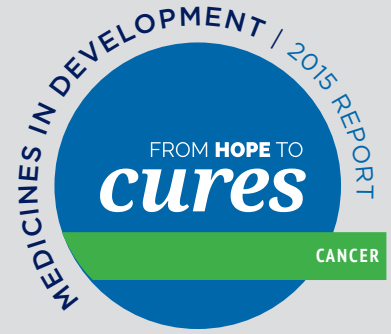

MEDICINES IN DEVELOPMENT FOR CANCER

A REPORT ON CANCER

More Than 800 Medicines and Vaccines in Clinical Testing for Cancer Offer New Hope to Patients

In recent decades, tremendous – almost previously unthinkable – progress has been made in the fight against cancer. Advances in molecular and genomic research have revealed underlying complexities and provided insights into cancer, which we now know is actually more than 200 unique diseases. Continued research has expanded our knowledge of how the disease develops and how to target medicines for specific cancer types – resulting in more effective therapies for patients.

Evidence of progress can be found in the number of cancer survivors living in the United States – a number that has increased from 3 million in 1971 to 14.5 million in 2014.[1] Currently, about 4 percent of Americans are cancer survivors.[1] Although great progress has and continues to be made in the fight against cancer, this complicated disease remains what author Siddhartha Mukherjee termed "the emperor of all maladies" – it is the second leading cause of death in the United States, accounting for nearly 1 of every 4 deaths. It is estimated that in 2015, more than 1.6 million new cancer cases will be diagnosed, and nearly 600,000 Americans will die from cancer, a rate of approximately 1,600 people per day.[1]

America's biopharmaceutical companies are responding to the needs of cancer patients, working to develop innovative approaches for more targeted treatments. Researchers are exploring new high-tech methods to fight the disease as well as new ways to maximize the use of existing medicines, either alone or in combination with other therapies, to treat various forms of the disease. In fact, biopharmaceutical companies are developing 836 medicines and vaccines[5] for cancer, all of which are in clinical trials or awaiting review by the U.S. Food and Drug Administration (FDA). The medicines in development include:

123 *for lung cancer, the leading cause of cancer death in the United States, with more than 158,000 deaths expected in 2015.*[1]

106 *for several types of leukemia, which account for more than 3 percent of all new cases of cancer.*[1]

92 *for lymphoma, including non-Hodgkin lymphoma which accounts for more than 4 percent of all new cancer diagnoses.*[1]

82 *for breast cancer, the leading cancer diagnosed in women in the United States with 231,840 new cases expected in 2015.*[1]

58 *for brain tumors, including gliomas, which represent 80 percent of all malignant brain tumors.*[6]

53 *for skin cancer, including melanoma, which accounts for 2 percent of all skin cancer cases, but most of the skin cancer deaths.*[1]

JUST THE FACTS

Cancer death rates have declined

22%

since the early 1990s[1]

83%

of survival gains in cancer are attributable to new treatments, including medicines[2]

Approximately

80%

of pipeline cancer drugs are potentially first-in-class medicines[3]

73%

of cancer medicines in the pipeline have the potential to be personalized medicines[4]

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(cont from page 1)

Other medicines in development target colorectal, ovarian, prostate and childhood cancers as well as solid tumors, among others.

The progress made in the fight against cancer has resulted in better outcomes for patients, helping them to lead longer, healthier lives. The more than 800 medicines being developed represent our best hope for continuing that progress and lessening the burden of cancer for patients around the world.

Some medicines are listed in more than one category. For a complete list of the 836 medicines in development, please visit http://phrma.org/sites/default/files/pdf/2015_cancer_drug_list.pdf

Medicines and Vaccines in Development to Treat Cancer

Many of the medicines in the pipeline today are using novel approaches to attack cancer at the molecular level. Among the 836 medicines and vaccines in development are potential treatments for:

COLORECTAL CANCER

A humanized monoclonal antibody in development for metastatic colorectal cancer targets the cell surface protein "endosialin," which is expressed on cells that are part of the tumor blood vessel structure. In preclinical studies, blocking the function of endosialin inhibited tumor growth and metastases.

LIVER CANCER

A medicine in development for hepatocellular carcinoma (the most common form of liver cancer) is a small molecule kinase inhibitor designed to selectively block transforming growth factor-beta (TGF-beta) signaling. The overexpression of TGF-beta in cells may enhance tumor growth and intensify metastases.

SOFT TISSUE SARCOMA

In malignant solid tumors, oxygen levels are often low (called tumor hypoxia) relative to oxygen levels in healthy tissue. Tumor hypoxia is associated with tumor progression, metastases, and resistance to chemotherapy and radiation treatment. A medicine in development is

Chronicling Progress: AACR Cancer Progress Report

AACR American Association for Cancer Research
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Cancer research is advancing rapidly and new medicines in development take many innovative approaches to fighting the disease.

The American Association for Cancer Research (AACR) – the oldest and largest professional organization in the world dedicated to advancing research to prevent and cure cancer – produces an annual "Cancer Progress Report" that encapsulates this progress and outlines the resources needed to continue to make further progress.

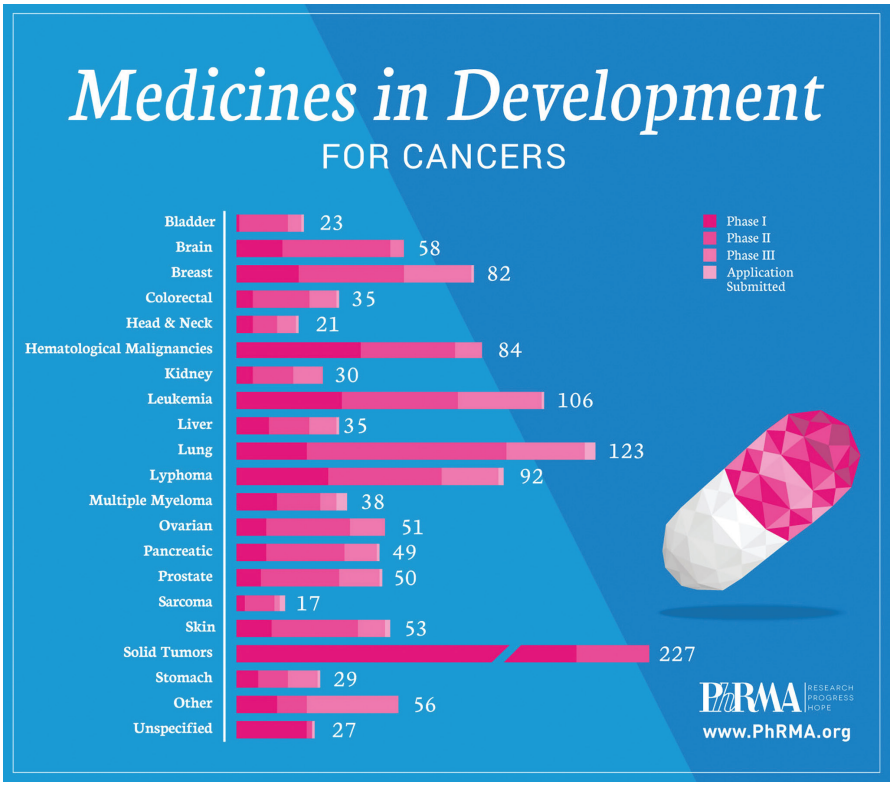
Although extraordinary advances are being made against cancer, the disease remains a major health care challenge and a huge financial burden, both nationally and internationally. Moreover, because most cancer diagnoses occur in those who are age 65 and older – a segment of the U.S. population that is expected to double by 2060 – it is predicted that the number of cancer diagnoses will increase dramatically in the future. The rising economic and personal burden of cancer highlights the crucial need for more research to develop new prevention and treatment approaches.

The fifth edition of the report, to be released Sept. 16, 2015, not only chronicles advances made against cancer between Aug. 1, 2014, and July 31, 2015, but also contains a special feature that documents the remarkable pace of progress against the collection of diseases we call cancer in the five years of publishing the report. The report delineates how advances in precision medicine are transforming lives, like those of the 13 courageous individuals who shared their experiences with cancer in the publication. These personal stories remind us that each advance, no matter how small, can be meaningful.

Learn more at **www.cancerprogressreport.org**

"RESEARCH IS THE FOUNDATION OF NEW AND BETTER APPROACHES TO CANCER PREVENTION, DETECTION, DIAGNOSIS, AND TREATMENT, WHICH ARE DRIVING DOWN OVERALL U.S. CANCER DEATH RATES AND INCREASING THE NUMBER OF PEOPLE WHO ARE LIVING LONGER, HIGHER-QUALITY LIVES AFTER A CANCER DIAGNOSIS."

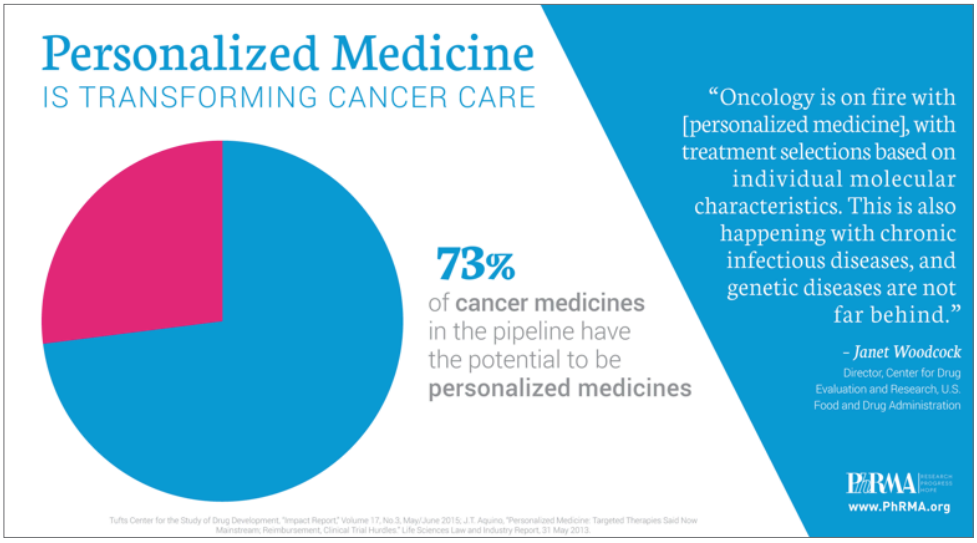
— American Association for Cancer Research



activated when it reaches the hypoxic region of the tumor environment where it eventually kills tumor cells in its vicinity, essentially attacking the tumor from the inside. If approved, it would be the first new medicine for soft tissue sarcoma patients in over 20 years.

STOMACH CANCER

A monoclonal antibody in testing for stomach cancer with high MET (mesenchymal-epithelial transition factor) expression is designed to inhibit hepatocyte growth factor (HGF)/scatter factor from binding to the c-MET receptor. The HGF/c-Met signaling pathway is thought to play a role in tumor growth and metastases in many cancers, including stomach cancer. An exploratory biomarker analysis found that the addition of the medicine to chemotherapy improved median overall survival in patients with tumors that expressed high levels of MET protein.



The 2015 PhRMA Research & Hope Awards

The PhRMA Research & Hope Awards recognize advances made across a critical disease or health condition by individuals or organizations in the biopharmaceutical sector, academia, government, and provider and patient organizations. The awards illustrate how biopharmaceutical researchers and others in the innovation ecosystem work together to not only bring new medical advances to patients, but thwart deadly diseases through increased awareness, public health efforts, and increased collaboration.

This year's program celebrates the progress and promise of oncology research and care. Awards will be presented in the following categories:

- Academic Research: J. Silvio Gutkind, Ph.D., National Institutes of Health
- Biopharmaceutical Industry Research: Merck & Co., Inc.
- Excellence in Advocacy & Activism: Vicki Kennedy
- Community Champion: Rick Dunetz, Side-Out Foundation
- Visibility & Progress: Liz and Jay Scott, Alex's Lemonade Stand

SPOTLIGHT: BREAST CANCER[1]

An estimated 234,190 new cases of breast cancer are expected to be diagnosed in 2015, with 99 percent of those in women. While more than 40,000 Americans are expected to die from breast cancer in 2015, death rates have steadily decreased in women since 1989. These decreases represent improvements in both early detection and treatment. There are 82 medicines being tested as potential treatments for breast cancer.

SPOTLIGHT: CHILDHOOD CANCER[1]

Although uncommon, cancer is the second leading cause of death in children ages 1-14, exceeded only by accidents. In 2015, an estimated 10,380 new cases of cancer are expected to occur among children less than 14 years of age, representing less than 1 percent of all new cancer diagnoses. About 1,250 children are expected to die from cancer this year. Survival rates for childhood cancers have increased 43 percent since the mid-1970s. The most common types of childhood cancer are leukemia (30 percent of all childhood cancers), and brain and central nervous system tumors (26 percent). Other pediatric cancers include: neuroblastoma, Wilms tumor, non-Hodgkin lymphoma, Hodgkin lymphoma, rhabdomyosarcoma, osteosarcoma, retinoblastoma and Ewing sarcoma.

SPOTLIGHT: MELANOMA[1]

Melanoma accounts for less than 2 percent of all skin cancer cases, but the vast majority of skin cancer deaths. An estimated 73,870 new cases of melanoma will be diagnosed in 2015, and 9,940 deaths from melanoma will occur this year. The treatment of advanced melanoma has changed in recent years with the FDA approval of targeted drugs and cancer immunotherapy. There are 53 medicines being tested as potential treatments for skin cancer.

SPOTLIGHT: GLIOMA[6]

There are over 120 types of brain tumors. Gliomas, a broad term which includes all tumors arising from the supportive tissue of the brain, represent 30 percent of all brain tumors and 80 percent of all malignant brain tumors. There are three types of glial cells that can produce tumors – astrocyte cells (which produce astrocytomas and glioblastomas), oligodendrocyte cells and ependymal cells. Mixed gliomas show a mixture of these different cells. Astrocytomas and glioblastomas combined represent 76 percent of all gliomas. There are 58 medicines being tested as potential treatments for brain tumors, including gliomas.

Footnotes:

1. American Cancer Society

2. E. Sun, et al., "The Determinants of Recent Gains in Cancer Survival: An Analysis of the Surveillance, Epidemiology, and End Results (SEER) Database," Journal of Clinical Oncology, May 2008 Supplement (Abstract 6616)

3. Analysis Group

4. Tufts Center for the Study of Drug Development

5. Number of medicines obtained through public, government and industry sources, and the Adis "R&D Insight" database; current as of August 21, 2015

6. American Brain Tumor Association

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