

**PhARMA**  
RESEARCH • PROGRESS • HOPE

**CHART** PACK

# VALUE OF PERSONALIZED MEDICINE

SPRING 2015





# TABLE OF CONTENTS

Chapter 1	A New Treatment Paradigm.....	3
Chapter 2	Benefiting Patients and the Health Care System	14
Chapter 3	An Evolving Pipeline.....	23
Chapter 4	Advancing Policies to Foster Innovation.....	28
Glossary.....		34



# 1. A NEW TREATMENT PARADIGM

## A Targeted Approach to Patient Care

Advancements in science and technology are changing the way we define disease, develop drugs, and prescribe treatments. Armed with a greater understanding of disease biology, it has become evident that a patient's response to treatment—with respect to both safety and efficacy—is greatly dependent upon his or her molecular profile.

The promise of personalized medicine is to get the right treatment to the right patient at the right dose the first time through the use of molecular biomarker tests and targeted therapies.

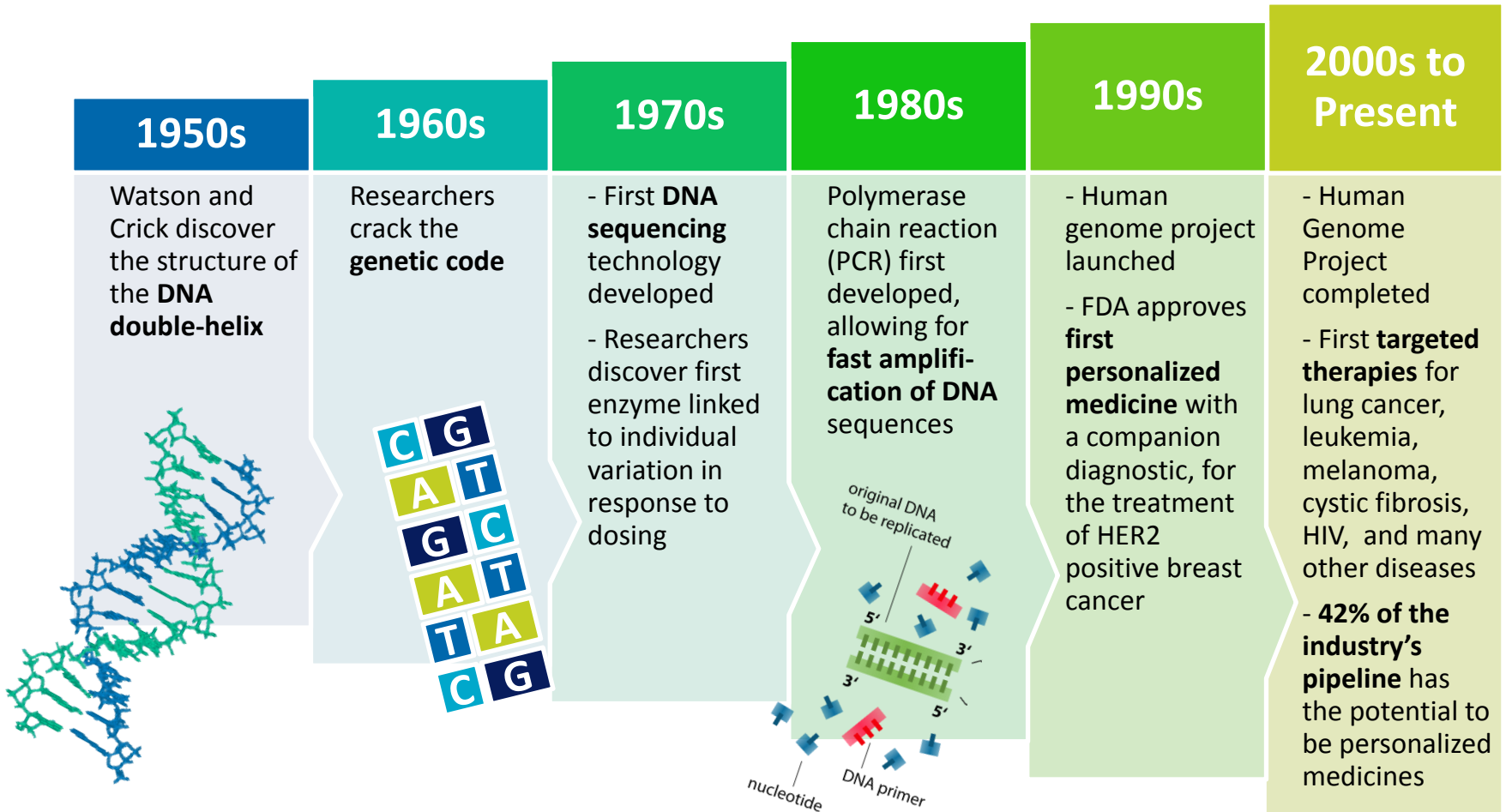
# What is Personalized Medicine?

**Personalized medicine**, sometimes referred to as *precision* or *individualized* medicine, is an emerging field of medicine that uses diagnostic tools to identify specific biological markers, often genetic, to help assess which medical treatments and procedures will be best for each patient.

Source: Personalized Medicine Coalition, "PMC Public Opinion Survey," [http://personalizedmedicinecoalition.org/Resources/PMC\\_Public\\_Opinion\\_Survey](http://personalizedmedicinecoalition.org/Resources/PMC_Public_Opinion_Survey), Jul 2014 (accessed May 2015).



# Scientific Advances Fuel Personalized Medicine



Sources: L Pray, "Discovery of DNA structure and function: Watson and Crick," *Nature Education*, 2008; 1(1):100; Office of National Institutes of Health History, "Exhibit: Deciphering the Genetic Code: Marshall Nirenburg," <http://history.nih.gov/exhibits/nirenberg/index.htm>; Scitable by Nature Education, "The Order of Nucleotides in a Gene Is Revealed by DNA Sequencing," <http://www.nature.com/scitable/topicpage/the-order-of-nucleotides-in-a-gene-6525806>; PJ Murphy, "The Development of Drug Metabolism Research as Expressed in the Publications of ASPET: Part 3, 1984–2008," *Drug Metab Dispos*, 2008 Oct;36(10):1977-82; National Center for Biotechnology Information, "Polymerase Chain Reaction," <http://www.ncbi.nlm.nih.gov/probe/docs/techpcr/>; NIH, "Fact Sheet: Human Genome Project," [http://report.nih.gov/NIHfactsheets/Pdfs/HumanGenomeProject\(NHGRI\).pdf](http://report.nih.gov/NIHfactsheets/Pdfs/HumanGenomeProject(NHGRI).pdf), Oct 2010; U.S. FDA, "Paving the Way for Personalized Medicine," Oct 2013; Tufts Center for the Study of Drug Development, "Impact Report," Volume 17, No.3, May/June 2015. (all sources listed accessed May 2015).

# Personalized Medicine is a Multi-Faceted Approach to Patient Care



**Risk Assessment:**  
Genetic testing to reveal  
predisposition to disease



**Diagnosis:**  
Accurate disease diagnosis  
enabling individualized  
treatment strategy



**Prevention:**  
Behavior/Lifestyle/  
Treatment intervention to  
prevent disease



**Treatment:**  
Improved outcomes through  
targeted treatments and  
reduced side effects



**Detection:**  
Early detection of disease  
at the molecular level



**Management:**  
Active monitoring of  
treatment response and  
disease progression

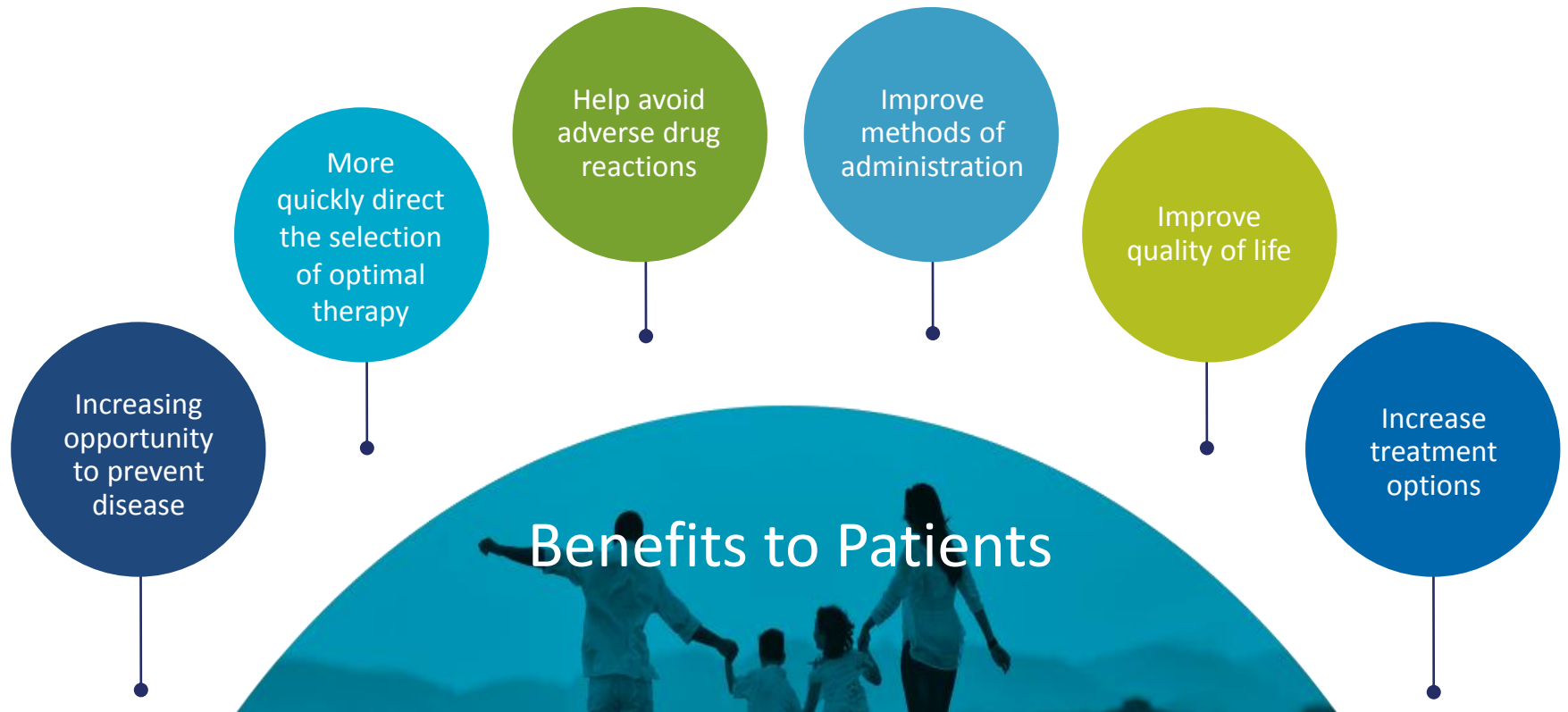
Source: The Age of Personalized Medicine, "What is Personalized Medicine?," [http://ageofpersonalizedmedicine.org/objects/pdfs/Age\\_PM\\_factsheet.pdf](http://ageofpersonalizedmedicine.org/objects/pdfs/Age_PM_factsheet.pdf) (accessed May 2015).

# Personalized Medicine Promises Improved Patient Outcomes



Personalized medicine is empowering because your personal genetic and other predictive information allows you to take action that is specific for you—rather than the ‘one size fits all’ approach.

—Francis Collins, MD, PhD, Director, National Institutes of Health



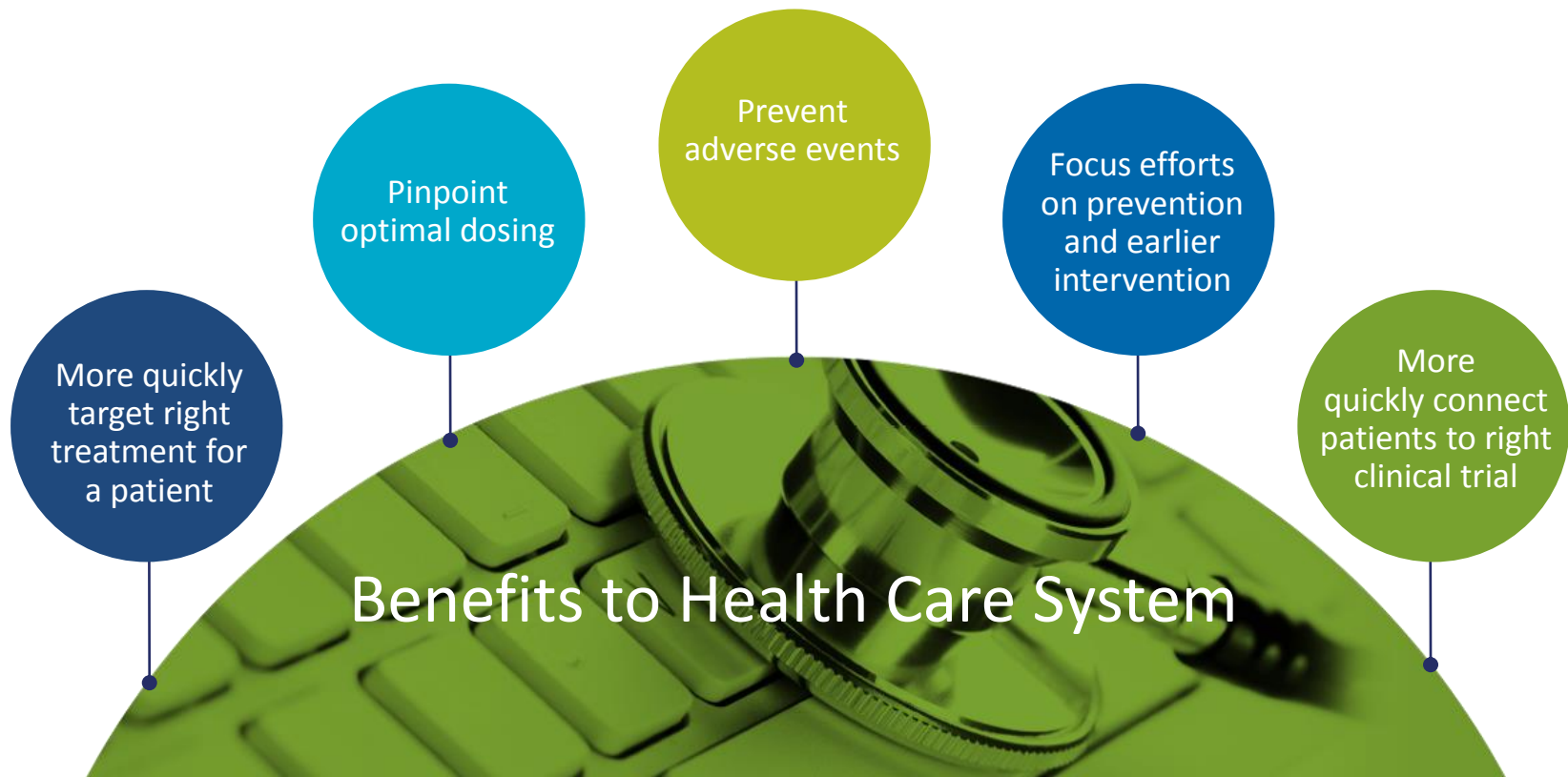
Source: National Institutes of Health, "The Future of Personalized Medicine," *NIH Medline Plus: The Magazine*, Winter 2010 Issue: Volume 5 Number 1 Pages 2 – 3.

# Personalized Medicine Can Improve Efficiencies within the Health Care System



Health care today is in crisis as it is expensive, reactive, inefficient, and focused largely on one-size-fits-all treatments for events of late stage disease. An answer is personalized, predictive, preventive, and participatory medicine.

—Ralph Snyderman, MD, Chancellor Emeritus, Duke University



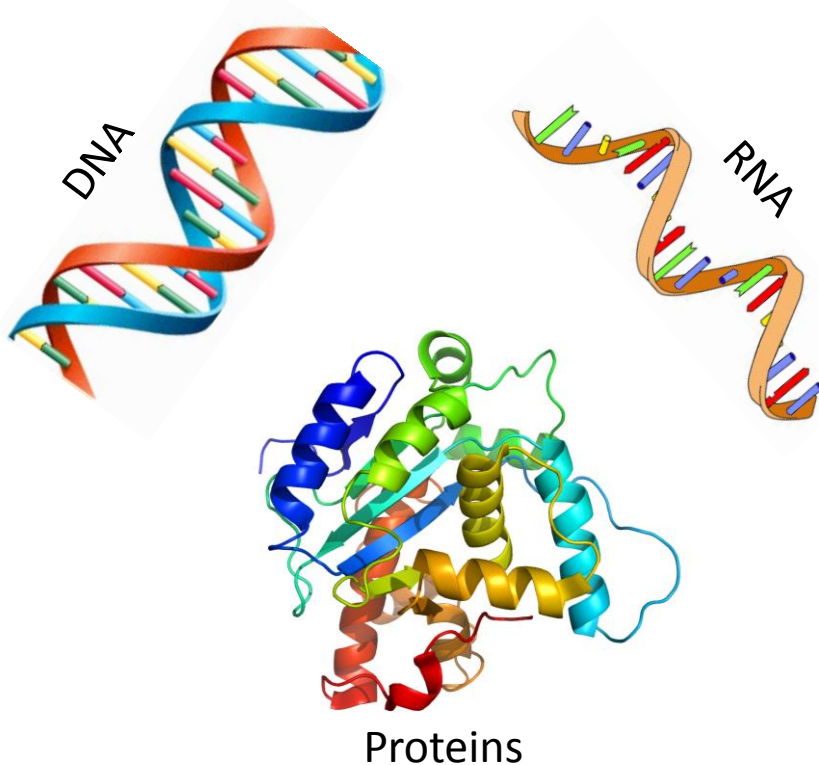
Source: Personalized Medicine Coalition, "The Case for Personalized Medicine, 4<sup>th</sup> Edition," 2014.



# What is a Biomarker?

A biomarker is a characteristic that is objectively measured and evaluated as an indicator of normal biologic processes, disease processes, or biological responses to a therapeutic intervention. Biomarkers can be used to reduce uncertainty and guide clinical care.

Molecular Biomarkers Can Include:



Biomarkers Help Inform Medical Decisions:

- Prevention measures?
- Which diagnosis?
- Treat or don't treat?
- What dose?

How Do You Detect a Biomarker?

- Diagnostics
  - Blood draw
  - Microscopic analysis
  - Gene sequencing
  - Biopsy
  - Protein analysis

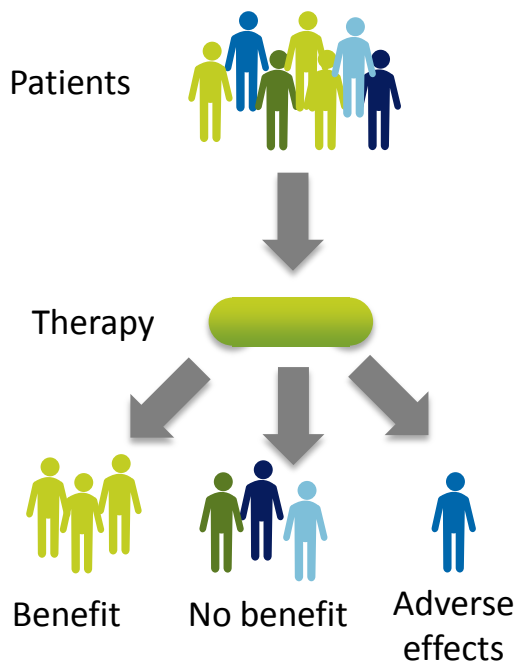
Source: National Cancer Institute, "NCI Dictionary of Cancer Terms" (accessed May 2015).

# A New Treatment Paradigm

The molecular profile of an individual patient and their disease influences the effect of a medicine; biomarker diagnostics help to target the right medicine to the right patient.

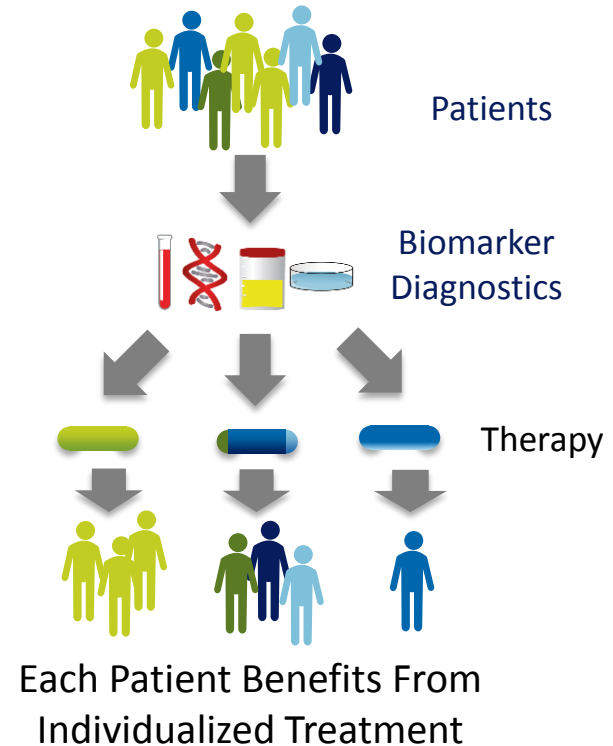
## Without Personalized Medicine:

Some Benefit, Some Do Not



## With Personalized Medicine:

Each Patient Receives the Right Medicine For Them



Source: Adapted from Bayer Healthcare, "Personalized Medicine." <https://pharma.bayer.com/en/research-and-development/research-focus/oncology/personalized-medicine/index.php>, (accessed May 2015).

# More Personalized Medicine Products are Available for Patients Than Ever Before



Total number of FDA-approved drugs with biomarker information provided on their drug label\*



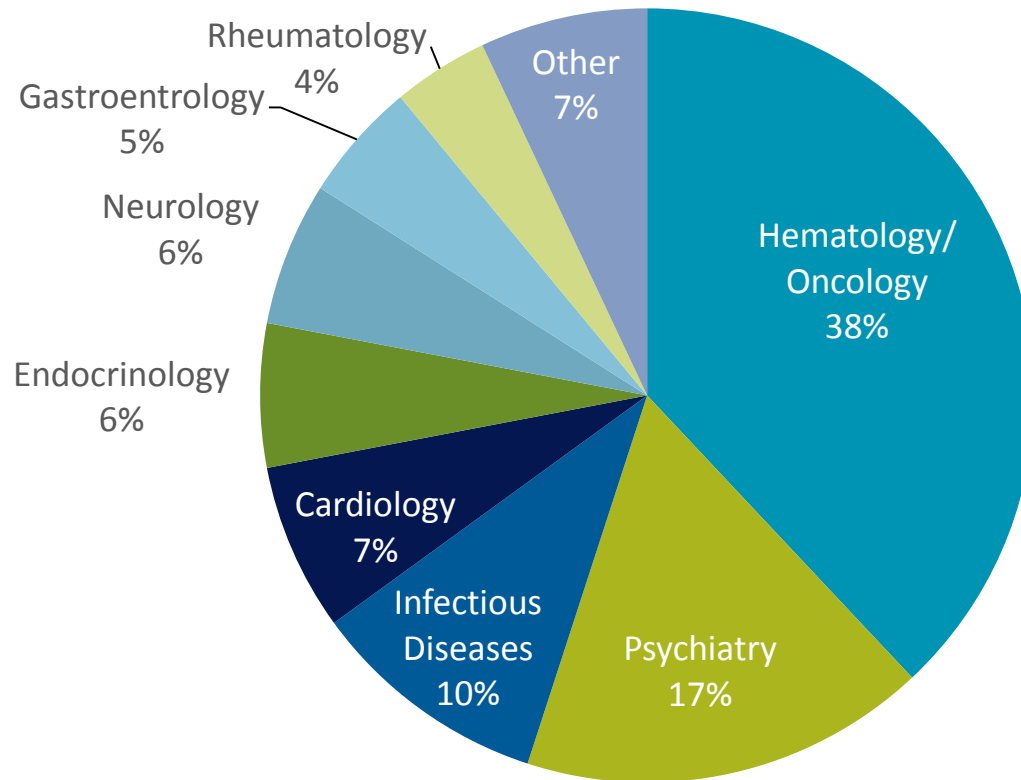
1 in 5 FDA approvals in 2014 were for targeted therapies\*

*\*As of March 27, 2015*

Source: U.S. FDA, "Table of Pharmacogenomic Biomarkers in Drug Labeling," <http://www.fda.gov/drugs/scienceresearch/researchareas/pharmacogenetics/ucm083378.htm> (accessed Apr 2015), PMC, "More than 20 percent of the Novel New Drugs Approved by FDA's Center for Drug Evaluation and Research in 2014 are Personalized Medicines," <http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/2014-fda-approvals-personalized-medicine2.pdf> (accessed May 2015).

# Personalized Medicines Are Benefitting Patients Across Many Different Diseases

Across a variety of therapeutic areas, an increasing number of treatments are personalized.\*



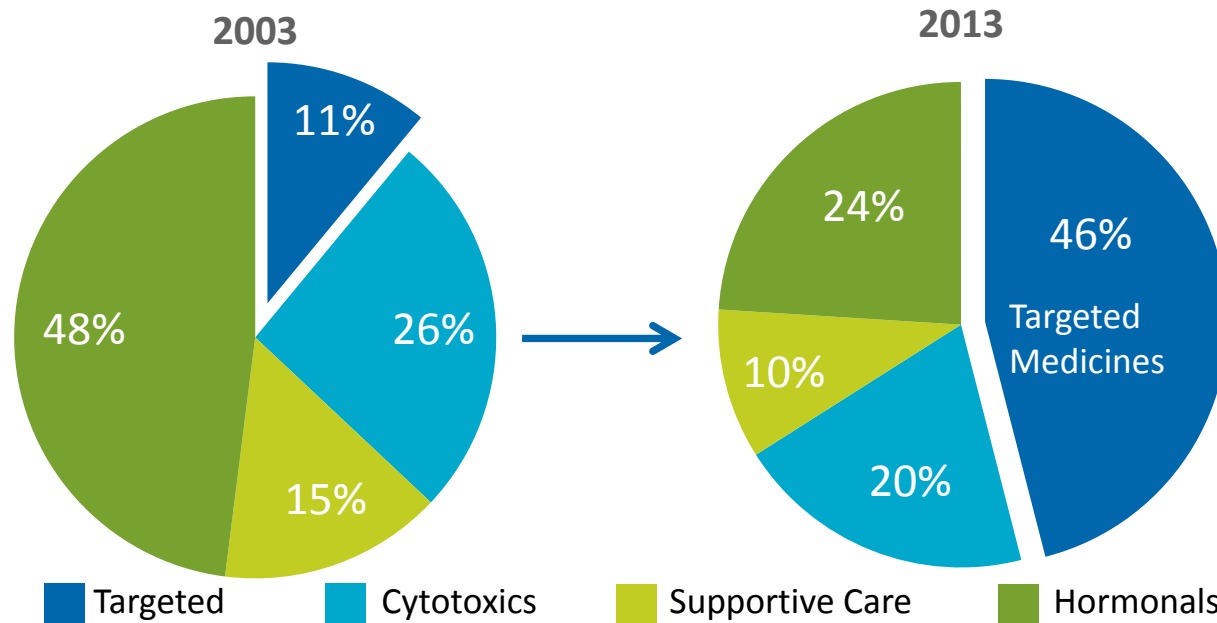
\*FDA approvals with biomarker information in the approved labeling

Source: U.S. FDA, "Paving the Way for Personalized Medicine," Oct 2013.

# Oncology is on the Leading Edge of Personalized Medicine

In ten years, cancer patients have seen a four-fold increase in their personalized medicine treatment options.

*Breakdown of Oncology Treatment Modalities, Global Market share 2003-2013\**



\*Definitions: Targeted therapies - identify and attack specific types of cancer cells with less harm to normal cells; Cytotoxics – agents that kill rapidly developing cells (as in chemotherapy); Supportive care - care given to improve quality of life by preventing or treating the symptoms of a disease or the effects of its treatment; Hormonals - treatments that add, block, or remove hormones to slow or stop the growth of certain cancers.

Sources: IMS Institute for Healthcare Informatics, "Innovation in Cancer Care and Implications for Health Systems: Global Oncology Trend Report," May 2014 (accessed May 2015); National Cancer Institute, "NCI Dictionary of Cancer Terms" (accessed May 2015).





## 2. BENEFITING PATIENTS AND THE HEALTH CARE SYSTEM

### Growing Impact of Personalized Medicine

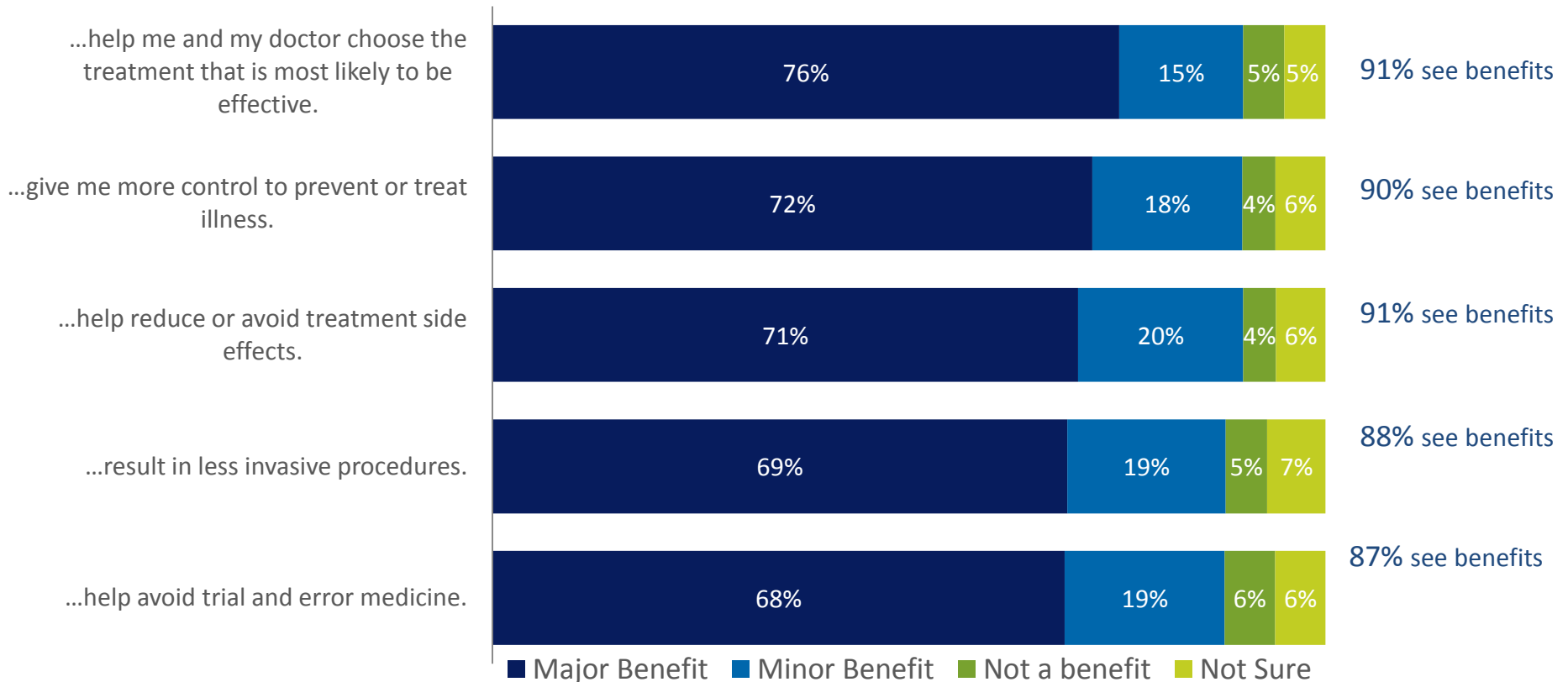
The first wave of personalized medicines have entered mainstream clinical practice and are changing the way many diseases are identified, classified, and treated. These advancements are particularly evident in the area of oncology.

From helping patients live longer and healthier lives to creating efficiencies in health care, these new personalized medicines are bringing great value to patients and the health care system.

# The Benefits of Personalized Medicine Are Well Recognized

Respondents in a national survey viewed personalized medicines as offering clear clinical benefits.

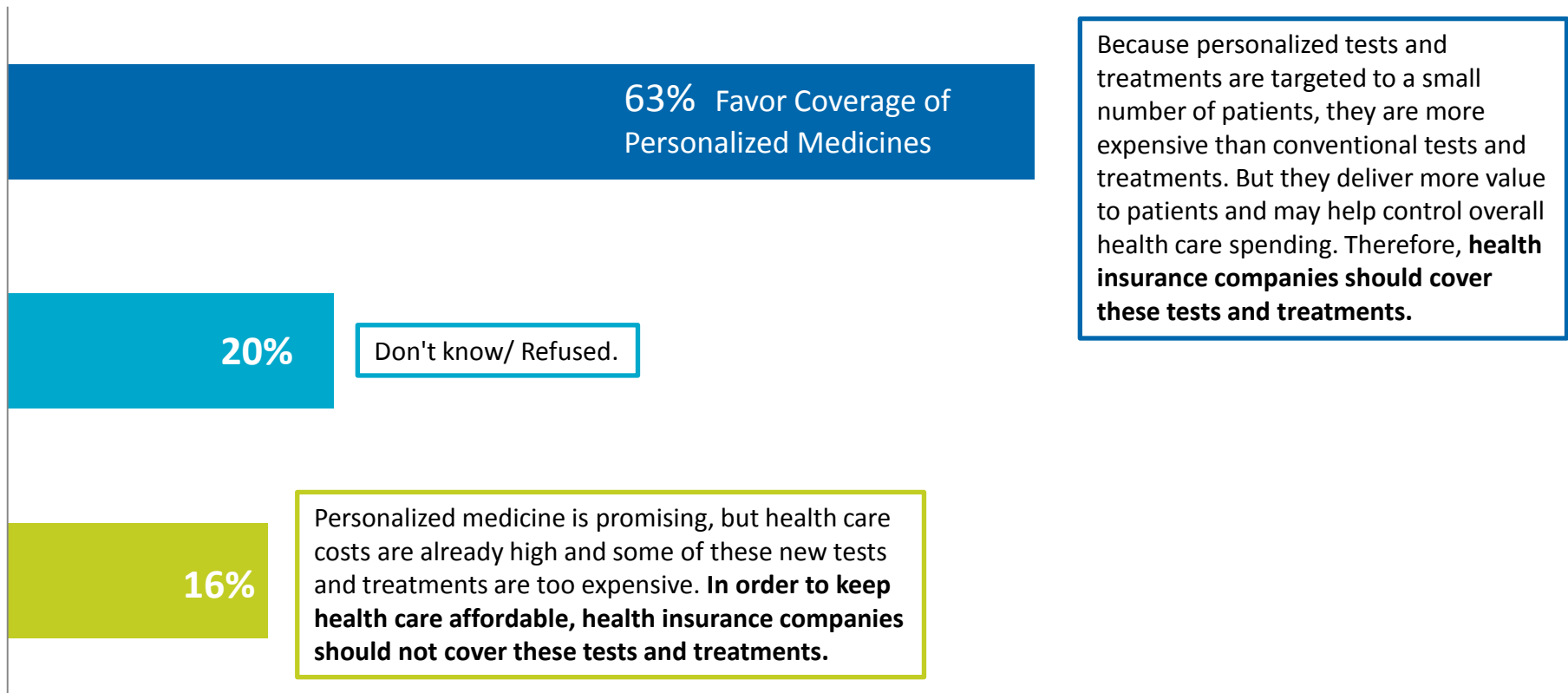
## Personalized medicine could...



Source: Personalized Medicine Coalition, "PMC Public Opinion Survey," [http://personalizedmedicinecoalition.org/Resources/PMC\\_Public\\_Opinion\\_Survey](http://personalizedmedicinecoalition.org/Resources/PMC_Public_Opinion_Survey), Jul 2014 (accessed May 2015).

# Public Supports Coverage of Personalized Medicine

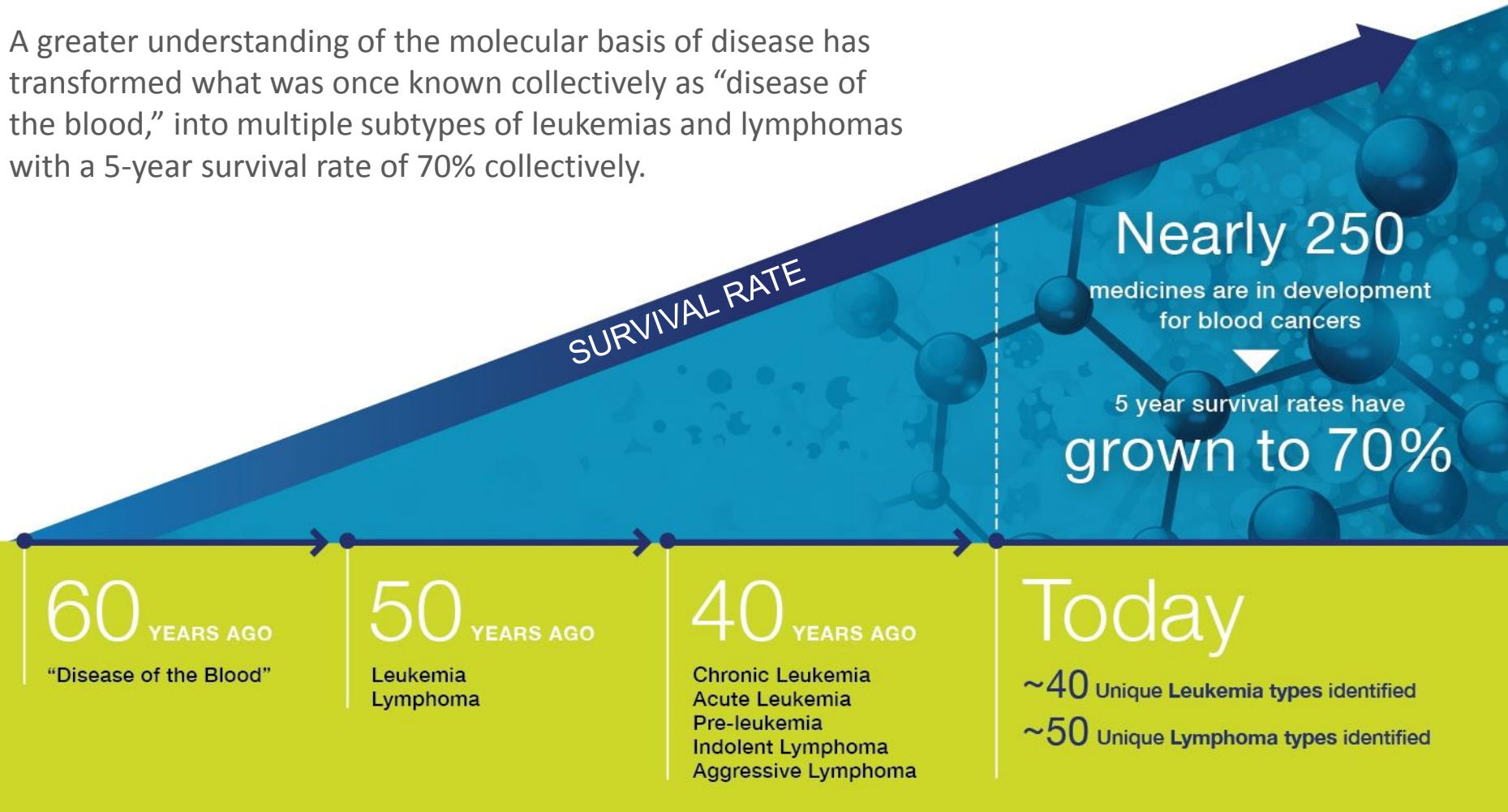
In a recent survey, nearly two-thirds of individuals surveyed believe payers should cover personalized medicine tests and treatments because they deliver more value to patients than conventional treatments and may help control overall health care spending.



Source: Personalized Medicine Coalition, "PMC Public Opinion Survey," [http://personalizedmedicinecoalition.org/Resources/PMC\\_Public\\_Opinion\\_Survey](http://personalizedmedicinecoalition.org/Resources/PMC_Public_Opinion_Survey), Jul 2014 (accessed May 2015).

# Advances in Personalized Medicine Improve Outlook for Patients with Blood Cancers

A greater understanding of the molecular basis of disease has transformed what was once known collectively as “disease of the blood,” into multiple subtypes of leukemias and lymphomas with a 5-year survival rate of 70% collectively.



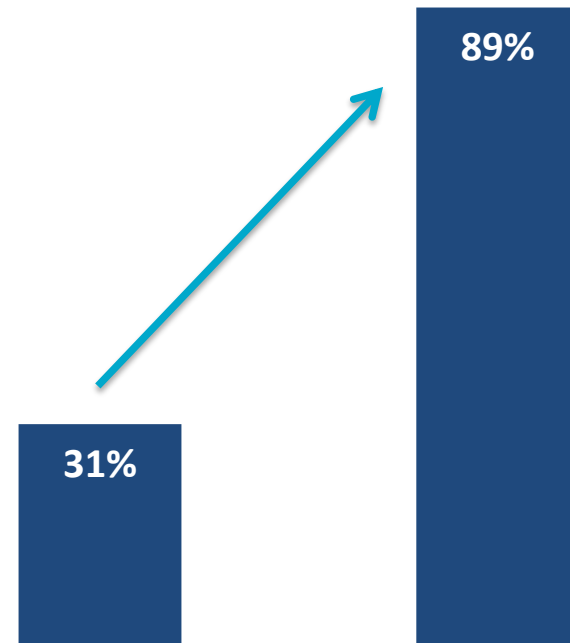
Source: M Aspinal, former President Genzyme Genetics (cited at [http://www.comtecmed.com/biomarker/2014/Uploads/Editor/PDF/ppt/Edward%20Abrahams\\_Key%20Note%20Lecture.pdf](http://www.comtecmed.com/biomarker/2014/Uploads/Editor/PDF/ppt/Edward%20Abrahams_Key%20Note%20Lecture.pdf)); National Cancer Institute,; SEER Cancer Statistics Review, 1975-2011, [http://seer.cancer.gov/csr/1975\\_2011/](http://seer.cancer.gov/csr/1975_2011/), based on November 2013 SEER data submission, posted to the SEER web site, April 2014; PhRMA, "Medicines in Development for Leukemia & Lymphoma," April 2015 (all cites accessed May 2015).

# Personalized Medicine Leads to Increased Survival Rates: Chronic Leukemias

A new wave of molecularly targeted therapies have turned chronic myelogenous leukemia (CML) from a deadly disease into one in which patients live close to normal life spans.

- Imatinib was the first in this new class of targeted therapies.
- Imatinib targets abnormal proteins found at the surface of certain cancer cells that signal for the cells to divide. Imatinib blocks these signals.
- Today, survival rates have improved dramatically as more drugs in this class have been approved and **CML patients are living close to normal life spans.**

*5-Year Survival Rates for CML Patients Nearly Triple After Introduction of Imatinib*



This drug [imatinib] really extends life for people who were facing almost certain death. That's the poster child for personalized medicine.

-Michael Snyder, PhD, Stanford University

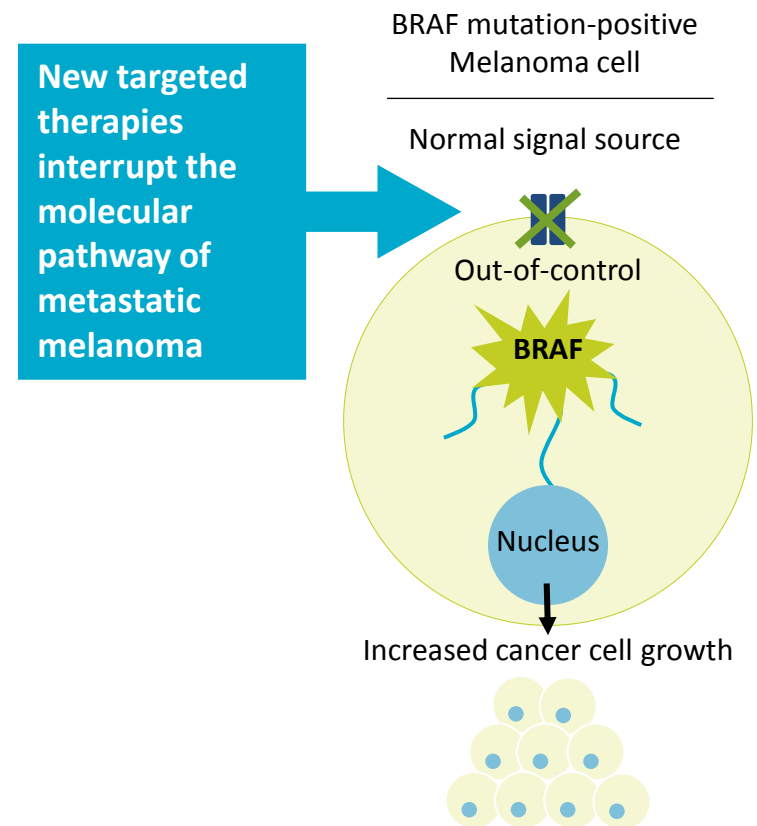
Sources: TF Goss, et al., "Recognizing Value in Oncology Innovation," Boston Healthcare Associates, Jun 2012; C Gambacorti-Passerini, et al., "Multicenter independent assessment of outcomes in chronic myeloid leukemia patients treated with imatinib," *J Natl Canc Inst* 2011; 103(7): 553-61; American Cancer Society, "Cancer Facts and Figures 2012," 2012; BJ Druker, et al., "Five-year follow-up of patients receiving imatinib for chronic myeloid leukemia," *NEJM* 2006; 355(23):2408-17.; S Mukherjee, "The revolution will be personalized: An interview with two Stanford geneticists," *BioPharmaDIVE*, Oct 2014.



# Personalized Medicine Yields Treatment Breakthroughs: Metastatic Melanoma

New targeted therapies and immunotherapies – medicines that harness the patient’s immune system to attack cancer cells – are improving survival rates.

- Metastatic melanoma is an aggressive cancer with a 5-year survival rate of 15-20%.
- In 2002, a mutation in the BRAF gene was identified and found to be present in ~ 50% of all melanomas. This mutation leads to the overproduction and spread of cancer cells.
- This discovery led to the development and FDA approval of 3 new targeted drugs that ***are improving the overall survival rate compared to chemotherapy.***
- Three new immunotherapies are also changing the treatment landscape. These medicines target proteins that prevent the immune system from attacking cancer cells.

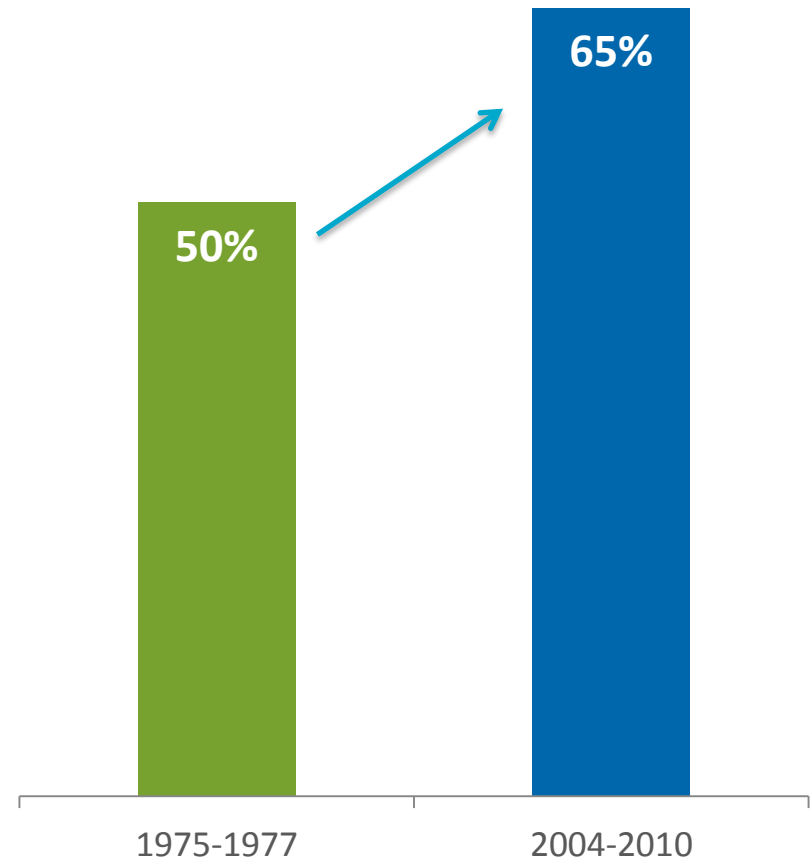


Sources: American Cancer Society, “Melanoma Skin Cancer,” <http://www.cancer.org/cancer/skincancer-melanoma/detailedguide/melanoma-skin-cancer-treating-targeted-therapy>, Mar 2015; Moffitt Cancer Center, “One of the Biggest Breakthroughs in 30 Years for Treating Advanced Melanoma,” <http://moffitt.org/the-moffitt-experience/what-differentiates-moffitt/one-of-the-biggest-breakthroughs-in-30-years-for-treating-advanced-melanoma> (both cites accessed May 2015).

# Personalized Medicine Helps Predict Treatment Response: Metastatic Colorectal Cancer

*5-Year Survival Rates for Colorectal Cancer Patients*

- In recent years, scientists have identified the molecular receptor on colorectal cancer cells that causes them to multiply (epidermal growth factor receptor, or EGFR)
- New medicines that target these receptors are improving survival outcomes.
- Cetuximab, a type of targeted monoclonal antibody therapy, blocks the signals from EGFR.
- Continuing research revealed that the presence of a specific mutation in a particular gene (KRAS) is associated with resistance to cetuximab.
- Testing for the KRAS gene allows for better targeting of therapy and improved patient survival.



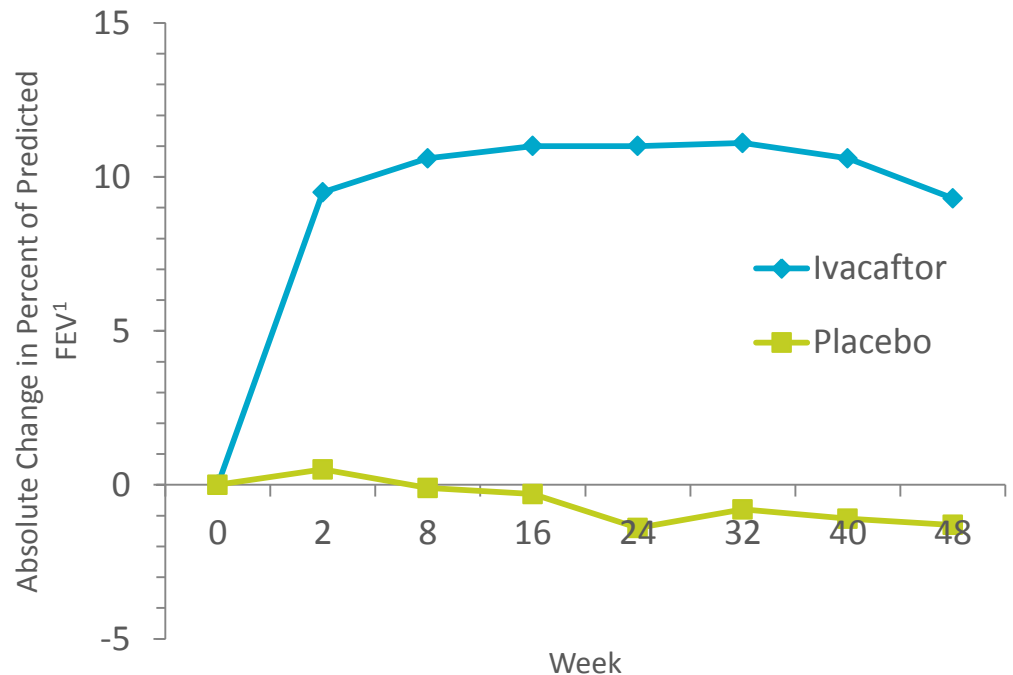
Sources: National Cancer Institute Surveillance, Epidemiology, and End Results (SEER) Program, "SEER Cancer Statistics Review 1975-2011," <http://seer.cancer.gov/>; National Cancer Institute SEER Program, "SEER Stat Fact Sheets: Colon and Rectum Cancer," <http://seer.cancer.gov/statfacts/html/colorect.html> (both cites accessed May 2015).

# Personalized Medicine Enables Targeting of the Underlying Cause of Disease: Cystic Fibrosis

Cystic fibrosis is a serious genetic disorder caused by mutations in a gene that encodes for a protein called CFTR, which regulates the absorption and secretion of salt and water in the body.

Ivacaftor targets the defective CFTR protein in patients that have one of several, resulting in significant and sustained improvement in lung function.

*Ivacaftor Improves Lung Function in Patients with CF*



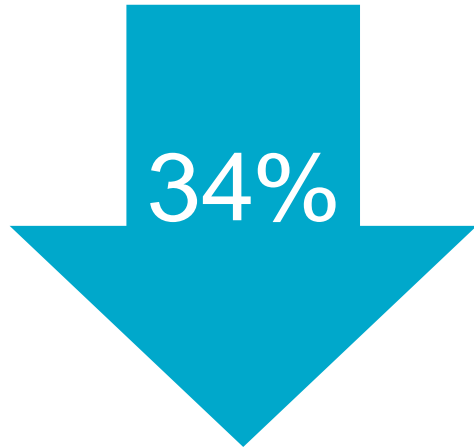
Kalydeco is the first available treatment that targets the defective CFTR protein, which is the underlying cause of cystic fibrosis. This is a breakthrough therapy for the cystic fibrosis community because current therapies only treat the symptoms of this genetic disease.

-Janet Woodcock, MD, Director, FDA's Center for Drug Evaluation and Research

Sources: BW Ramsey, et al., "A CFTR Potentiator in Patients with Cystic Fibrosis and the G551D Mutation," *N Engl J Med* 2011;365:1663-1672; U.S. FDA, "FDA approves Kalydeco to treat rare form of cystic fibrosis," <http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm289633.htm>, Jan 2012 (accessed May 2015).

# Personalized Medicine Can Create Efficiencies in the Health Care System

## Breast Cancer



Reduction in chemotherapy use would occur

If women with breast cancer receive a genetic test of their tumor prior to treatment

## Metastatic Colorectal Cancer



In annual health care cost savings would be realized

If patients with metastatic colorectal cancer receive a genetic test for the KRAS gene prior to treatment

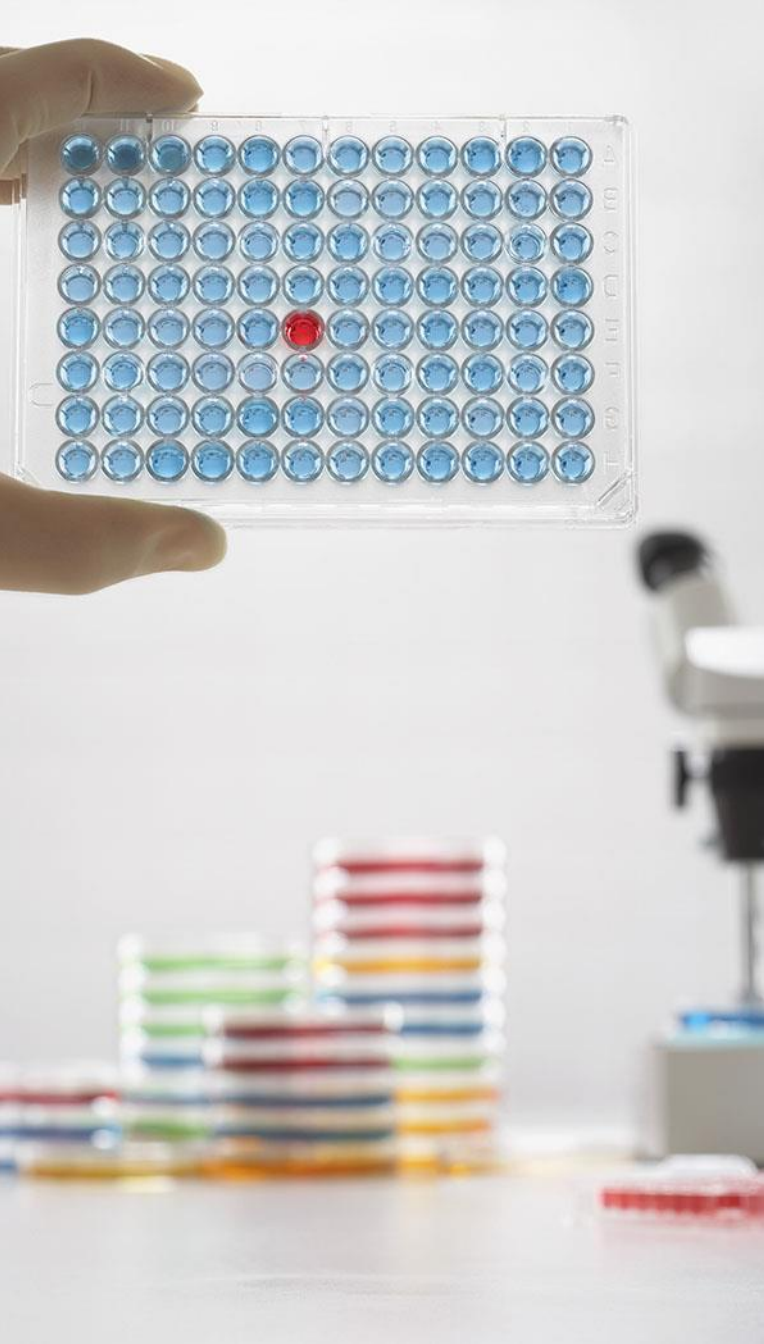
## Stroke



Strokes could be prevented each year

If a genetic test is used to properly dose blood thinners

Sources: Personalized Medicine Coalition, "Personalized Medicine by the Numbers," 2014 (accessed May 2015).



## 3. AN EVOLVING PIPELINE

### Innovative Therapeutic Advances on the Horizon

A greater understanding of the underlying biological mechanisms of many diseases is enabling researchers to pursue new targeted approaches to fighting them.

Across many disease areas, we are already seeing the benefits of personalized medicine. Looking at the pipeline, we see even more potential.

Today, there are more personalized medicines in the clinical pipeline across many disease categories than ever before.



# Partnerships and Collaborations are Transforming the Research and Development of Personalized Medicines

Partnerships are crucial to maintaining robust biopharmaceutical innovation in the United States. Collaborations come in many different shapes and sizes. Here are some selected examples of key collaborative efforts across the research and development spectrum.

## AMP (Accelerating Medicines Partnership)

Developing new diagnostics and biological targets for treatments in Alzheimer's disease, type 2 diabetes, rheumatoid arthritis, and lupus.

*The Partners: biopharmaceutical companies, NIH, patient and disease organizations*



## Biomarkers Consortium

Combining expertise and resources to rapidly identify, develop, and qualify biomarkers, which will then advance new therapies and guide improvements in regulatory and clinical decision-making.

*The Partners: biopharmaceutical companies, NIH, CMS, FDA, patient and disease organizations*



## Lung-MAP (Lung Cancer Master Protocol)

Using comprehensive genetic screening to identify mutations in lung cancer patients in order to direct them to a specific investigational treatment, all operating under a single clinical trial protocol.

*The Partners: biopharmaceutical companies, NIH, FDA, patient and disease organizations*



Sources: National Institutes of Health, "Accelerating Medicines Partnership," <http://nih.gov/science/amp/index.htm>;

The Biomarkers Consortium, <http://www.biomarkersconsortium.org/index.php>; Lung Cancer Master Protocol, <http://www.lung-map.org/> (all cites accessed May 2015).

# Lung-MAP: Personalized Medicine is Driving a New Clinical Research Paradigm

Lung-MAP (Lung Cancer Master Protocol) uses genomic profiling to match patients to specific investigational personalized medicines, all under one umbrella study. Shared information and resources accelerates drug development and increases trial efficiency, delivering new medicines to patients faster.

## THE PARTNERS:

Lung-MAP is a unique public-private partnership between:

- Patient and disease advocacy groups
- Biopharmaceutical companies
- National Cancer Institute
- Foundation for the National Institutes of Health
- SWOG Cancer Research Consortium



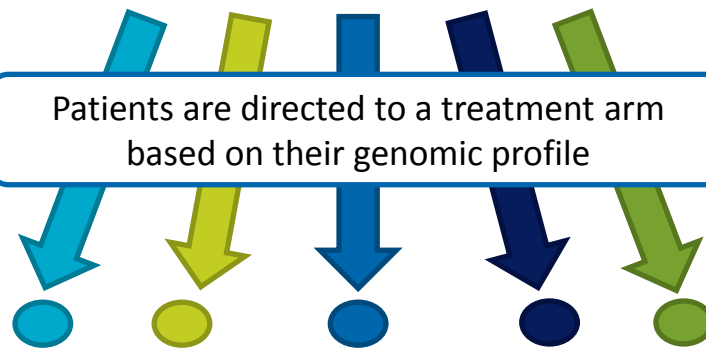
This is an entirely new way of looking at the development of cancer drugs. This is no longer business as usual. This approach changes the paradigm.

-David Gandara, MD, UC Davis

## HOW IT WORKS:

Patients undergo genomic profiling to identify mutations that may cause squamous cell lung cancer

Patients are directed to a treatment arm based on their genomic profile



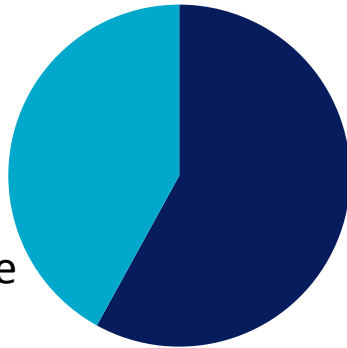
Patients receive highly targeted therapies and researchers collect data to advance the study of new medicines

Source: Lung Cancer Master Protocol, <http://www.lung-map.org/> (accessed May 2015).

# Biopharmaceutical Companies Are Committed to Advancing Personalized Medicines

The research and development pipeline holds great promise for targeted therapies.

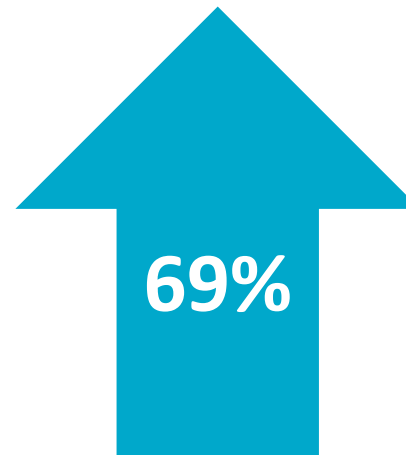
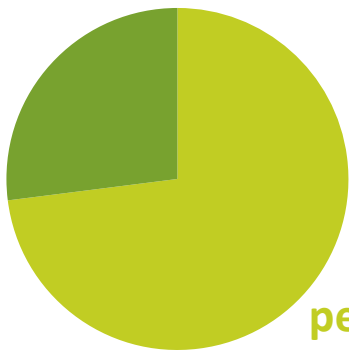
**42%** of **new medicines** in the pipeline have the potential to be **personalized medicines**



**33%** expected increase investment in **personalized medicines** over the next five years



**73%** of **cancer medicines** in the pipeline have the potential to be **personalized medicines**



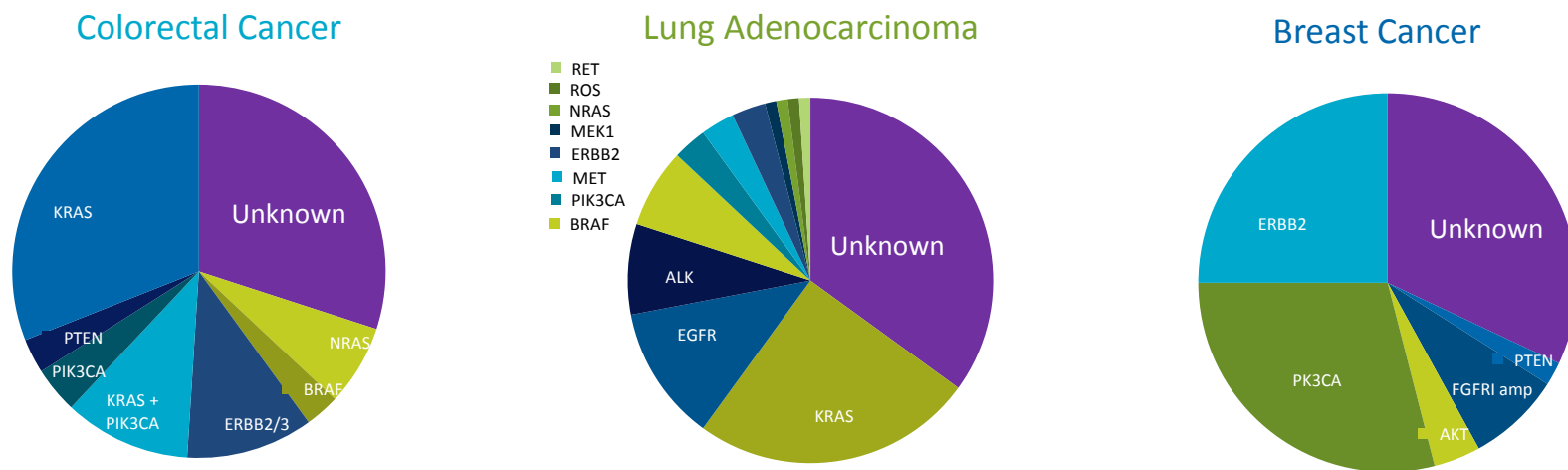
**Expected increase** in the number of **personalized medicines** in development over the next 5 years

Sources: Tufts Center for the Study of Drug Development, "Impact Report," Volume 17, No.3, May/June 2015.

# Researchers Have Made Great Progress Identifying Genes that—when Mutated—Drive Many Cancers, but Challenges Remain

We now know that cancer is not a single disease, but rather more than 200 unique diseases, many of which are caused by genetic mutations. Identifying these mutations has led to tremendous advances against many cancers, but the complexity of each disease presents great challenges for researchers, as they explore still yet unknown alterations.

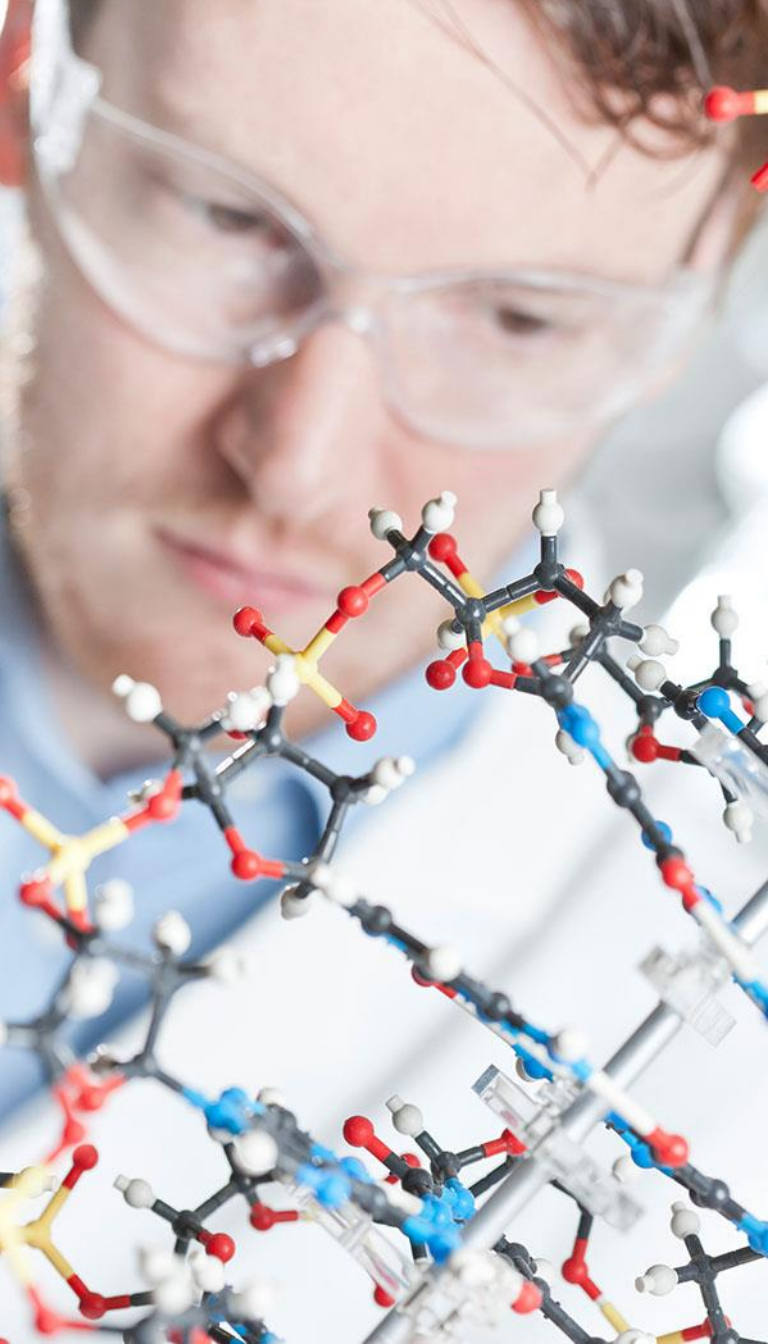
Selected Genomic Alterations Known to Drive Disease Progression in Common Cancers



Cancer relapses and treatment resistance have always been among the most daunting challenges in cancer care...The good news is that genomic medicine is helping to overcome these challenges by revealing new ways to target a cancer cell’s inner workings.

-Gregory A. Masters, MD, Helen F. Graham Cancer Center

Sources: LA Garraway, “Genomics-Driven Oncology: Framework for an Emerging Paradigm,” *J Clin Onc.* 2013; 31(15):1806-1814; American Society of Clinical Oncology, “Studies Reveal Potential New Targeted Therapies for Common, Hard-to-Treat Cancers,” <http://www.asco.org/press-center/studies-reveal-potential-new-targeted-therapies-common-hard-treat-cancers>, May 2014 (accessed May 2015).



## 4. ADVANCING POLICIES TO FOSTER INNOVATION

### Sustaining Advances in Personalized Medicine

Despite the tremendous advances that have been made to date, much work is needed to further stimulate innovation in personalized medicines.

From the early stages of discovery through drug development and the delivery of targeted therapies to patients, it is important that public policies create an environment that provides incentives for accelerated innovation and ensures patient access to these important treatments.

# Policy Interest in Personalized Medicine is Growing

There is increasing recognition from both government and private stakeholders of the value and promise of personalized medicine.



*“To harness the benefits of personalized medicine in patient-centered care delivery, policymakers and regulators must create an environment that encourages increased investment in diagnostics; enables new advances in patient care that are safe, accurate and reliable; and establishes a viable pathway toward patient access.”*

- Turning the Tide Against Cancer initiative

*“Precision medicine in the future will be even more precise, not just regarding therapy selection but also with respect to risk assessment, establishing prognosis, monitoring treatment effectiveness, and predicting its tolerability for each individual. Health care delivery will also change dramatically as point-of-care molecular diagnostics become commonplace and crowdsourcing of information affects clinical decision-making and patient engagement.”*



- American Society of Clinical Oncology (ASCO)



*“Precision medicine gives us one of the greatest opportunities for new medical breakthroughs that we have ever seen.”*

- President Barack Obama, Launching new Precision Medicine Initiative

*“If we want to save more lives and keep this country the leader in medical innovation, we have to make sure there’s not a major gap between the science of cures and the way we regulate these therapies”*



- Fred Upton, Chairman of the Energy and Commerce Committee, U.S. House of Representatives, 21<sup>st</sup> Century Cures Initiative

*“It is clear that personalized medicine is increasingly becoming an integral part of clinical care and we expect this trend to continue along with greater recognition of the value of personalized medicine by payers and providers.”*

- Daryl Pritchard, PhD, vice president, science policy, Personalized Medicine Coalition



Sources: A Abernethy, et al., “Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress,” *Clin Cancer Res* 2014, 20(5), 1-6; Congressman Fred Upton, “A Path to 21<sup>st</sup> Century Cures,” <http://upton.house.gov/news/documentsingle.aspx?DocumentID=378238>, Apr 2014; AM Tsimberidou, et al., “Precision Cancer Medicine: The Future Is Now, Only Better,” American Society of Clinical Oncology 2014 Educational Book, <http://meetinglibrary.asco.org/content/114000061-144>; The White House, “Remarks by the President on Precision Medicine,” <https://www.whitehouse.gov/the-press-office/2015/01/30/remarks-president-precision-medicine>, Jan 2015; Personalized Medicine Coalition, “PMC Analysis: More Than 20 Percent of FDA’s 2014 Novel New Drug Approvals are Personalized Medicines,” [http://www.personalizedmedicinecoalition.org/News/Press\\_Releases/PMC\\_Analysis\\_More\\_Than\\_20\\_Percent\\_of\\_FDA’s\\_2014\\_Novel\\_New\\_Drug\\_Approvals\\_are\\_Personalized\\_Medicines](http://www.personalizedmedicinecoalition.org/News/Press_Releases/PMC_Analysis_More_Than_20_Percent_of_FDA’s_2014_Novel_New_Drug_Approvals_are_Personalized_Medicines), Jan 2015 (all cites accessed May 2015).



# From Discovery Through Delivery, Policies Must Foster Continued Innovation and Patient Access to Personalized Medicines

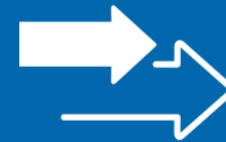
At a time when the scientific promise is greater than ever, thoughtful policies are necessary to accelerate advances in targeted therapy for patients.



Discovery



Development



Delivery



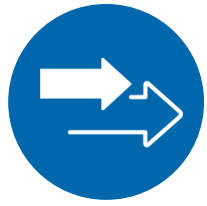
# Policy Solutions to Foster Innovation and Access to Personalized Medicines: **Discovery**

- Ensure an environment that provides strong intellectual property protections for both patents and data generated during the R&D process
- Advance research on molecular-based biomarkers to support the development of targeted therapeutics and molecular diagnostics
- Maintain robust funding for each element of the scientific ecosystem, e.g., strengthen and make permanent the R&D tax credit
- Expand the pool of qualified STEM workers to continue to drive scientific and technological innovation



# Policy Solutions to Foster Innovation and Access to Personalized Medicines: **Development**

- Ensure clear and transparent regulatory framework for the development of targeted therapies and co-developed diagnostics
- Develop framework for incorporation of larger and more diverse data sets (real world data, patient-centered outcomes, biomarkers, etc.)



# Policy Solutions to Foster Innovation and Access to Personalized Medicines: **Delivery**

- Ensure that value assessments are patient-centered and align with the way value emerges/ changes over time
- Ensure that emerging payment models are aligned with personalized medicine
- Recognize improved health outcomes from personalized medicines

# Glossary

**Biomarker:** A biological molecule found in blood, other body fluids, or tissues that is a sign of a normal or abnormal process, or of a condition or disease. A biomarker may be used to see how well the body responds to a treatment for a disease or condition. Also called molecular marker and signature molecule.

**Companion Diagnostic:** Companion diagnostics are a test or measurement intended to assist physicians in making treatment decisions for their patients. They do so by revealing the efficacy and/or safety of a specific drug or class of drugs for a targeted patient group or sub-groups.

**Diagnostics:** A program or routine that uses symptoms or characteristics to identify the nature of a medical condition.

**Gene Mutation:** A permanent change in the DNA sequence that makes up a gene. Mutations range in size from a single DNA building block (DNA base) to a large segment of a chromosome. Gene mutations occur in two ways: they can be inherited from a parent or acquired during a person's lifetime.

**Genetic Marker:** An alteration in DNA that may indicate an increased risk of developing a specific disease or disorder.

**Genome:** The complete genetic material of an organism.

**Genomic Sequencing:** A laboratory method that is used to determine the entire genetic makeup of a specific organism or cell type. This method can be used to find changes in areas of the genome that may be important in the development of specific diseases, such as cancer.

**Personalized Medicine:** Sometimes referred to as precision or individualized medicine, personalized medicine, is an emerging field of medicine that uses diagnostic tools to identify specific biological markers, often genetic, to help assess which medical treatments and procedures will be best for each patient.

**Targeted Therapies:** A type of treatment that uses drugs or other substances to identify and attack specific types of cancer cells with less harm to normal cells.