

Over the past decade, pharmaceutical companies have pushed the scientific envelope, working at the cellular and molecular levels to dramatically advance the treatment of disease. At the end of 2002, 28 percent more medicines were being investigated by pharmaceutical companies for approval by the Food and Drug Administration (FDA) than was true one decade before.<sup>1</sup> More than 1,000 medicines are now in the development pipeline.<sup>2</sup>

Between 1993 and 2003, more than 300 new drugs, biologics, and vaccines that prevent and treat over 150 conditions were approved by the FDA.<sup>3</sup> The FDA also gave the go-ahead for numerous new indications for previously approved medicines, allowing physicians to tailor treatment strategies to meet a patient's individual disease status, past medication history, side effect tolerance, and preferences.

The new medicines that are the product of this decade of innovation have dramatically changed the "standard of care" for several major conditions—including, among others, those highlighted in this publication. Medical treatment guidelines have been revised to recommend early intervention with these new, more effective medicines.

Throughout the decade, pharmaceutical companies shifted research to more complex diseases, clinical trial failure rates remained high, and a rigorous regulatory environment prevailed. The result of these growing demands on drug development has been an escalation in the cost to develop new drugs. Additionally, the marketplace has become more demanding, more patients qualify for treatment, and medicines are playing an increasing role in patient health care. All of these factors have contributed to the recent increase in prescription drug costs, even though prescription drug expenditures still make up only a small fraction of every dollar spent on health care.<sup>4</sup>

This report, the first in a series, focuses on eight conditions that affect millions of Americans: rheumatoid arthritis (RA), HIV/AIDS, Parkinson's disease, Alzheimer's disease, schizophrenia, diabetes, high blood pressure, and high blood cholesterol. Years ago, diagnosis of many of these diseases often meant death following a terrible illness. Today, the picture is quite different. Many people with these conditions are able to lead productive, healthy lives, due in large part to the rapid pace of discovery and innovation in pharmaceutical treatments.

## A Decade of Innovation



NEW MEDICINES. NEW HOPE.

**Pfizer**  
**PRMA**

# Rheumatoid Arthritis

*Rheumatoid arthritis is a chronic inflammatory autoimmune disease that primarily affects the joints. In this disease, the body's immune system attacks the cells of a fluid that surrounds the joints.*

Medicines seek to relieve the symptoms of RA in three main ways: reducing pain, decreasing inflammation, and slowing damage to the joints. Before 1998, treatment of rheumatoid arthritis depended largely on **non-steroidal anti-inflammatory drugs (NSAIDs)** like aspirin. However, since 1998, patients suffering from rheumatoid arthritis have benefited from a surge in the approval of new treatments for their often painful condition.

## PHARMACEUTICAL ADVANCES

In 1998, the FDA approved the first new **disease-modifying anti-rheumatic drug (DMARD)** specifically developed for the treatment of rheumatoid arthritis in

more than a decade. This class “has the potential to reduce or prevent joint damage, preserve joint integrity and function, and ultimately, reduce the total costs of health care and maintain economic productivity of the patient with RA.”<sup>5</sup> The newest product can be used alone or in combination with other DMARDs, thus allowing physicians to maximize patient responses to both products.



That same year, the FDA also approved the first in a new category of biologic products known as **biological response modifiers**. Medical products in this category reduce inflammation by blocking the protein in the immune system that causes excessive inflammation in those with RA.<sup>6</sup> These medicines are especially helpful in patients who no longer respond to DMARDs, and many patients' conditions improve within the first two weeks of treatment.<sup>7</sup>

Advances in drug treatment continued in 1998 with the FDA's approval of a medicine in a third new class of drugs known as **COX-2 (cyclo-oxygenase-2) inhibitors**. Like NSAIDs, these drugs block COX-2, an enzyme that causes inflammation. However, unlike NSAIDs, they do not block COX-1, an enzyme that protects the lining of the stomach, thus reducing risk of the gastrointestinal ulcers and bleeding that can occur with NSAIDs.<sup>8</sup> According to the American College of Rheumatology, “patients with RA are nearly twice as likely as patients with osteoarthritis to have a serious complication from NSAID treatment.”<sup>9</sup>

## DEVELOPING NEW MEDICINES

Although RA still has no cure, drug treatments are helping patients live more comfortable, productive lives. Currently, 29 new medicines to treat RA are undergoing testing in pharmaceutical company laboratories. These include:

- **therapy focused on the early stages of the immune response to block only those specific immune system cells involved in autoimmune disease;**
- **so-called “next generation biologics,” including co-stimulatory blockers that prevent the initial signaling and chain of chemical reactions that turn on the immune system; and**
- **therapies that inhibit the migration of inflammatory cells into the joint tissues, thus preventing cartilage and bone destruction.**

Trials of gene therapy products that affect factors regulating the immune system also have shown promising results.

*“The management of RA has undergone a revolution in the past decade, reflecting a growing armamentarium of drugs and a shift in treatment strategies.”<sup>10</sup>*

With rheumatoid arthritis, the body's immune system attacks the cells of a fluid that surrounds the joints. This fluid normally lubricates and nourishes the bones and cartilage within a joint, but with RA, the inflammatory process causes this fluid to become thicker and begin to destroy the cartilage and bone. This leads to RA's characteristic effects on the joints: pain, swelling, and loss of function.<sup>11</sup> RA also



can lead to bone loss that causes osteoporosis, as well as the development of anemia, neck pain, dry eyes

and mouth, bumps under the skin, and very rarely, inflammation of the blood vessels, the lining of the lungs, or the sac enclosing the heart.<sup>12</sup>

# HIV/AIDS

*Like other viruses, the human immunodeficiency virus (HIV) that causes acquired immune deficiency syndrome (AIDS) replicates by entering a healthy cell and taking over its machinery.*

## PHARMACEUTICAL ADVANCES

Fourteen of the 17 medicines available to treat HIV infection have gained FDA approval in the past decade, including four new classes of medicines that target three different stages of the HIV virus' life cycle.

Two new classes of medicines, along with one class approved in the late 1980s, use different mechanisms to



interfere with the reverse transcriptase enzyme, thereby interrupting an early stage of the HIV life cycle. Medicines in the earliest class of drugs (**nucleoside analogues**) “provide faulty DNA building blocks, halting the DNA chain that the [HIV] virus uses to make copies of itself.”<sup>13</sup>

A second drug class, introduced in 1996 and called **non-nucleoside reverse transcriptase**

**inhibitors**, binds to the enzyme so it cannot copy itself. The third class, **nucleotide analogue reverse transcriptase inhibitors**, was first introduced in 2001. These drugs block the reverse transcriptase to prevent replication of the HIV virus.

A second enzyme, protease, is a critical player in a later stage of the HIV life cycle. The first drug in a class of **protease inhibitors** to combat this enzyme was approved in 1995. Health care providers combine protease inhibitors with the other classes of antiviral medicines in a strategy known as combination therapy (using more than one type of medicine to treat a condition). This strategy has been used to treat both early-stage and advanced-stage HIV disease and is an important factor

behind the significant decline in AIDS deaths in the United States in recent years.

In March 2003, the FDA approved the first in another new class of drugs that prevents the HIV virus from attaching to healthy cells. This class, known as **fusion inhibitors**, “blocks the virus’ ability to infect certain components of the immune system.”<sup>14</sup> Recent clinical trials showed that when added to combination therapy regimens, fusion inhibitors can decrease the amount of virus in the bloodstream to undetectable levels.<sup>15</sup> Because these drugs attack the HIV virus in a totally different way, they can be of particular benefit to individuals who have developed resistance to previously available medicines.

Beyond the entirely new classes of drugs, important innovations within existing drug classes have made the treatment of people living with HIV/AIDS more effective as well as more tolerable. For example, the first treatments had to be taken multiple times a day, but many new drugs are available in twice-daily or even once-daily dosage forms.

## DEVELOPING NEW MEDICINES

Another 83 medicines are now under development for AIDS and AIDS-related conditions, including 33 antivirals and 14 vaccines. These include, among others:

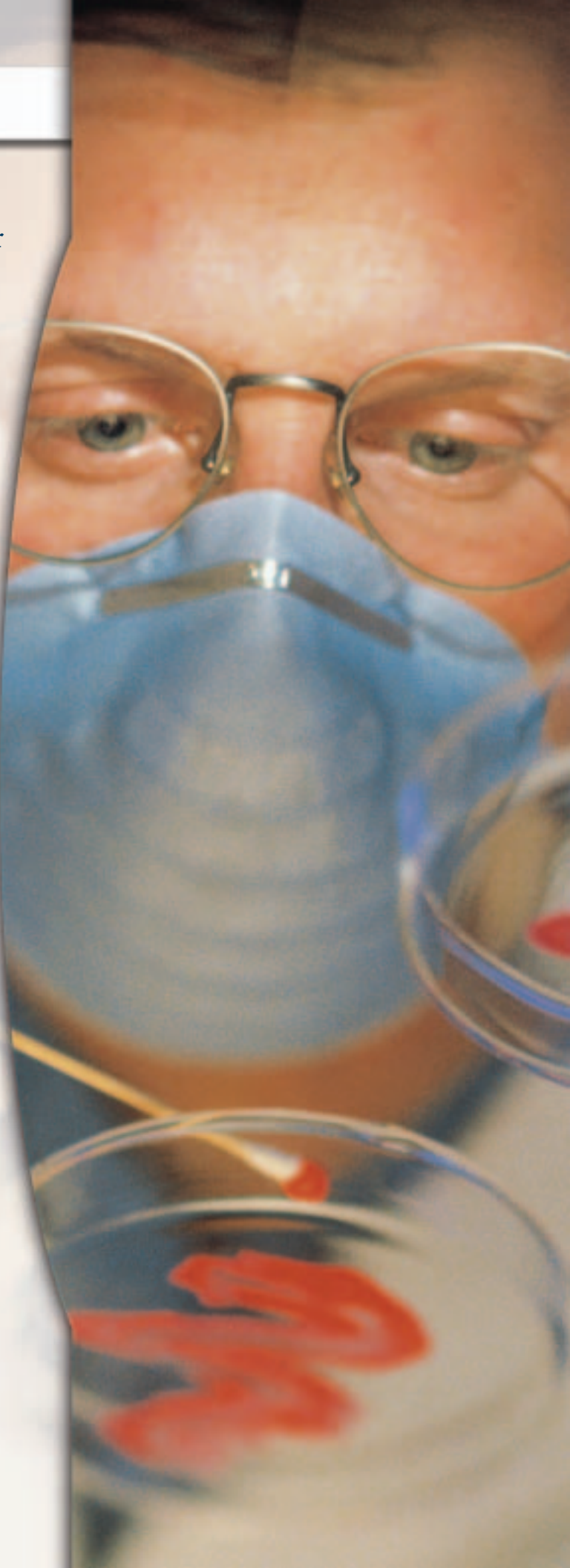
- an antisense gene therapy medicine that uses two novel technologies to boost immune responsiveness against HIV;
- a new medicine that blocks a third enzyme, known as integrase, that the HIV virus uses to copy itself; and
- a number of HIV/AIDS vaccines that may prevent the spread of the virus.

Although a cure has not yet been found for HIV/AIDS, new medicines that are the product of research have dramatically affected the length and quality of life for those infected with the HIV virus. Because people receiving pharmaceutical therapy for HIV/AIDS are better able to maintain their health, they use fewer health care services and use them less often. A study published in *The New England Journal of Medicine*, for example, found that in the 16 months after the introduction of antiretroviral therapy, hospital inpatient care for HIV patients



decreased by 43 percent. According to Samuel A. Bozzette, who headed the study, “The drugs are almost a perfect substitute for hospital care. We can afford them because, in fact,

we are already spending the money on HIV care” in the form of hospitalization.<sup>16</sup>



# Parkinson's Disease

*Parkinson's disease is a condition that results from the breakdown of neurons in the part of the brain that controls movement. This breakdown causes a shortage of a chemical called dopamine.*

Dopamine is responsible for relaying the brain's instructions for movement, and a lack of it causes the tremors, rigidity, and slower-than-normal movement that many Parkinson's patients experience.<sup>17</sup>

Until recently, patients suffering from Parkinson's disease usually were first treated with levodopa, a chemical that is converted to dopamine once it enters the brain. Unfortunately, levodopa is often broken down in the bloodstream before it reaches its target in the brain, causing decreased effectiveness.



## PHARMACEUTICAL ADVANCES

In 1997, researchers made a major advance in treating Parkinson's disease by introducing a second generation of medicines called **dopamine agonists**. These medicines mimic the effect of dopamine and stimulate neurons to act as though sufficient dopamine were present in the brain.<sup>18</sup> The development of dopamine agonists caused the American Academy of Neurology to revise its clinical practice guidelines in early 2002. The guidelines now include both levodopa and dopamine agonists as potential front-line treatments against Parkinson's disease.<sup>19</sup> The Academy stresses that the choice between treatments depends on each individual's needs.

In the past five years, patients have benefited from continuing innovation in medicines to treat Parkinson's disease. A new class of drugs introduced in 1998, known as **COMT (catechol O-methyltransferase) inhibitors**, blocks the enzymes that break down levodopa as it moves through the bloodstream, allowing it to get to the brain and be converted to dopamine. Consistent exposure to dopamine allows patients to function more independently and for longer periods of time between doses with fewer of the burdensome symptoms of Parkinson's disease.

Seventeen new medicines are now being tested as possible treatments for Parkinson's disease. One of these medicines may improve the success of transplanting dopamine neurons in the brains of Parkinson's patients. Although such transplants have been shown to reduce Parkinson's symptoms, only 5 to 10 percent of the transplanted neurons survive. This new compound is a selective and potent inhibitor of the stress-



activated protein kinase pathway, an essential component of the response that leads to the death of neurons. In laboratory tests, at least 300 percent

more neurons treated with the compound survived compared to those not treated.

# Alzheimer's Disease

*Alzheimer's disease (AD) is a progressive neurological disease that affects memory, personality, and behavior.*

The pathological processes involved in AD disrupt the three key functions of nerve cells in the brain—communication, metabolism, and repair. When these processes are disturbed, brain cells stop working, lose connections with each other, and eventually die. Over a period of years, those with AD gradually lose their ability to remember things and think clearly.<sup>20</sup>

## PHARMACEUTICAL ADVANCES

All four of the prescription medicines, belonging to two therapeutic classes, approved by the FDA to treat Alzheimer's disease have been developed in the past decade. The first class, **acetylcholinesterase inhibitors**, prevents the breakdown of a neurotransmitter in the brain called acetylcholine.<sup>21</sup> This chemical is thought to carry messages between nerve cells. The breakdown of acetylcholine can lead to disruptions in thinking and memory.<sup>22</sup> These medicines were first introduced in 1993. The second class, **cholinesterase inhibitors**, also prevents the breakdown of acetylcholine as well as another similar chemical, butyrylcholine. This class was first approved for use in 2000.

By preventing the breakdown of these chemicals, these new medicines ensure that more acetylcholine is available for memory-related and cognitive functioning. They also help with some behavioral problems commonly experienced by those with AD, including delusions and agitation. Although these drugs do not stop or reverse AD, they allow people with the disease to maintain their independence for longer periods of time. These AD treatment innovations are the current standard of care among neurologists for those with mild to moderate AD.<sup>23</sup>



As the Baby Boom generation reaches its older years, the number of Americans suffering from Alzheimer's disease is likely to increase dramatically, and this will have major financial and social consequences. As a result, the search for new AD treatment strategies is a high priority, and currently 20 new medicines are under development. For example, one new medicine now in clinical trials is the first in a new class of drugs known as **NMDA (N-methyl D-aspartate) receptor antagonists**.



The medicine works by modulating the levels of glutamate, a nerve-signaling agent in the brain. Too

much glutamate can lead to the death of nerve cells.



# Schizophrenia

*Schizophrenia is a condition that causes those who suffer from it to lose their sense of reality, become delusional, suffer from hallucinations, become emotionally unstable, and find it difficult to make decisions and relate to people.*

Little is known about the causes of schizophrenia and it has no cure, but medications are now available to treat many of the symptoms. The first medicines to treat schizophrenia were introduced in the 1950s, but these drugs often caused side effects such as muscle stiffness, tremor, and abnormal movements.<sup>24</sup>



## PHARMACEUTICAL ADVANCES

The past decade witnessed the introduction of new **atypical antipsychotic medicines**. These atypical antipsychotic medicines work by blocking receptors of the neurotransmitters dopamine and serotonin. Serotonin controls “mood, emotion, sleep and appetite and thus is implicated in the control of numerous behavioral and physiological functions,”<sup>25</sup> while dopamine acts on the cardiovascular, renal, hormonal, and central nervous systems.<sup>26</sup> These drugs appear to change the chemical balance of serotonin and dopamine in the brain.

The new medications are able to control the so-called “positive” symptoms of schizophrenia—symptoms and behavior that should not be there—as well as the “negative” symptoms—lack of characteristics that should be present<sup>27</sup>—thereby allowing patients to lead more normal, independent lives. Each of these new medications has “a unique side effect profile, but in general, these medications are better tolerated than the earlier drugs.”<sup>28</sup>

The introduction of new medicines caused an expert panel of physicians to rewrite their treatment guidelines in 1999, with atypical antipsychotics now considered to be the first-line therapy for the treatment of schizophrenia.<sup>29</sup>

## DEVELOPING NEW MEDICINES

Another 11 medicines are now being developed to treat schizophrenia.

Several studies have documented how atypical antipsychotic medications for the treatment of schizophrenia are helping patients live better, more productive lives. One study,



which examined the employment rates of schizophrenia patients using a new atypical antipsychotic medication, found that employment rates

doubled with the use of the new medication.<sup>30</sup> In another study, patients taking a new antipsychotic medication reported greater improvements in quality of life, better social functioning, and more improved ability to work than those taking an older drug.<sup>31</sup> The study found that patients taking the newer medicine were much less likely to discontinue maintenance therapy due to adverse events or “patient decision” than those taking the older drug. Patients using the newer medicine were also more likely to remain as outpatients, and were more likely to work full or part time.

# Diabetes

*Diabetes is a metabolic disorder in which the body is unable to make enough of and/or properly use the hormone insulin to control blood glucose levels.*

## PHARMACEUTICAL ADVANCES

During the past decade, research breakthroughs have led to the approval of two new **insulin** products to treat type 1 and advanced type 2 diabetes. One closely mimics the action of human insulin by providing a slow release over a 24-hour period, with no pronounced peak. The other medicine works quickly and for a short period of time, allowing patients to take the medication right before they eat a meal, instead of the 30 minutes before eating that insulin doses have traditionally required.

Beginning in 1995, a string of additional treatment advances have allowed people with type 2 diabetes to more effectively manage their condition.



Until 1995, only one category of oral medicines was available in the United States to treat patients with type 2 diabetes. This category of drugs, the **sulfonylureas (SU)**, was a major advance in treatment for type 2 diabetes because it was the first oral medicine that could be used

to treat the disease. Available in the United States since 1954, SU drugs stimulate the pancreas of a patient with type 2 diabetes to produce more insulin and remain an important part of diabetes treatment today.<sup>32</sup> New SU drugs with fewer side effects have been developed and are used as “monotherapy” or as part of combination therapy with other types of diabetes pills and/or insulin.

In 1995, one class of medicines known as **biguanides** was introduced in the United States after having been available in Europe for a number of years. This class lowers blood sugar levels by preventing the liver from making too much glucose and by improving the sensitivity of the muscle to the body’s own insulin.

Since 1995, four totally new classes of medicines have been introduced in the United States, allowing doctors to better customize treatment regimens to fit their patients’ needs. Another class approved in 1995,

**alpha-glucosidase inhibitors**, controls blood sugar by slowing down the digestion of carbohydrates in the small intestine after meals. By blocking the enzyme that digests carbohydrates, the medicine keeps blood sugar levels from rising too dramatically after a diabetic eats a meal.

A second new class of medications, known as **thiazolidinediones**, is designed to reduce insulin resistance. These medicines were first introduced in 1997. By making cells more sensitive to insulin, thiazolidinediones allow insulin to more effectively move sugar from the blood into cells.<sup>33</sup> The third class of type 2 diabetes medications, **meglitinides**, also was introduced in 1997. The drugs stimulate insulin secretion from the pancreas, which lowers blood sugar levels. The first drug in the most recent class of new medicines for type 2 diabetes was approved by the FDA in 2000. The class, **D-phenylalanine derivatives**, stimulates rapid, short-acting insulin secretion from the pancreas, effectively lowering overall blood sugar levels and blunting the increases in these levels that most people with type 2 diabetes experience after meals.

Because these medications have different mechanisms of action and different side effects, combination therapy can prevent patients from becoming hypoglycemic (having blood sugar levels that are too low) or experiencing serious complications such as kidney problems.

## DEVELOPING NEW MEDICINES

Currently, 24 new diabetes medicines are in development. These experimental pharmaceutical treatments include:

- a protein to promote increased insulin secretion when blood glucose levels are high but not when they are normal;
- inhaled forms of insulin that do not require injections;
- dual-acting sensitizers that increase muscle cell uptake of blood sugar and inhibit the liver’s production of blood sugars, as well as reduce blood lipid levels; and
- drugs that are designed to lessen diabetic nerve disease and complications involving small blood vessels, such as those in the eye or kidney.

Glucose provides the basic fuel for all cells in the body, and insulin transports glucose from the blood into the cells for storage. When glucose builds up in the blood instead of going into cells, it can cause two problems. Immediately, cells may be starved for energy, and over time, serious problems develop for many body systems.<sup>34</sup> According to the American Diabetes Association, “undiagnosed and untreated diabetes can lead to many serious and often fatal health conditions. Diabetes is the main cause of kidney



failure, new cases of blindness, and lower limb amputations, and is a major risk factor for heart disease and stroke.”<sup>35</sup>

# High Blood Pressure

*Approximately one in every four adults has high blood pressure,<sup>36</sup> a condition in which the force of blood against the walls of the arteries remains too high for an extended period of time.<sup>37</sup>*

High blood pressure is a symptomless condition, and nearly one-third of people with it do not know they have it. Fewer than 3 out of 10 people with blood pressure have it adequately controlled by medication.<sup>38</sup> High blood pressure can lead to stroke, blurred vision or blindness, congestive heart failure, heart attack, kidney damage, and hardening of the arteries.<sup>39</sup>

## PHARMACEUTICAL ADVANCES

Fortunately for patients with high blood pressure, major advances continue to be made in treating this condition. As pharmaceutical researchers have learned more about existing drug classes—such as **calcium channel blockers**, **ACE inhibitors**, **alpha-blockers**, **beta-blockers**, and **diuretics**—they have been able to develop new medicines with easier dosing schedules

(such as once-daily dosing) and better side effect profiles. Researchers also have learned that combining multiple types of high blood pressure medications can help patients.

Pharmaceutical researchers have continued to find new ways of attacking this potentially deadly condition. Over the past decade, two new therapeutic classes

for treating high blood pressure have been developed. The addition of these new medicines to the arsenal of weapons to treat high blood pressure allows doctors and patients to continue to choose the most effective and least burdensome treatment for each individual patient.

The first class, introduced in 1995 and known as **angiotensin-II antagonists**, blocks the hormone angiotensin-II. This hormone normally causes blood vessels to narrow, but angiotensin-II antagonists cause blood vessels to dilate, resulting in decreased blood pressure.<sup>40</sup> The formulation of these medicines allows patients to take them once daily and provides smooth, gradual, 24-hour blood pressure reduction. Like earlier treatment classes, each angiotensin-II antagonist may have a different side effect profile, allowing for customization of treatment for patients based on the medicines they can best tolerate.

In 2002, the FDA approved a second new class of medicines to treat high blood pressure—**selective aldosterone receptor antagonists**. These medicines work to block aldosterone, a hormone that helps the kidneys absorb sodium and water. If too much absorption takes place in the kidneys, blood pressure can increase. By blocking the hormone, selective aldosterone receptor antagonists can prevent that increase in blood pressure.

## DEVELOPING NEW MEDICINES

An additional 17 new medicines are now being developed to further refine and improve treatments for high blood pressure.



In May 2003, the National Institutes of Health's National Heart, Lung, and Blood Institute (NHLBI) updated its clinical practice guidelines, increasing the number of Americans who need to control their blood pressure. According to the chairman of the committee that produced the new guidelines, "Though improved, the treatment and control rates are still too low. The new guidelines zero in on this problem, recommending factors that often lead to inadequate control such as not pre-



scribing sufficient medication."<sup>41</sup> The NHLBI also notes that combination therapy allows physicians the ability to customize treatment to a patient's needs.

"... most persons will need two, and at times three or more, medications to lower blood pressure to the desired level."<sup>42</sup>



# High Blood Cholesterol

*High blood cholesterol is a primary risk factor for coronary artery disease, the nation's number one killer. Nearly 100 million Americans now meet the definition of having high blood cholesterol.<sup>43</sup>*

According to recently revised guidelines by the NHLBI, more than one-third of people with high blood cholesterol should be taking medications for this condition. These new guidelines, issued in May 2001, expand the drug therapy eligibility criteria to include patients with lower levels of cholesterol as well as patients with multiple risk factors—increasing the number of patients who should take cholesterol-lowering drugs from 13 million to 36 million.<sup>44</sup> According to NHLBI Director Dr. Claude Lenfant, if the recommendations in the guide-



lines were followed, heart disease “would no longer be the No. 1 killer.”<sup>45</sup>

Continuing the search for cholesterol-lowering medicines is critical to the cardiovascular health of millions of Americans, as

lowering cholesterol levels by 10 percent may decrease the incidence of heart disease by 30 percent.<sup>46</sup>

## PHARMACEUTICAL ADVANCES

During the past decade, pharmaceutical researchers have continued to develop the class of breakthrough cholesterol-lowering drugs known as **statins (HMG-CoA reductase inhibitors)**. First introduced in the late 1980s, statins act by preventing the body from manufacturing cholesterol, reducing absorption of dietary cholesterol, or removing cholesterol from the bloodstream. Some statins work by slowing down the liver’s production of cholesterol and increasing that organ’s ability to remove low-density lipoprotein (LDL) cholesterol already in the blood. Some statins also modestly increase high-density lipopro-

tein (HDL), which carries cholesterol to the liver, where it can be broken down and removed from the body, and reduce other fats in the blood.

The growing number of people who are now seeking and benefiting from drug treatment for high cholesterol has spurred pharmaceutical research companies to continue their efforts to find new ways to treat the condition. In 2002, a new class of medicines was approved by the FDA. These medicines, called **cholesterol absorption inhibitors**, act in the small intestine, keeping cholesterol from ever entering the liver. This means that less cholesterol is stored in the liver, and more is removed through the blood.<sup>47</sup> Because this class works differently than statins, it can be used in combination with statins, resulting in improved cholesterol levels.

Pharmaceuticals also play a supporting—and vital—role in a new procedure for coronary artery disease. In April 2003, the FDA approved a new kind of stent, called a drug-eluting stent, for use in angioplasty procedures to open clogged coronary arteries in individuals with heart disease. (Often, arteries are clogged due to high blood cholesterol.) The device holds the blood vessel open while the drug, which prevents a build-up of new tissue that can relog the artery, is slowly released. The drug-coated stent is an advance over older model stents because the drug it releases substantially reduces the need for repeat procedures. The new device has been shown to reduce reblockages and the need for repeat procedures from 15 to 30 percent to less than 5 percent.<sup>48</sup>

## DEVELOPING NEW MEDICINES

An additional eight new medicines are currently being developed to treat high cholesterol. For example, a new vaccine in development is designed to prevent the transfer of “good” cholesterol to “bad” LDL cholesterol, thus keeping “good” cholesterol levels high. The vaccine stimulates the body to produce antibodies to a protein responsible for the transfer process between HDL and LDL cholesterol.

The 2002 *Economic Report of the President* notes the value of cholesterol-lowering drugs, saying that, “even though annual expenditures on cholesterol-lowering drugs is well into the billions of dollars, they have been proved to be highly cost-effective for many patients and have contributed to the improved life expectancy and better



functioning of Americans today.”<sup>49</sup>

The report also notes, “it is just now being understood that the effectiveness of cholesterol-lowering drugs

depends significantly on the characteristics of the patient. As we develop a clearer understanding of the genetic and molecular mechanisms of diseases, treatments are likely to become even more tailored to individual circumstances.”<sup>50</sup>

# Conclusion

## PROMISE FOR THE FUTURE

As the examples in this report demonstrate, the past decade has witnessed many important gains in the treatment of conditions and diseases that affect millions of Americans. America's pharmaceutical research companies continue to work intensely to discover and develop new treatments for these debilitating conditions, investing an estimated \$32 billion in 2002 alone and more than \$200 billion in the past decade in researching and developing new medicines.<sup>51</sup>



In addition to continuing to emphasize the discovery of totally new ways to treat diseases, research continues on other possible uses for existing medicines. For example, a recent *New York Times* article notes that exploration into other uses for cholesterol-lowering statins may result in the prevention or treatment of stroke, Alzheimer's disease, osteoporosis, and multiple sclerosis or other autoimmune diseases.<sup>52</sup> Further understanding of these and other conditions and current methods of treatment will lead to more and better treatments for patients in the future.



# Endnotes

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