



The FDA Role in Rapid Learning for Cancer

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Agenda

- n Current role of FDA in cancer product evidence development
- n Future FDA participation in learning healthcare
 - n Rapid translation of new science
 - n Increased community participation in trials
 - n Rapid evaluation of outcomes postmarket
 - n Comparative effectiveness



FDA Role in Evidence Development for Cancer

- n FDA sets standards for evidence required for medical product (drug, vaccine, device, diagnostic) to get on the market
- n This evidence get developed
- n Areas where there are no FDA-generated evidence requirements (procedures, laboratory developed tests) may often not have a robust evidence base at market—less for learning healthcare to build on
- n Off label uses—common in cancer—often have little formal evidence base



FDA Role in Evidence

- n Cancer: many “accelerated” approvals that require postmarket verification—ongoing clinical trials. Results may modify practice
- n FDA regulates what sponsors can say about the product based on the label. Detailing is currently a principal mode of evidence dissemination in community.
 - n ESA safety changes
 - n K-ras—do these changes occur at an acceptable rate? Probably not.



FDA Role in Evidence: Surveillance

- n FDA may require sponsors to conduct additional studies as postmarket commitments: FDAAA
- n FDA conducts/requires postmarket surveillance—mainly relates to learning about new safety issues
- n Novel approaches—e.g., Sentinel Initiative—are currently being launched by the Agency
 - n Rapid learning about safety outcomes from healthcare data



FDA Role in Evidence Development for Cancer

- n FDA regulates manufacturers of medical products, not the healthcare system
- n Agency has not been highly involved in translational into practice—regulates some of those who are involved
- n New FDA requirements for drugs under FDAAA have impact on healthcare
 - n Restrictions on distribution
 - n Registries, required patient followup, etc



Future Participation of FDA in Learning Healthcare System

- n Fundamental problem in cancer—lack of highly effective diagnostics, preventatives, therapies
- n Need more effective translation of new science to the clinic
- n Critical path initiative: stimulate innovation and improve information
- n Greater partnership between development enterprise and healthcare needed



Challenges in Learning for Cancer

- n Cancers are highly heterogeneous
- n Biological behavior and response to treatment linked to genetic/molecular pathway abnormalities
- n Subsets: is cancer a collection of thousands of different diseases
- n Studying outcomes will become more challenging as more knowledge accumulates



More Community Participation in Trials

- n Time and effort to do trials unacceptable: limits what can be tested
- n Need broader community-based network of investigators
 - n Supportive infrastructure
 - n Links to cancer centers
- n Allow cancer patients to stay in their community
- n Capacity to recruit patients and study outcomes efficiently
- n Useful as more effective options are developed



What Would Community Infrastructure Look Like?

- n Trained clinical trial personnel who go TO the community
- n Structured, convenient, brief training for community practitioners
- n Central administrative support to handle trial paperwork, deal with IRBs, etc.
- n Computerized support of trial documentation



Rapid Evaluation of Outcomes Postmarket: Efficacy

- n Healthcare system needs to learn from use—but need systematized data collection
- n Cancer—multiple off-label uses, slow evidence development
- n Opportunity for community involvement in large simple trials of secondary uses
- n Network of investigators required



Rapid Evaluation of Outcomes

Postmarket: Safety

- n Safety data collection more amenable to record-based ascertainment
- n Ability to do cohort studies, registries, or electronic record surveillance
- n Many safety questions in cancer, however will be closely tied to efficacy, and may required formal studies to address (e.g., use of ESAs)
- n Use of community networks could accelerate learning process



Learning More about Health Outcomes in Cancer

- n Traditional comparative studies take a long time, and are criticized for lack of real world relevance
- n Possibility for large, simple outcome trials evaluating multiple interventions and treatment pathways in community settings.
- n Provide for more extended followup of patient outcomes
- n Would require support for each phase
- n FDA usually accepts results of such studies for purposes of label changes



Summary

- n FDA requirements drive much current evidence development
- n Clearly, current evidence base is seriously limited
- n Evaluating new science brings the need for more studies and more enrolled patients
- n Current paradigm limits capacity



Summary

- n Future learning needs to enlist the community: patients want to be treated there
- n Networks of community investigators feasible if well supported
- n Capacity for development trials and outcome evaluations (i.e., comparative effectiveness)
- n Would require expansion and reconfiguring of existing clinical research structures in the US