Precision Medicine: FDA's Involvement

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Personalized Medicine?

- "Precision" Medicine
  - Companion Dx
  - "Complementary Dx"

  Incorporating:
  - NGS
  - Investigational regulations
  - LDTs

Personalized Medicine Guidances

- Companion Diagnostic Guidance
  - Defines companion Dx, requirement for approval, co-approval of drug and device, labeling
  - www.fda.gov/companiondiagnostics for list of approved CoDx
- Codevelopment Draft Guidance
  - Describes processes, provides explanations, not policy
  - Publication “soon”
- “Investigational IVD Devices Used in Clinical Investigations of Therapeutic Products” Draft guidance (tentative title)
  - Describes Investigator, IRB responsibilities, risk decision making, submission process
- Complementary Diagnostics Guidance
  - Diagnostics that are not required for safe and effective drug use but provide significant information about drug use
  - Writing group forming

Companion Dx

- Companion Dx final guidance issued 2013
  - Definition, requirement for approval, co-approval, labeling, investigational issues
- Evolution
  - Multiple drugs and diagnostics
    - Harder to tell which Dx goes with which drug
  - NGS panels for Dx
  - Comparability in PD-L1 space?

"Blueprint" Initiative

- Workshop: "Complexities in Personalized Medicine: Harmonizing Companion Diagnostics Across a Class of Targeted Therapies"
- Outcome: Industry/professional association project to assess analytical comparability of PD-L1 IHC tests
- FDA is monitoring but not directing
- Information may be available soon

NGS Panels

- Many labs offering Oncopanels
  - Multiple companion targets plus targets that are not directly involved in administration of any approved drug
  - No FDA regulation yet
- Possible outcome
  - Approve panels with CoDx and “send to trial” markers
    - What are the claims?
    - What is the evidence?
      - Guidance on “me-too” approach, panel approach

Precision Medicine Initiative: Modernizing FDA Regulation of Genomic Laboratory Tests

- New regulatory strategies for next generation sequencing
  - Develop and implement ***standards to ensure quality***
  - Develop ***open-source tools*** to help test developers meet standards
  - Promote translation and innovation to advance precision medicine by adopting a ***flexible, dynamic regulatory system***

Evolving Issues

- "Liquid biopsies"/circulating nucleic acids
  - Early detection of cancer?
  - Detect response, relapse, resistance
    - Performance characteristics vary by tissue
    - Sensitivity, specificity?
    - Appropriate use
- Others?