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 vaccines5 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
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...
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
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
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
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
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
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