



# **Delivering Value Through Personalized Medicine: An Industry Perspective**

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# What is Personalized Medicine?

- Tailoring of medical treatment to the individual characteristics of each patient
- Ability to classify individuals into subpopulations based on susceptibility to a disease or response to a treatment
- Preventive or therapeutic interventions are concentrated on those who will benefit, sparing expense and side effects for those who will not

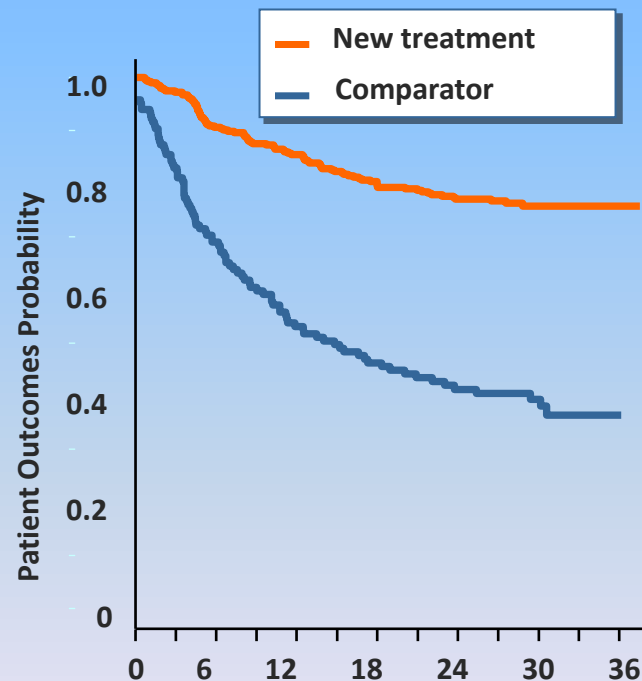
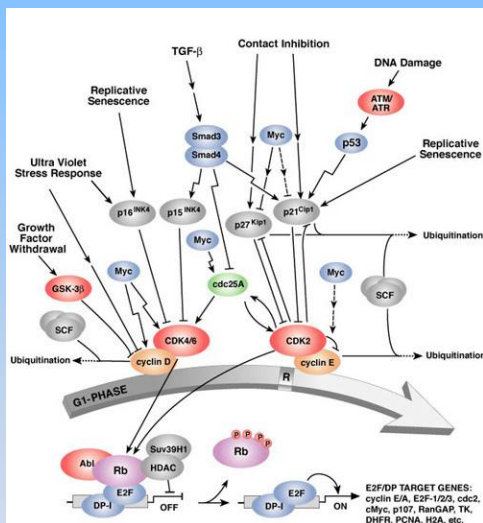
# What Are We Trying to Accomplish?

Delivering the right treatment, to the right patient, at the right time

*Right Drug*

*Right Patient*

*Improved Outcomes*



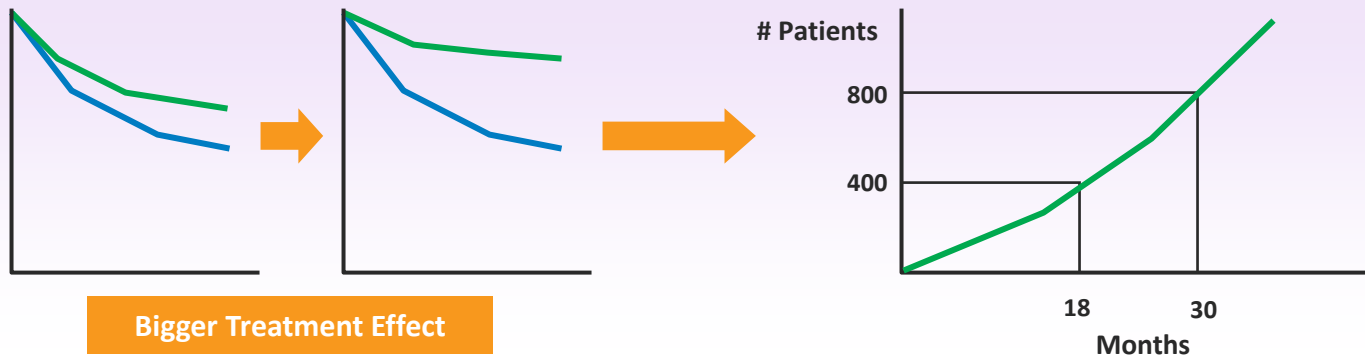
*Drug targeted to specific oncogene or aberrant pathway driving the specific cancer*

*Patient identified through molecular profiling*

*Significant improvement in patient outcomes*

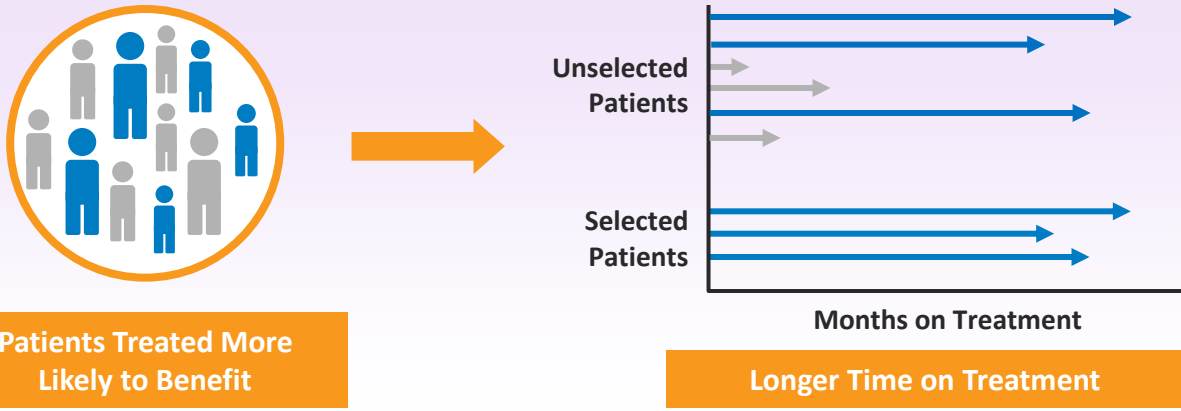
# The Impact of Personalized Medicine

## Benefit to Clinical Development



More Efficient Trial Design  
+  
Improved Efficacy of Medicines Developed →  
Smaller, more efficient Ph 3 Trials

## Benefit to Patients



More Dramatic Effect in Treated Patients →  
Clear Value of Treatment

# Everyone Benefits

## Industry



### Pharmaceutical Manufacturer

- Keener ability to identify patients most likely to respond
- Increased productive and efficient drug development

### Diagnostic Developer

- Increased opportunity to innovate
- Greater acceptance and use of diagnostic testing within medical community

## Regulatory Agencies



- With narrower, better defined patient populations, there may be an enhanced ability to protect patient's health, ensure safety, quality and efficacy of health products
- More specific information about medicines to help balance the benefit risk profile
- Increased assurance that patients most likely to benefit from a treatment will be those to receive it

## Four Ps



### Patients

- Provides directed care with optimal therapies
- Receive most appropriate therapy early in their disease process

### Payors

- Provides clear evidence for which to base reimbursement decisions
- Avoids unnecessary costs

### Physicians

- Enables the prescribing of therapies to pts most likely to benefit
- Reduces trial-and-error prescribing

### Policymakers

- Increased ability to formulate policy to benefit specific populations

# Our Personalized Medicine Approach

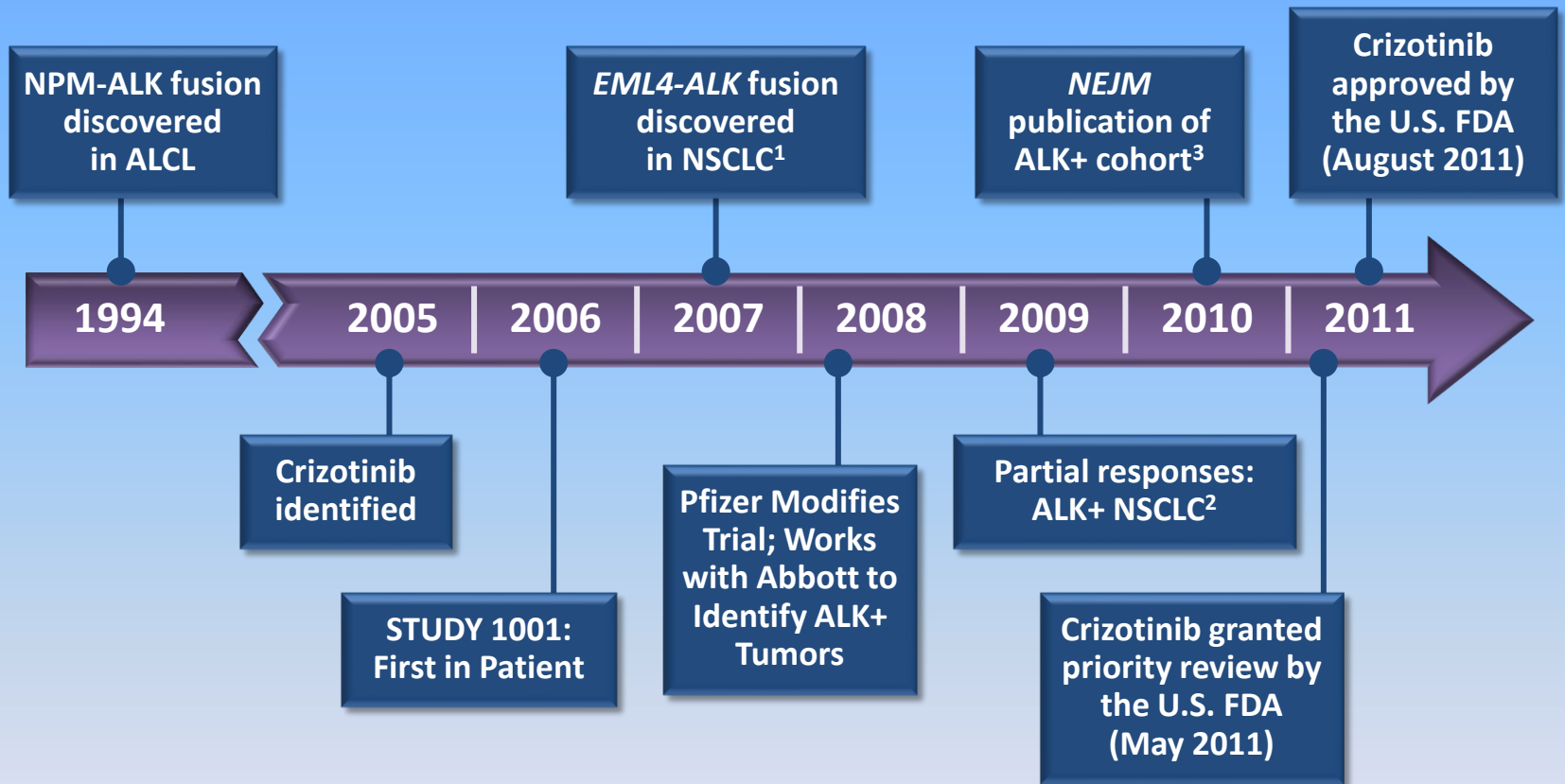
- Scientific research and clinical development have **advanced significantly in recent decades**; we now have a deeper understanding of several diseases at a far deeper biological and molecular level.....
  - **30 years ago**, lung cancer was divided into small-cell and non-small cell cancer
  - **Today**, we understand it to be a group of heterogeneous diseases with different molecular origins
- Pfizer has recognized the potential of this **new way of thinking**—our R&D efforts now focus on:
  - Identifying molecules that block genetic biomarkers driving certain diseases—**and shifting focus from a one-size-fits-all approach**
  - Delivering medicines and vaccines in a more tailored and targeted approach

## *Our Goal*

To develop therapies with greater safety and efficacy that will be approved for smaller, well-defined patient populations and will ultimately impact disease and improve patient outcomes



# XALKORI: Timeline from Compound Identification, Target Discovery and Clinical Results



<sup>1</sup> Soda M, Choi YL, Enomoto M, et al. *Nature*. 2007;448(7153):561-566.

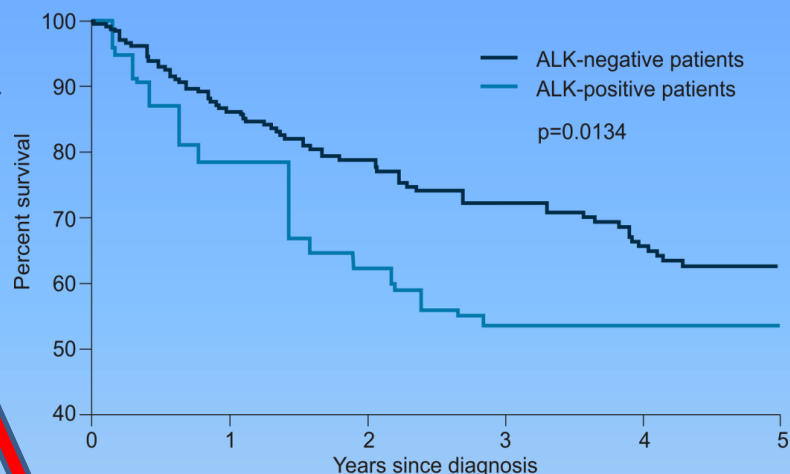
<sup>2</sup> Kwak EL, Camidge DR, Clark J, et al. *J Clin Oncol*. 2009; 27:Suppl:148s.Abstract 3509.

<sup>3</sup> Kwak EL, Bang YJ, Cambridge R, et al. *N Engl J Med*. 2010;363:1693-1703.

# Evidence Development within a New Drug Development Paradigm for Payors and Other Stakeholders

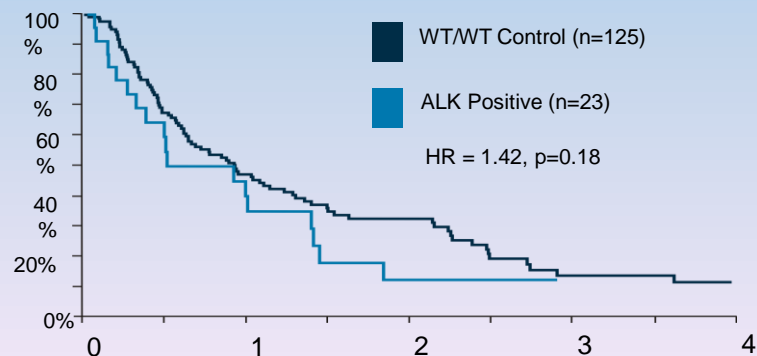
- Understanding the unmet need
  - Molecular epidemiology studies, chart reviews, natural history studies, case studies
- Ability to deal with unique trial designs
  - Single-arm, open-label
  - Cross-over, stacked therapy,
  - Adaptive designs
- Shortened development cycles
  - Availability of Tx prior to ph3 completion
  - Fewer patients exposed, limited data elements and endpoints for approval and reimbursement submissions
- Applying companion diagnostics
  - Consideration for cost of screening and pt. identification
  - Addition of new testing platforms - multiplexing or multiple marker tests

Retrospective analysis of never-smokers with lung adenocarcinoma of all stages



Yang et al. Journal of Thoracic Oncology 2012;7 (1):90-97

Retrospective case-matched survival analysis of ALK-positive patients who did not receive crizotinib



Shaw A et al., Lancet Oncology, Oct. 2011; 12 (11), 1004-1012





# XALKORI: Example of Personalized Medicine in Action

- **Efficient Development**
  - Time from discovery to approval exemplifies Pfizer's new approach and commitment to drug development
  - FDA's approval of Xalkori marks the first new lung cancer drug approval in 6 years
- **Well Defined Patient Population with Improved Efficacy**
  - 3%-5% of advanced NSCLC patients who test positive for the anaplastic lymphoma kinase (*ALK*) re-arrangement are eligible to receive therapy
  - *ALK*<sup>+</sup> NSCLC patients who were treated with XALKORI had significantly improved PFS compared to SOC therapy<sup>1</sup>
- **Efficient Healthcare Spending**
  - Unnecessary costs may be avoided as therapy is prescribed only to selected patients more likely to respond

<sup>1</sup> PROFILE 1007 Phase 3 study top line report , Pfizer Press Release, June 19, 2012



## Still Work to Do: An Evolving Process

- How best to gather a substantial body of evidence to satisfy regulators and payors?
- Incorporation of molecular profiling into “usual” care of cancer patients
- Synchronization of the timing between:
  - Drug development
  - Biomarker discovery
  - Development of companion diagnostic
- Future diagnostic practice: single tests vs. multiple marker tests for specific cancers



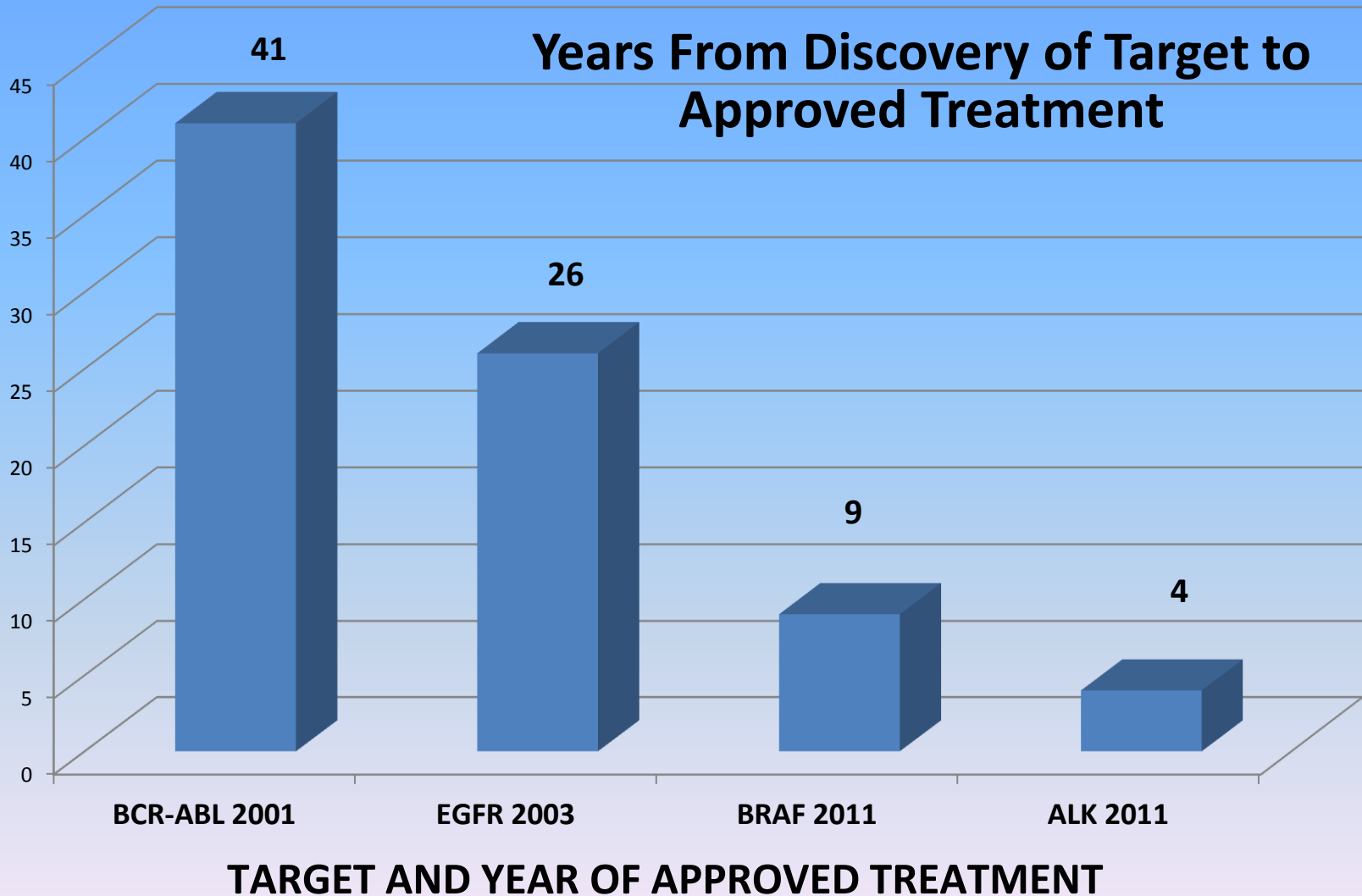
# How Far Have We Come in 40 Years Since the Declaration of the “*War on Cancer*”?

***Then***

***Now***

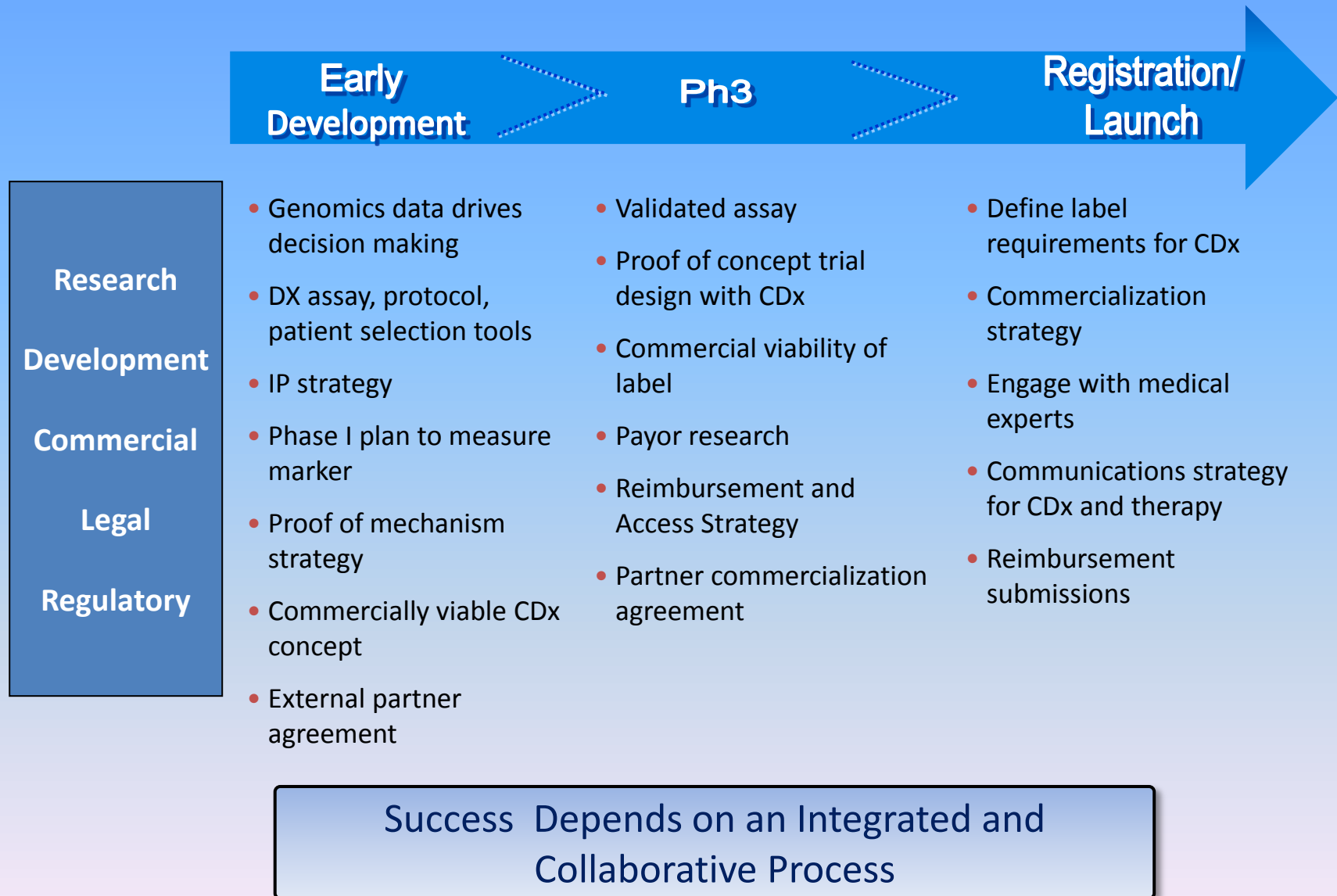
<b>Understanding of Cancer</b>	
Tumor-focused; uncontrolled cell proliferation	Genetic disease
<b>Detection &amp; Prevention</b>	
Physical examination; limited understanding of the importance of early detection	Sophisticated screening and greater understanding of the disease entity; Increasing use of molecular testing
<b>Treatment Options</b>	
Surgery, radiation, chemotherapy	Targeted therapies

# As Science Advances, Oncology Drug Development Accelerates



Adapted from Gerber and Minna Cancer Cell: 18: 548, 2010

# Pharma & Diagnostic Companies Harness the Potential of Personalized Medicine





# Summary

- Personalized medicine is about delivering **the right treatment, to the right patient, at the right time**
- **Innovation and incremental efficiencies** may be facilitated as additional biomarkers are identified, greater synergies and partnerships are established between pharmaceutical and diagnostic developers, and increased numbers of patients are swiftly diagnosed and treated
- There is a need for newer thinking and methodological approaches to the development of **supportive regulatory and reimbursement evidence** within this new paradigm of drug development
- Personalized Medicine benefits multiple stakeholders and supports the **efficient use of healthcare resources** by concentrating efforts on patients most likely to benefit and sparing expense and side effects for those who will not
- **Alignment of incentives** for personalized medicine **among all stakeholders** is critical to support the highest degree of clinical innovation that will improve health outcomes, health care delivery and ultimately, improve the quality of life of patients