Defending the Dream:
The Other Risks to Drug Development

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Evolving Negative Views of Clinical Trials

a. Money over safety
b. Drugs not effective
c. Not concerned about individuals

"Trial and Terror
It was the stuff of nightmares. Six men volunteered for a clinical trial to test a new drug and within minutes were fighting for their lives…. the race to develop life-saving medicines ... and the grave risks that can be involved"
Guardian 3/19/2006

The Social Media Matrix

And Continuing Issues

The Importance of a Well-Crafted Informed Consent

Participation is voluntary.

Special considerations with children.

This is an experimental drug.

There may be side effects; some of which are unknown.

Including death…

Or life under circumstances you may find unacceptable.

This may not cure or even help you.

The Power Patient Outreach

Example: BioMarin, *RareConnections*™

http://www.biomarin.com/patients/biomarin-rareconnections

Example: Alexion, *Uncommon Strength*.

http://www. uncommonstrength.com

Sarepta: an Example of the Power of Affinity Groups

"Patients and their advocates were uniquely positioned to give valuable input to drug developers and the FDA and that their voice should not — and would not — be ignored at the agency. "I can assure you that the commitment to patient involvement in the system of product development and evaluation is deep and fundamental across the [FDA]," Dr. Robert M. Califf, M.D. FDA commissioner." (medscape medical news): 10/19/2016

Risk Management, Insurance, Crowd Control

Risk Management in Clinical Trials

Insurance against lawsuits

Pro-active management of social media and public perception.

Further Reading..

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- Chin, William W. (2015) "A Delicate Balance -- Pharmaceutical Innovation and Access" New England Journal of Medicine;
- O'Sullivan, B.P. , et. Al.(2013) Pricing for Orphan Drugs: Will the Market Bear what Society Cannot?" JAMA
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