of attempting to answer a question is the investigator (or if done by a team, the investigation team)
- This idea is said to be investigator initiated



Therefore...

- The initiator having come up with the idea, could be a researcher or investigator (someone who does research or investigates) and/or scientist (someone who does science)
- A collaborator is someone who helps
- Independence discusses how free someone is...



Semantic Confusion

- Will you sponsor our team for Cincinnati Walks?
- I can sponsor your application for membership for the Pediatric Academic Societies meeting.
- You need someone to sponsor your research project.



Sponsor vs. Funder

- Funder – the one who provides the resources to conduct the research
- Sponsor is the person, institution or organization that has the responsibility for regulatory compliance
- It is possible to have different roles on different projects



Officially, by FDA (1)

- Sponsor means a person who takes responsibility for and initiates a clinical investigation.
- The sponsor may be an individual or pharmaceutical company, governmental agency, academic institution, private organization, or other organization.



Officially, by FDA (2)

- The sponsor does not actually conduct the investigation unless the sponsor is a sponsor-investigator.
- A person other than an individual that uses one or more of its own employees to conduct an investigation that it has initiated is a sponsor, not a sponsor-investigator, and the employees are investigators.



Officially, by FDA (3)

- A Sponsor-Investigator is an individual who both initiates and conducts a clinical investigation and under whose immediate direction the investigational drug is being administered or dispensed.
- For administrative reasons, only one individual should be designated as sponsor.



Monitoring

- Monitor – an individual designated by a sponsor to oversee the progress of an investigation
- Monitoring – a continuous quality assurance program that ensures following GCP and data quality
- DMC or DSMB – group that oversees data and determines if the study should continue



The Bad

- Funding for research is always an issue
- Funding comes from the government, industry, foundations, institutions and investigators themselves
- Without funding, research is severely limited in what it can accomplish

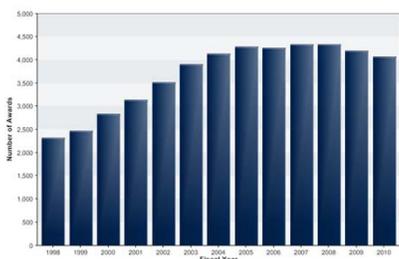


The Ugly

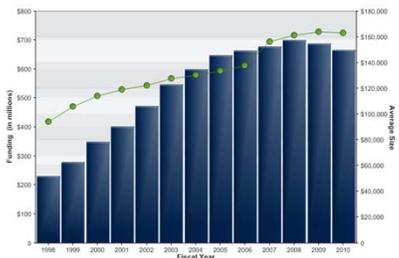
- Research funding is becoming more and more scarce
- NIH is projecting less growth
- The expectations and regulations are becoming greater
- Research is increasingly more necessary as we strive to change the outcome

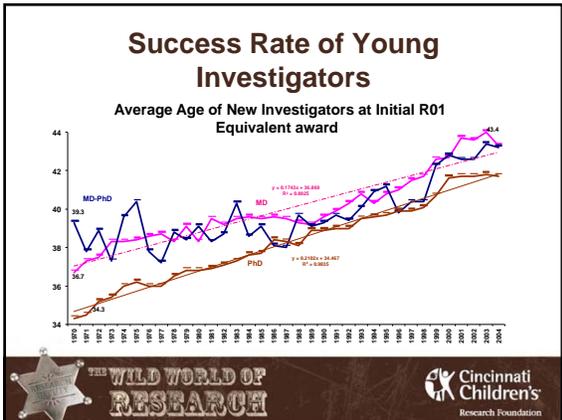


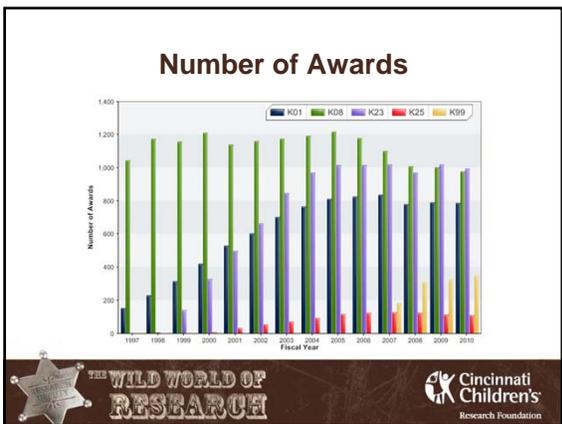
K23 Awards

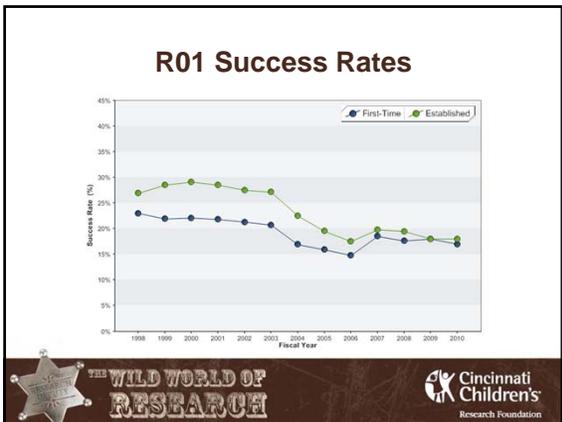


K23 Funding







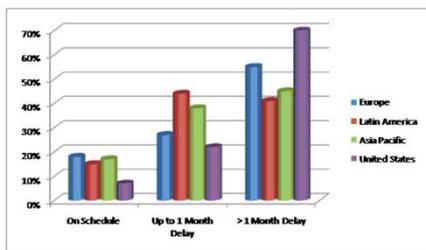


Recruitment Is Key

- Delays in recruitment for clinical trials account for an average of 4.6 months lost per trial
- This is an annual cumulative loss of 26 years, on average, across all drug development.



Clinical Trial Delays



Perspective

- 10 years of bench study and laboratory animal work to reach the point of testing for safety and effectiveness in humans
- Americans spend ~ \$5,000/yr on health care.
- Less than \$20 of that is invested in research to prevent, treat and cure diseases and disabilities.



What Does It Take

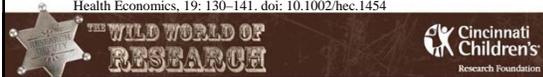
- Approximately one in 50 drugs in pre-clinical testing prove safe and effective enough to be tested in a clinical trial
- One in five drugs that enter clinical trials will prove safe and effective enough to be approved
- All together, that is one in 250 drugs that started as a good idea, make it



Cost and Benefit

- Cost to bring a new drug to market:
- \$1,098,000,000
- The cost of pediatric trials increased nearly eight fold from 2000 to 2006.
- New medicines generated 40% of the gain in life expectancy over the past 25 years

Adams, C. P. and Brantner, V. V. (2010), Spending on new drug development. Health Economics, 19: 130-141. doi: 10.1002/hec.1454



Opinion of Funding

- Currently six cents of every dollar spent on Health Care in the US is spent on medical research
- When polled:
 - 55% would like to see it increased
 - 14% believe it is adequate
 - 4% think it should be reduced
 - 27% percent did not know



Active Investigators

- Active investigators in the US has declined 3.5% annually since 2001
- Active investigators outside the US has increased 13.5% each year during that time.



Some Thoughts

- Healthcare is complex and will always need new and better ways to achieve improved results
- Research is the only way to answer those questions and develop the path to the future
- It will require innovation, perseverance and the highest quality



Advantages

- Reputation of CCHMC is stellar
- Institution has been and will continue to plan for future growth
- Adaptation is the way forward



Questions?



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RESEARCH**



Cincinnati
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