Personalized Medicine

President's Council of Advisors on Science and Technology

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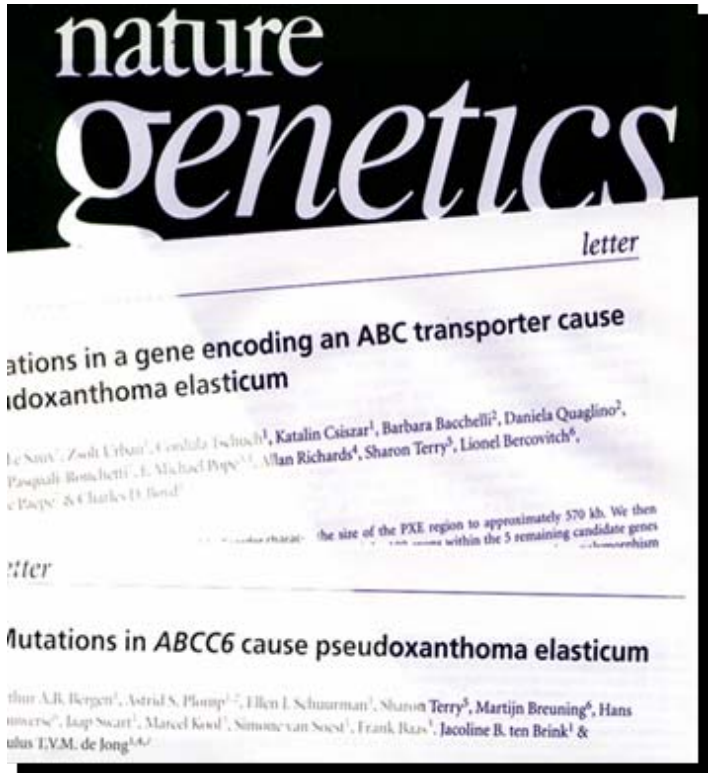
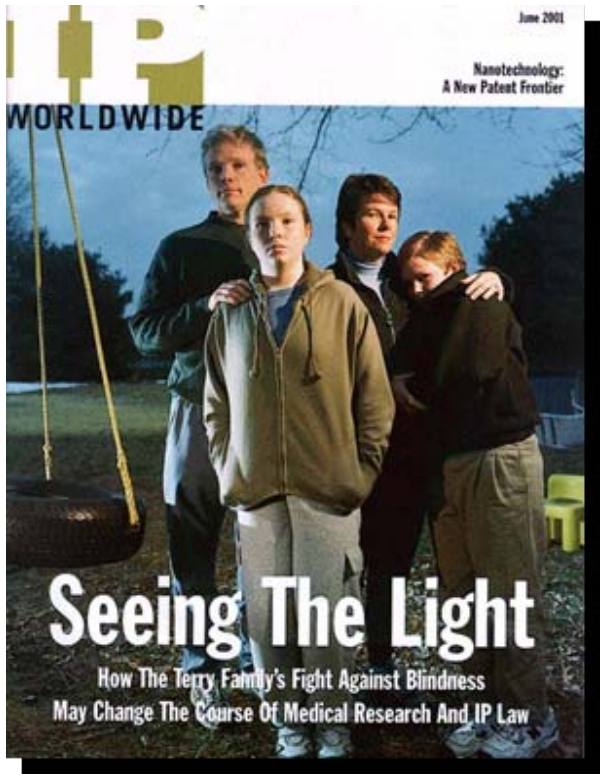
PXE INTERNATIONAL

Gene Discovery



BioBank

Diagnostic testing development via FDA & CLIA strategies



Patent

Licensing & IP management

Creation of a Diagnostic Test - CETT

Network of over 600+ disease specific organizations and thousands of other health related organizations: academic, industry, research, government.

Founded 1986

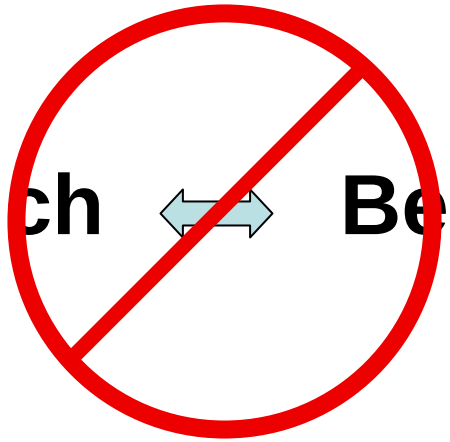
Transform the systems of research and services through the disruptive innovation of advocacy and genetics.

Genetic Alliance BioBank

A New Age of Consumer Advocacy and Translational Research

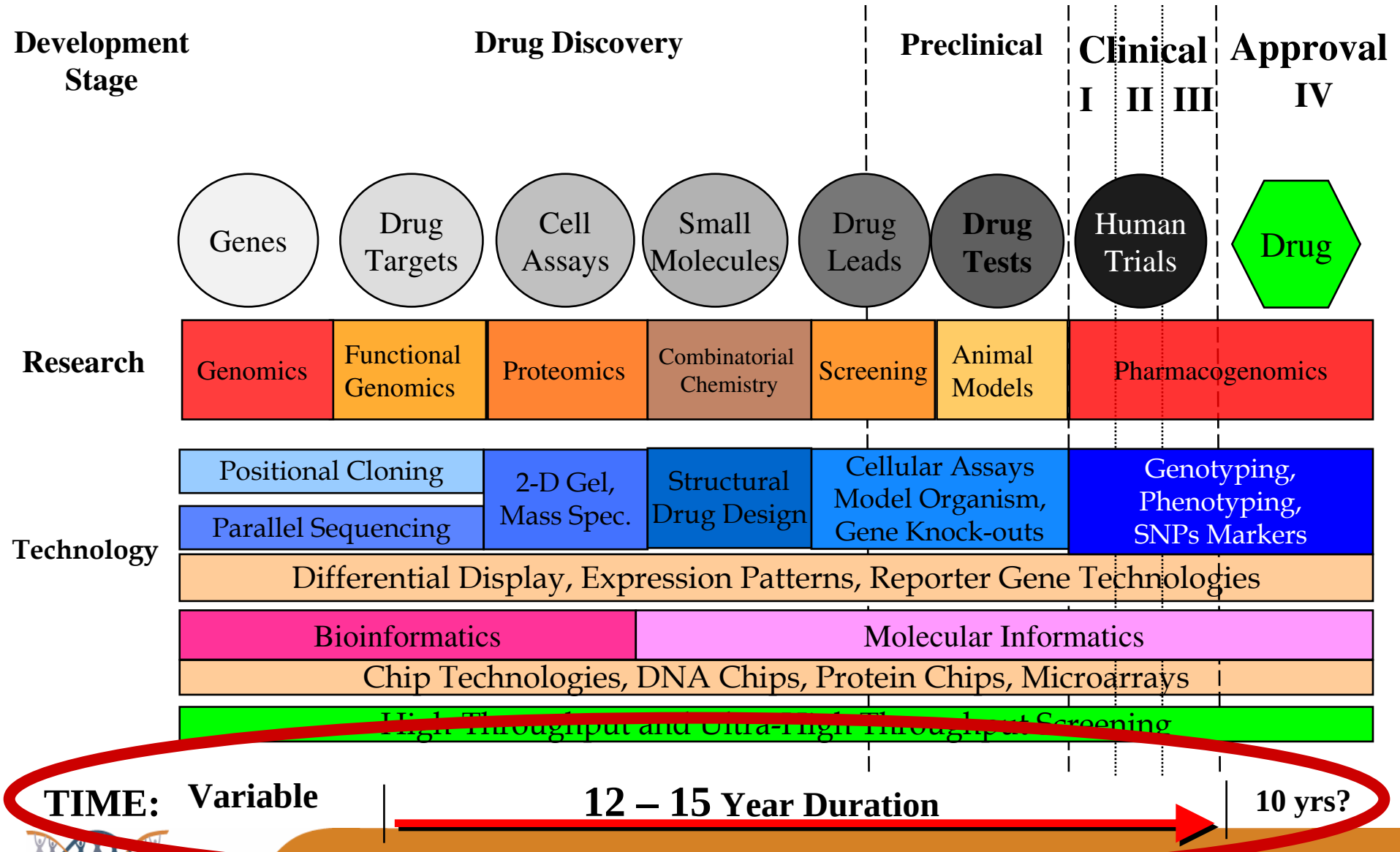
www.biobank.org

What Matters?



Bench ⟺ Bedside ⟺ Practice

Drug Discovery & Development



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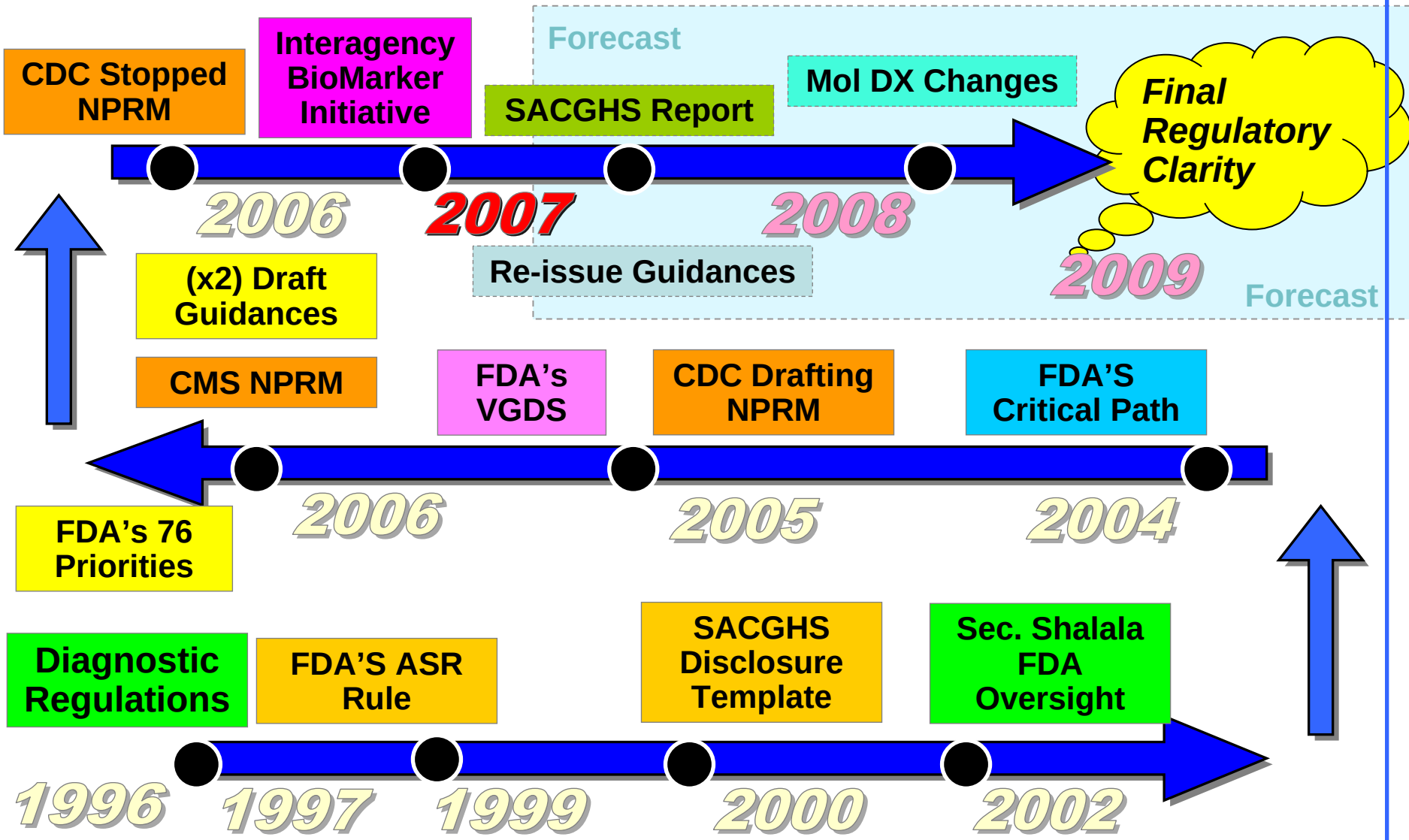
Concepts and Realities of Personalized Medicine

Basic Principles

- **The community believes:**
  - That innovation and quality patient care are the keys to sustainable 21st century healthcare
  - Timely access to new information by physicians and patients is critical to improving the quality of care and providing more personalized healthcare
  - That we must balance regulation and innovation if we are to improve the quality and economics of our overall healthcare system efficiently
  - That consumers will overtake the medical field just as they have in other areas, and demand solutions without wide margins

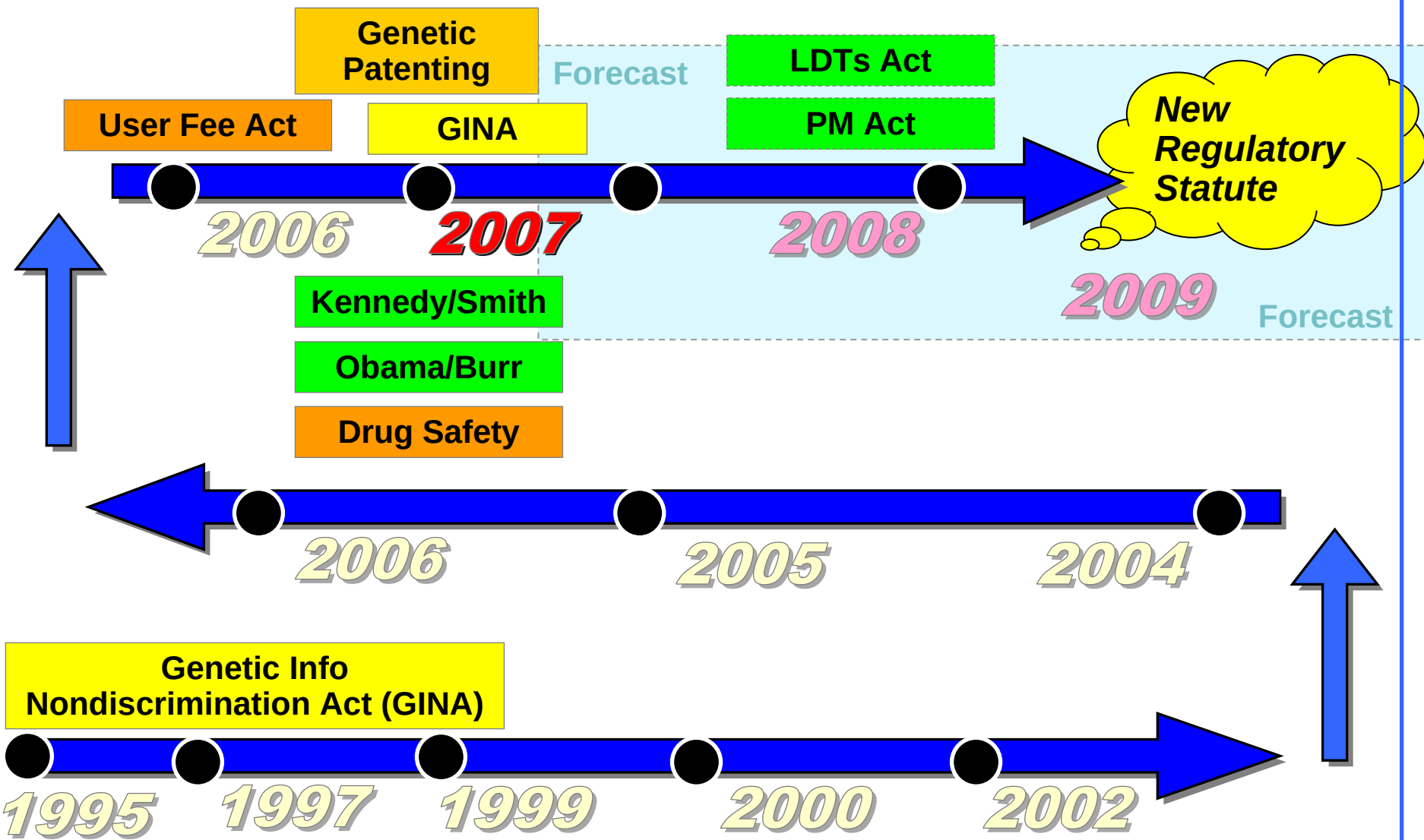
DX Regulation Timeline

Changing Political & Regulatory Landscape



Legislative Timeline

Congressional & Federal Agency Activity



Recurring Themes

Personalized Health Care: striving toward personalized therapies and interventions based on genetic data is creating tensions in the system.

Education: it is of paramount importance that better education materials and vehicles be developed and promoted.

Resource Allocation: equal access to testing and treatments from rare diseases to international and developing world issues.

Public- Private Partnerships: strong support for public-private partnerships. These types of arrangements could go a long way toward relieving the pressures on the current system.

Reimbursement: reimbursement has been called the "ultimate bar", the definitive regulation. But do the payers really understand the value of genetic testing? Is this even the right structure for the healthcare system? Value-based pricing works for drugs, can it work for diagnostics, and ultimately, can it work for the entire system interminably?

Biobanks: they are not regulated, the resource is often not shared. How important are they? Who should maintain them?

World Health: how can the transfer of genomics technology to the developing world be streamlined?

Evidence: how much clinical evidence is enough? Are the pressures to bring tests into the marketplace overriding the scientific need for evidence? Or are demands for evidence unnecessarily slowing down the approval process? All tests cannot be held to a single standard – there are legitimate variables in the need for clinical data. What role does post-market data play in collecting evidence?

IP Models: other industries have wrangled with some of the issues that are currently facing the genomics industry. What lessons have been learned in other industries that can be applied to the genomics industry?

Genetic Discrimination: strong support for the passage of GINA.

Rare Diseases: CETT model has been successful. Can it be expanded?

Role of Patients and the Advocacy Community: considered the bridge between the scientific community and the public. It is crucial that they take great care with the messages that they bring to the public.

Study Design: predictable, well-designed studies, streamline to move tests from research to clinical practice.

Regulatory Authority: who has the regulatory authority for genetic testing? What should be the role of the FDA , CLIA (under CMS), and the FTC? How can the regulatory scheme be coordinated so that it promotes transparency, predictability, and clarity?

Tensions between the Product and the Process: technology takes great leaps but behavior has a slow, iterative rate of change.

Intellectual Property: place for patents within the genetic testing industry. How to craft an intellectual property policy so that it encourages investment while ensuring adequate access to data? Where should the pre-competitive bar be set?

Registries: should they be voluntary or mandatory? What type of data should be deposited? Who should maintain the registries?

Medical Record Aggregation: would the public support large databases, or would privacy concerns override perceived benefits?

Professional Organizations: what role should they play in regulatory oversight? Given that participation in professional organizations is voluntary, how much impact can they have on "bad actors?"

Costs and Value: how do we determine the value of a test?

Risk-Based Regulation: acknowledges the fact that some tests pose more risk to patients and society than others.

Proficiency Testing: critical role of proficiency testing in achieving superior quality control. How to increase the amount of proficiency testing without placing an undue financial burden on small laboratories?

Direct-to-Consumer Tests: distinguish between marketing and testing. How can the public be protected from fraudulent or exaggerated claims?

Test Interpretation: who determines clinical utility?

Conclusions

- NIH put more requirements on funding – require various standards so that basic and translational research is more informative, achieves evidence standards efficiently.
- Discourse with, and responsiveness from, federal agencies that have jurisdiction over genetic testing.
- Coordination of jurisdiction and activities of CMS and FDA, and other relevant agencies.
- Clarity and predictability – current process is not conducive to a growing, or stable, marketplace.
- A risk-based regulatory system is desirable, with the caveat that allowances need to be made for volume.
- Direct-to-Consumer tests need special oversight.

Conclusions, continued

- Public-Private partnerships are desirable as a means for ensuring the pipeline from discovery to tests is efficient and effective.
- Education (at all points in the process) is desirable.
- We need outcomes-data collections and clear evidence bars.
- The industry must have the means to rid itself of “bad actors”, but regulation of the industry should not be based on bad actors.
- A mandatory registry must be established and managed by either a public private partnership or government agency.

Policy Issues

- Innovation Access
- FDA Regulations
- CLIA Oversight
- Reimbursement
- Biomarker Funding
- Biobanking
- Healthcare Reform
- Adaptive Trials
- IP Rights
- Combinations & Hybrid products
- Info Medicine

Genetic Information Nondiscrimination Act

- House - 224 co-sponsors
  - ✓Markup in Education and Labor
  - ✓Markup in Energy and Commerce
  - ✓Markup in Ways and Means
  - ✓Full House vote: 240 - 3
- Senate Vote - 2008???
- President signs - issued an SAP

The significant problems we have cannot be solved at the same level of thinking with which we created them.

- Albert Einstein

Thank You

www.geneticalliance.org