What patients think of ‘molecular diagnostics’

IOM
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Promises: are we there yet?

Few successes

HER2

Real life issues

Most drugs stop working

“...nearly a decade since distinct breast cancer genotypes... 5 years since the first multiparameter gene expression assays...

Although there is compelling evidence regarding the clinical validity of these assays in providing prognostic information about distant disease recurrence, there is little information about their clinical utility—that is, how does the test result influence clinical decision making, and do patients benefit from that change?”

- Sporano et al. JCO 2010

Defining the Clinical Utility of Gene Expression Assays in Breast Cancer: The Intersection of Science and Art in Clinical Decision Making
We HAVE to get this right

Data ≠ Knowledge ≠ Results

Dangers of not doing this well....

- Un–validated biomarkers kill products
- What do False +/- do to people?
- Ethnicities?
- Rare cancers?
- Wastes time/$$$, erodes trust, & costs lives

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Who is the real end user?

People  ⇒  Public  ⇒  Patients

People/patients want...

- True prevention, but not at all costs
- To reduce risk of cancer &/or recurrence
- Maintain lifestyle
- Believe the ‘cure’ hype
- A safe system
- Worried well:
  - Lower risk, absolutely!
What do diagnostics mean to people?

- Loss of self, family, culture, community, privacy
- RISK
- Nothing
- Fear
- Vulnerability
- Knowledge
- Family genetics
- Disease
- Hope
- Cost

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RISK
To me (absolutely)
Relative to population

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Which risk strategy is used?

1. Google?
2. Abdication?
3. 2\textsuperscript{nd}, 3\textsuperscript{rd}, 4\textsuperscript{th} opinions?
4. Statistical equations?
5. Gut?
6. Clinical trials?
7. Dice?
8. Family?
For patients, it isn’t about the test!

It’s a life decision, not just medical

1. Extra procedures, pain/suffering?
2. What do test results mean?
3. Something to prevent/lower risk?
4. Hereditary?
5. Will options remain open, or close?
6. What about work, family, social life?
7. Am I protected against misuse?
8. ‘Personalized medicine’???? Where?

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What is ‘clinical utility’ to Aunt Mildred?

Reliability
- Treatment response in <x> cancer + ramifications

Focus on true indicators (i.e. not LVEF)
- Concrete numbers v. spectrum v. variance

Convey test results clearly and simply
- What is most important?
- Quick updates to ALL

Part of the whole
- Other diagnostic tools?

If it’s genetic...
- Give me counseling
CLINICAL UTILITY

Personal guidance

Acceptable v. out-of-range results, relevance of intervention

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Clinical utility?
-or-
PERSONAL UTILITY?
Personal utility = reality

- Getting test results in reasonable timeframe
- Results explained clearly in plain language
- Doctors/HCPs comfortable interpreting test info
- Something you can do about it
- Ability to say yes or no to receiving test results
  - Some people want to know even if no intervention is available
  - ...and some don’t

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Levels of evidence? Value?

People/patients like registries!

CTs + QOL + observational studies + outcomes

Clinical settings useful

Impact on life and family
  - Physical
  - Financial
  - Emotional
  - Social
  - Etc.
Don’t make the screening mistake

Clear messages needed, not confusion
Rethink our approach

- Human body = integrated circuit
- Pay attention to ALL at once
  - Gen- + prote- + metabol- + other- OMICS together
  - Multiple pathway interactions
  - Microenvironment
  - Immune system
  - Adverse events
  - QOL
- RCTs can’t do it all

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Include past lit & recs, e.g.

- FDA changes to medical device advisory committee deliberation
- Joint NIH-CDC Workshop on Personal Genomics
- Secretary’s Advisory Committee on Genetics, Health, and Society at NIH
  - [http://oba.od.nih.gov/SACGHS/sacghs_home.html](http://oba.od.nih.gov/SACGHS/sacghs_home.html)
- CDC’s Evaluation of Genomic Applications in Practice and Prevention
  - [http://www.egappreviews.org/about.htm](http://www.egappreviews.org/about.htm)
- “Evaluating the utility of personal genomic information”

Key message: Need new INFRASTRUCTURE that enables by clinicians while doing their daily work!
Requests from patients & advocates

1. Honor **true** meaning of “patient-centered”
   - Mean what you say
   - Risk? Be absolute, not relative

2. Resolve IP issues, get to work & **share**
   - Data, biospecimens, updates, results

3. “Nothing about us **without** us”
Thank you...

Patient Advocates
- PAIR advocates (~200)
- SPORE advocates (~220)
- NCI CARRA advocates (~170) and DCLG (15)
- Cooperative Group advocates (~80)
- FDA advocates (~20)
- Many others

Experienced:
- for creating new opportunities

New:
- for fresh ideas & energy

And to those who made a difference before their death

Research teams for
- Dedication
- Efforts
- Collaborations

Thanks for all you do for cancer patients and their families

- For more information, contact Deborah@tumortime.com