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Overview

• We have made remarkable progress in the fight against cancer and current research holds enormous promise to address the great unmet need.

• Too many cancer patients face financial burdens, and these come from a variety of sources including treatment costs, non-medical costs, and insurance benefit design.

• The cost of cancer treatment come from a range of sources including medicines, hospital and ED visits, diagnostics, and physician services.

• The oncology market is working to control spending on cancer medicines and overall treatment costs.

• Reforms are needed to support value-based contracting, informed decision-making, and better quality measurement in cancer.
1. Advances in Cancer Treatment
Since 1975, the chances that a cancer patient will live 5 years or more have increased by 41% across cancers.¹

5-Year Survival Rates Among the Most Common Cancers, 1975-2012²

73% of recent survival gains in cancer are attributable to treatment advances including new medicines.³

Since Peaking in the Early 1990s, Cancer Death Rates Have Declined 25%

Increases in cancer survival are estimated to translate to the avoidance of nearly 2.1 million cancer deaths.

U.S. Death Rates from Cancer Decline Over Time

Introduction of Novel Cancer Medicines Associated with Survival Increases

Medicines are one factor driving increased survival, along with screening, earlier diagnosis, and lifestyle changes.

Change in Incidence and Survival (2004-2013)

Source: QuintilesIMS Institute, “Global Oncology Trends 2017,” June 2017.
The continued increase in survival rates is in large part attributable to earlier detection and better treatments.¹

U.S Cancer Survivors Over Time (millions)¹,²,³

<table>
<thead>
<tr>
<th>Year</th>
<th>Survivors (millions)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1971</td>
<td>3</td>
</tr>
<tr>
<td>2001</td>
<td>9.8</td>
</tr>
<tr>
<td>2016</td>
<td>15.5</td>
</tr>
<tr>
<td>2026 (Projected)</td>
<td>20.3</td>
</tr>
</tbody>
</table>

Cervical Cancer: Reduced Incidence of Cancer-Causing HPV Infections

Among teenage girls, widespread use of the quadrivalent human papilloma virus (HPV) vaccine has driven down infection rates by nearly two-thirds.

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, opening up new treatment approaches.

60 YEARS AGO

“Disease of the Blood”

50 YEARS AGO

Leukemia

Lymphoma

40 YEARS AGO

Chronic Leukemia

Acute Leukemia

Pre-leukemia

Indolent Lymphoma

Aggressive Lymphoma

TODAY

~ 40 Unique Leukemia types identified

~ 50 Unique Lymphoma types identified

5 year survival rates have grown to 70%

There are nearly 250 medicines in development for blood cancers

Targeted Therapies Drive Survival Gains in Chronic Leukemias

Since the approval of the first tyrosine kinase inhibitor (TKI) for chronic myeloid leukemia (CML), survival rates have improved dramatically and patients are living close to normal life spans.¹

- Imatinib—the first TKI—was approved in 2001 to treat CML. The transformative impact of this class of medicines had not been completely realized.
- After initial approval, continued research revealed that imatinib had a greater impact when initiated earlier in the progression of the disease.
- Further research also revealed that imatinib was effective in combating other types of cancer.
- Additional TKIs have since been approved for CML and offer alternatives to imatinib.

5-Year Survival Rates for CML Patients Nearly Triple After Introduction of Imatinib²

Prior to Introduction of Imatinib: 31%
After Introduction of Imatinib: 89%

Initially approved to treat patients with ALK+ mutated NSCLC, rapidly evolving science and ongoing research revealed that crizotinib is effective in treating another rare, difficult-to-treat form of the disease as well.

2011
Accelerated approval in ALK+ patients, based on evidence of tumor shrinkage (surrogate endpoint)

2013
Regular, confirmatory approval granted based on superior progression-free survival

2016
NEW indication approved for patients with a different genetic subtype (ROS-1+)

“The expanded use of Xalkori will provide a valuable treatment option for patients with the rare and difficult to treat ROS-1 gene mutation by giving health care practitioners a more personalized way of targeting ROS-1 positive NSCLC.”

- Dr. Richard Pazdur, director of the Office of Hematology and Oncology Products, U.S. FDA Center for Drug Evaluation and Research

The Role of Personalized Medicines Has Grown in the Last Decade

Personalized medicines provide effective and efficient care by targeting the right medicine to the right patient.

Oncology Treatment Modalities in Top Pharmaceutical Markets, Share of Sales, 2003-2013

Cancer Treatment Advances Result in Substantial Gains to Society

Between 1988 and 2000:

- **23 million** years of life saved due to cancer treatment advances
- **$1.9 trillion** value of improved cancer treatment to society based on improved productivity, extended life and other factors

Continued Innovation in Cancer Treatment Could Echo HIV/AIDS Successes

“[W]e foresee accomplishing in oncology what has been achieved against other major public health problems, such as HIV/AIDS, in which scientific advances yielded major gains for patients and averted a predicted health spending crisis.”

- Turning the Tide Against Cancer Initiative

2. Emerging Advances
Transforming Cancer Care
Promise in the Pipeline: More than 800 Medicines in Development for Various Cancers

Number of Medicines in Development in the United States, September 2015, Selected Cancer Types*

<table>
<thead>
<tr>
<th>Cancer Type</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
<th>Application Submitted</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bladder Cancer</td>
<td></td>
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<td></td>
<td></td>
</tr>
<tr>
<td>Brain Cancer</td>
<td></td>
<td></td>
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<tr>
<td>Breast Cancer</td>
<td></td>
<td></td>
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<td></td>
</tr>
<tr>
<td>Colorectal Cancer</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Hematological Malignancies</td>
<td></td>
<td></td>
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<td></td>
</tr>
<tr>
<td>Kidney Cancer</td>
<td></td>
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<tr>
<td>Leukemia</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Liver Cancer</td>
<td></td>
<td></td>
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<td></td>
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<tr>
<td>Lung Cancer</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lymphoma</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Multiple Myeloma</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Ovarian Cancer</td>
<td></td>
<td></td>
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<tr>
<td>Pancreatic Cancer</td>
<td></td>
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<td></td>
</tr>
<tr>
<td>Prostate Cancer</td>
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<td></td>
<td></td>
</tr>
<tr>
<td>Skin Cancer</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Stomach Cancer</td>
<td></td>
<td></td>
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<td></td>
</tr>
</tbody>
</table>

“These are exciting times… the pace of discovery and application of new knowledge to patient care is rapidly accelerating.”

— Dr. Jose Baselga,
Physician-in-Chief,
Memorial Sloan Kettering Cancer Center

*Some medicines are being explored in more than one therapeutic category.

Promise in the Pipeline: More than 200 Immuno-oncology Medicines in Development

Number of Medicines in Development in the United States, May 2017, Selected Classes of Immunotherapy

- Adoptive Cell Therapies: 40
- Bispecific Antibodies: 30
- Checkpoint Modulators: 45
- Cytokines: 23
- Oncolytic Cell Therapies: 14
- Vaccines: 96

“In the past 5 years, immunotherapy has emerged as one of the most exciting new approaches to cancer treatment that has ever entered the clinic.”

- American Association for Cancer Research

New Approaches to Treating Cancers Represent the Majority of Medicines in the Oncology Pipeline

Researchers are using novel approaches to attack cancer at the molecular level. An average of 85% of drugs in the oncology pipeline, including 79% in the clinical research phase, may be first-in-class medicines.

Percentage of Projects in Development that are Potentially Novel Approaches in Selected Cancer Areas, 2016

- Bladder cancer: 71%
- Blood cancers: 83%
- Breast cancer: 80%
- Colorectal cancer: 78%
- Lung cancer: 88%
- Melanoma: 92%
- Prostate cancer: 84%
- Cancer, general: 85%

Biopharmaceutical Companies are Researching New Targeted Cancer Therapies

42% of all medicines in development have the potential to be personalized medicines.

73% of cancer medicines.
Immunotherapy is Revolutionizing the Treatment of Many Advanced Cancers: Metastatic Melanoma

"No recent cancer advance has been more transformative than immunotherapy."

- Dr. Julie M. Vose, former President of the American Society of Clinical Oncology

Major Scientific Advances in Cancer Treatment Pipeline Give Patients Hope

The cancer pipeline is ripe with innovative therapeutic options. Emerging combinations of medicines hold particular promise for controlling and killing cancer cells.

Chimeric antigen receptor (CAR) T-cell adoptive cell therapy involves the modification of individuals’ immune-boosting T-cells to target and kill blood cancer cells.

PARP inhibitors interrupt cancer’s hyperactive DNA repair systems, thus allowing tumors to be crippled and die.

Oncolytic viral therapies zero in on cancer cells, replicate, and cause them to rupture.

CRISPR/Cas9 gene editing allows researchers to manipulate cancer cell function.

Immunotherapies help target and kill cancer cells by unleashing the immune system.

“We are in the midst of a sea change in how we are treating cancer. We’re really seeing the fruits of many years of research into what drives cancer and how it interacts with the immune system to defeat it and survive.”

- Dr. Louis Weiner, director of the Georgetown Lombardi Comprehensive Cancer Center

Engineered immune T-cells can recognize, zero in on, and kill cancer cells.
Developing a new cancer medicine is a complex process, fraught with setbacks, but these so called “failures” are not wasted efforts. Researchers learn from them to inform future study and direct research efforts.

Cancer Researchers Build on Knowledge Gained from Setbacks in Order to Inform Future Advances

- **MELANOMA**
  - 96 unsuccessful attempts
  - 7 new medicines

- **BRAIN CANCER**
  - 75 unsuccessful attempts
  - 3 new medicines

- **LUNG CANCER**
  - 167 unsuccessful attempts
  - 10 new medicines

*Setbacks and advances from 1998 to 2014

3. Cancer Patient Spending and Financial Burden
Understanding financial hardship among cancer patients requires looking at a broad range of medical and non-medical factors.

Multiple Factors Contribute to the Financial Burden Faced by Cancer Patients

Top Patient Financial Concerns*

- Co-pay for facility/doctor visits: 10.4%
- Transportation: 8.8%
- Co-pay for drugs: 7.4%
- Rent/mortgage: 6.2%

*Among cancer patients who contacted the National Patient Advocate Foundation for financial help with in 2015.

Multiple Factors Contribute to the Financial Burden Faced by Cancer Patients

More than 40% of patients say medical and non-medical costs cause equal financial hardship.

### Top Patient Financial Concerns

<table>
<thead>
<tr>
<th>Non-Medical</th>
<th>Medical</th>
</tr>
</thead>
<tbody>
<tr>
<td>Gasoline</td>
<td>Diagnostic Tests or Scans</td>
</tr>
<tr>
<td>Food (Groceries or Dining Out)</td>
<td>Prescription Medicines</td>
</tr>
<tr>
<td>Over-the-Counter Medicines</td>
<td>Physician Office Visits</td>
</tr>
<tr>
<td>Special Clothing and/or Wigs</td>
<td>Outpatient Treatments (Incl. Radiation)</td>
</tr>
<tr>
<td>Car Repairs</td>
<td>Surgery</td>
</tr>
</tbody>
</table>

A Cancer Diagnosis Impacts Productivity and Employment for Patients and Caregivers

Patients

67% of patients who were employed full-time when diagnosed either stopped working or reduced their work hours

Caregivers

More than 25% of cancer caregivers made extended employment changes

Out-of-Pocket (OOP) Costs for Other Services Account for Larger Share than Cancer Drugs

At 6 months post-diagnosis, cancer treatments represent less than 10% of OOP spending for commercially insured patients with breast, lung and colorectal cancer on average. 60-70% of OOP costs are driven by physician and non-inpatient facility costs.

Breast Cancer Patient Out-of-pocket Costs
At 6 Months Following Diagnosis

- 41% Professional Services
- 22% Facility Services
- 21% Non-Cancer Drugs
- 8% Cancer-Related Drugs
- 5% Radiation Therapy
- 4% Hospital Inpatient

Out-of-pocket (OOP) costs can be high when insurance doesn’t cover the services patients need. For cancer patients with high OOP spending, a large portion of their costs are due to out-of-network (OON) services.

### Percent of Total Out-of-pocket Costs Incurred Out of Network, All Cancers (2011)

- **Low (10th percentile):** 7%
- **Moderate (50th percentile):** 7%
- **High (90th percentile):** 10%
- **Highest (99th percentile):** 41%

Some plans place treatments for certain high-cost conditions on the highest drug formulary cost sharing tier (sometimes called adverse tiering).

### Percentage of Silver Plans Placing All Drugs per Class on Specialty Tier, 2016

- **Molecular Target Inhibitors***: 23%
- **Antiangiogenics***: 50%

*There are no generic drugs available in this class. All products are single-source.

Source: Avalere Health PlanScape®, a proprietary analysis of exchange plan features, April 2016. This analysis is based on data collected by Managed Markets Insight & Technology, LLC.
High Cost Sharing Leads to Abandonment or Delays in Cancer Treatment

Oral Oncolytic Abandonment Rate by Patient Out-of-Pocket Amount

- Highest co-pay group was 6 times more likely to delay treatment than the lowest co-pay group.

4. Cancer Costs in Context
Spending on Cancer Medicines Represents About 1% of Overall Health Care Spending

Cancer Medicines as a Portion of NHE Projected Total U.S. Health Care Spending, 2016

- Cancer Drug Spending: $45.5 Billion**
- All Remaining Health Care Spending: $3.3 Trillion*

* 2016 CMS total National Health Expenditures is a projection
** Cancer drug invoice spending and does not include discounts

Cancer Medicines Represent About 20% of Cancer Spending

**Medicare, Actively Treated Cancer Population, 2014**

- 18% Cancer Drugs
- 5% Hospital Inpatient
- 21% Radiation Oncology
- 3% Cancer Surgeries
- 8% Other Out Pt Services
- 11% Professional Services

**Commercially Insured, Actively Treated Cancer Population, 2014**

- 20% Cancer Drugs
- 4% Hospital Inpatient
- 28% Radiation Oncology
- 13% Cancer Surgeries
- 10% Other Out Pt Services
- 4% Professional Services

The trend of volume-weighted Average Sales Price (ASP) for cancer drugs administered through Medicare Part B has been growing in line with medical inflation.

Weighted ASP (Oncology Drugs vs. All Other Drugs) vs Consumer Price Index – Medical (CPI-M)

* 2015 and 2016 Weighted ASP numbers are projections.

Better Use of Cancer Medicines Can Reduce Health Care Costs

Advanced melanoma patients who were adherent to immunotherapy experienced 10% lower health care costs.

**Source:** Gupte-Singh K, Lin J, Lingohr-Smith M, Menges BL, Rao S. Adherence to cancer therapies and the impact on healthcare costs among patients with advanced melanoma in the USA. Proceedings of the 22nd Annual International Meeting International Society of Pharmacoeconomics and Outcomes Research; 2017 May; Boston, MA. Abstract available at: https://www.ispor.org/ScientificPresentationsDatabase/Presentation/70971?pdfid=49558
5. Cancer Spending and Evolving Market Dynamics
Health Plans Have Powerful Tools to Control Spending on Cancer Medicines

Current and Anticipated Payer Measures to Manage Oncology Costs (Q1 2017)

- Utilizing one or more value frameworks* in determining reimbursement: 31%
- Using clinical pathways to determine treatment regimens: 35%
- Contracting for preferred first-line therapies: 59%

*Value Frameworks: NCCN Evidence Blocks, ASCO Value Framework, etc.

Source: Zitter Health Insights, Managed Care Oncology Index, 2016.
Non-small cell lung cancer patients treated according to a clinical pathway incurred lower drug and total costs.

Market Shift to New Payment Models

Early results from oncology medical homes, bundled payment and specialty ACOs show potential for reducing cancer costs.

- Patient-centered medical homes: Pilot reduced total costs 35% annually
- Episode- or bundled payment: Pilot reduced total costs of care 34%
- Specialty ACOs: Reduce drug spending 5%, total spending 2% +

“Notably, all those interviewed suggested that the use of clinical pathways was a driver of financial savings, either through reduced drug spending or indirectly through more appropriate patient treatment.”

- Sonal Shah, PharmD, and Greg Reh, American Journal of Managed Care

Manufacturers and Health Insurers Pursuing Novel Outcomes-Based Contracts

Shared risk or outcomes-based contracts (OBCs) between health insurers and manufacturers are becoming more common across diseases, including oncology.

35% of payers with outcomes-based contracts have or are considering OBCs in oncology.

Private Sector Risk-Sharing Contracts Announced

Late 1990s-2013: 7
2015-2017: 16

Cancer Treatments Face Growing Competition

Total Oncology Sales at Risk Due to Estimated Loss of Exclusivity
(Billions of US Dollars)

Sources: QuintilesIMS Market Prognosis, National Sales Perspectives, QuintilesIMS Institute, Mar 2017. Includes small and large molecules.
Market Drives Rapid Switch to Generic Medicines: Example - Injectable Cancer Medicine Docetaxel

Market Drives Rapid Switch to Generic Medicines: Example - Injectable Cancer Medicine Gemcitabine

Site of Care Shifts Drive Higher Cancer Costs

Infused Chemotherapy Drug Spending by Site of Service

Costs per episode of care for several types of cancers were **38% higher in the hospital** compared to similar services provided in physician offices.

Hospital Consolidation Associated with Increases in Cancer Spending

A 1% increase in the proportion of medical providers affiliated with hospitals and/or health systems is linked to significant spending increases.

Changes associated with a 1% increase in the proportion of medical providers affiliated with hospitals and/or health systems

- Per Person Price of Treatment: 23%
- Per-Person Annual Spending: 34%

Other developed countries use centralized government price setting and coverage decisions to manage drug spending, resulting in access to fewer medicines than in the US.

Availability and Reimbursement Status by 2015 of 49 Cancer Medicines
Launched Globally 2010 to 2014

Note: In the UK and Scotland, only drugs reimbursed through NICE and the SMC were included among “Reimbursed” drugs. Any additional medicines reimbursed through the Cancer Drug Fund (CDF) were not included in the Reimbursed category due to the uncertainty of the continuation of this fund.

Source: IMS Institute for Healthcare Informatics, May 2016.
US Patients Have Access to New Cancer Medicines on Average About Two Years Earlier in the US Than Patients in Other Developed Countries

Other developed countries use centralized government price setting and coverage decisions to manage drug spending, resulting in significantly slower access to medicines than in the US.

Average Time Delay Compared to the US in the Approval and Reimbursement of Oncology Medicines from 2010 to 2014

<table>
<thead>
<tr>
<th>Country</th>
<th>Delay Between US Approval and Country-Specific Approval</th>
<th>Delay Between Country Approval and Reimbursement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Germany</td>
<td>10</td>
<td>4</td>
</tr>
<tr>
<td>France</td>
<td>10</td>
<td>7</td>
</tr>
<tr>
<td>UK</td>
<td>10</td>
<td>13</td>
</tr>
<tr>
<td>Italy</td>
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<td>Spain</td>
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<td>Australia</td>
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<td>17</td>
</tr>
<tr>
<td>Taiwan</td>
<td>15</td>
<td>22</td>
</tr>
</tbody>
</table>

6. Solutions For Advancing Value in Cancer Care
As stakeholders seek solutions to ensure patient access to the treatments they need and accelerate the discovery of tomorrow’s cures, biopharmaceutical companies are working to advance patient-centered solutions to drive better value.

**SOLUTIONS for BETTER CANCER CARE**

- Expand Value-Based Contracts
- Improve Use of Medicines
- Strengthen Decision Support Tools
- Develop Quality Measures
- Increase Availability of Evidence on Value
### Enabling the Cancer Drug Market’s Move to Value

#### Value Based Contracts
- Expand value-based contracts by modernizing outdated regulations.

> “[R]egulatory reforms can address these concerns and encourage more robust competition within the drug market.”

- Scott Gottlieb & Kavita Patel

#### Value Frameworks
- Develop better data and tools to support informed decision-making by patients, physicians and payers.

> “[E]merging approaches for assessing drug value are welcome….The frameworks will require refinement, however, before they're ready to be broadly applied.”

- Peter Neumann & Joshua Cohen

#### Quality Measures
- Close gaps in clinical and patient-focused quality measures.

> “All phases of the cancer care continuum…need new measures.”

- National Academy of Medicine

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A Commitment to Solutions for Patients

To learn more about how the biopharmaceutical industry is working to support patient-centered, high-value health care visit:

phrma.org/advocacy/the-value-collaborative