



INSTITUTE OF MEDICINE

Combination Investigational Cancer Therapies: Patient Advocate Perspective

Jane Perlmutter

Patient Advocate

janep@gemini-grp.com



Questions



1. Whose perspective?
2. What's the problem
3. What patients/advocates want?
4. What are patient/advocates concerns?
5. How can advocates help?



Who's Perspective?



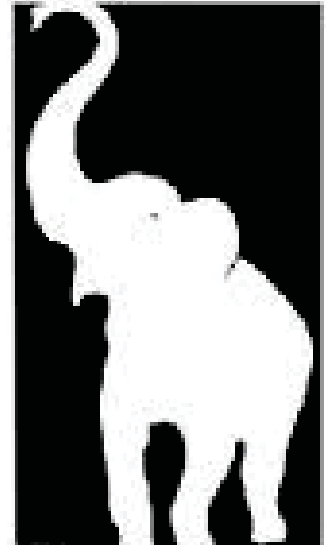
What's the Problem?

- The biology of cancer is very complicated
- While technology keeps advancing, personalized medicine is still mostly a promise
- Targeted agents:
 - Don't work as well as hoped—both magnitude and durability of response
 - Are sometime more toxic than expected
- Regulation is confusing and/or interpreted very conservatively
- There is great need, but limited incentive to collaborating



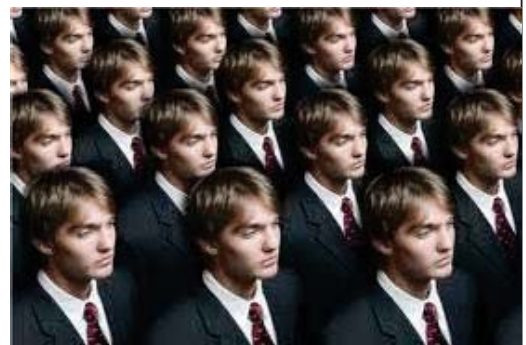
What are the White Elephants?

- Its not just the genome
 - www.genomicglossaries.com/content/omes.asp for dozens of other 'omics
 - Epigenome
 - Junk DNA
- Its not just the tumor
 - Microenvironment
 - Somatic genome
- Stem cells



What do Patients/Advocates Want?

- ***We all want the same thing***
 - Highly effective therapies
 - Few side effects
 - Manageable costs



What do Patients/Advocates Want?

- ***We're each unique***

- Trade offs between length and quality of life
- Trade offs among type, severity, and length of toxicities
- Ability to pay
- Health literacy
- Need for information
- Opinions and approaches



What do Patients/Advocates Want?

On the One Hand



On the Other Hand

- | | |
|---|--|
| <ul style="list-style-type: none">• Few care if treatment includes:<ul style="list-style-type: none">– One or multiple agents– One or multiple modalities• As long as it works, and is not more toxic | <ul style="list-style-type: none">• We increasingly understand that single agents are of limited value• We have been impressed by the significant success of AIDs cocktails |
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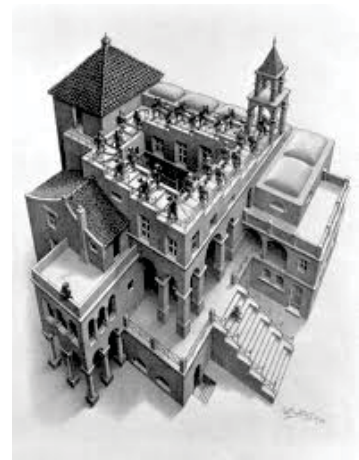
What are Patients/Advocates Concerns?

- Increases in survival have not be significant
- Combination therapies are often more toxic
- Targeted therapies are added to chemotherapy
- Personalized medicine causes delay in treatment due to time consuming testing
- Tests are not always accurate
- Multiple biopsies are burdensome
- Cost of treatment is out of control



Why is it Taking So Long?

- Patients/advocates increasingly understand the complexity of the cancer biology and its challenges
- We increasingly hear the hype of:
 - Personalized medicine
 - More, better, cheaper genomic technologies
- As we loose more patients, we loose patience



How Can Advocates Help?

- Understand and help others understand the complexity of cancer biology
- Encourage and support innovation
- Advocate for policy change—if we understand and believe in the change
- Demand collaboration among all stakeholders



Challenge to Other Stakeholders

- Think and act outside of the box
- Stop doing things that are tried and true, but will not get us what we all want
- Share tissue and data
- Work together on trials
- Clarify impediments
- Let us know how we can help

