

RESEARCH *in* YOUR
BACKYARD

Developing Cures, Creating Jobs

Pharmaceutical clinical trials in
MONTANA



Executive

This report shows how biopharmaceutical research companies continue to be vitally important to the economy and patient health in Montana.

Since 2004, biopharmaceutical research companies have conducted or are conducting more than 1,200 clinical trials of new medicines in Montana in collaboration with clinical research centers, hospitals and local research institutions. These clinical trials have investigated or are investigating some of Montana's biggest health care challenges, including asthma, arthritis, cancer, diabetes, cardiovascular disease and gastrointestinal diseases.

Summary

Clinical trials in **MONTANA**

CLINICAL TRIALS IN MONTANA ARE A VITAL PART OF THE FDA DRUG APPROVAL PROCESS

In the development of new medicines, clinical trials are conducted to establish therapeutic effectiveness and safety and compile the evidence needed for the U.S. Food and Drug Administration (FDA) to approve new treatments.

Clinical trials of new medicines are typically conducted in three phases and, on average, account for nearly seven of the more than 10 years it takes to bring a new medicine from development to patients. Clinical trials are responsible for more than half of the \$2.6 billion average cost of developing one new innovative medicine.

Institutional Review Boards (IRBs), independent committees of physicians, statisticians, local community advocates and others, review and approve clinical trials in advance to ensure trials are ethically conducted and patient rights are protected.

Clinical Trials in Montana since 2004— Completed and Open

All Clinical Trials	Open Clinical Trials
1,253	130

Source: www.clinicaltrials.gov. Search criteria: Montana, United States; Phase: early 1, 1, 2, 3; Industry only, first posted on or after 1/1/2004. Search performed 10/6/2021. Open clinical trials are recruiting, not yet recruiting or expanded access available.

Executive Summary (cont.)

CLINICAL TRIALS MAY OFFER IMPORTANT THERAPEUTIC OPTIONS FOR PATIENTS

For patients, clinical trials may offer the potential for another therapeutic option, or provide for a treatment where no FDA-approved treatments exist. Clinical trials may provide a new avenue of care for some chronic disease sufferers who are still searching for the medicines that are best for them.

Additionally, some clinical trials are conducted to compare existing treatments and some are done to explore whether a medicine is appropriate for a different patient population, such as children or the elderly. Still others are conducted to find ways to make existing approved treatments more effective and easier to use with fewer side effects.

ECONOMIC IMPACT OF THE BIOPHARMACEUTICAL SECTOR IN MONTANA

Biopharmaceutical research companies have been and continue to be a good source of jobs, tax revenue and research spending in Montana.

A study by TEconomy Partners¹ found that in 2017, the industry supported more than 2,800 jobs throughout Montana. Wages and benefits for employees whose jobs were supported by the biopharmaceutical sector resulted in \$31 million in state and federal taxes paid.

Biopharmaceutical research companies supported the generation of \$574 million in economic activity in the state, including the direct economic output of the sector itself, the output of the sector's vendors and suppliers and the output generated by the buying power of its workforce.

Company employees in Montana include life science researchers, management executives, office and administrative support workers, production workers, engineers, architects, computer and math experts, and sales representatives. Biopharmaceutical companies also supported the jobs of their vendors and suppliers, including construction and IT firms. And the employees of biopharmaceutical companies help to support local restaurants, day care centers and other community businesses.

ECONOMIC IMPACT OF CLINICAL TRIALS IN MONTANA

A separate study by TEconomy Partners² found that in 2017 alone, there were 133 active industry-sponsored clinical trials in Montana, with an estimated enrollment of 3,242 Montana residents. Infectious diseases/virology was the largest clinical trial disease area by total estimated enrollment in the state.

The investment at clinical trial sites was more than \$153 million and the estimated total economic impact was more than \$394 million.

¹ *The Economic Impact of the U.S. Biopharmaceutical Industry: 2017 National and State Estimates*, TEconomy Partners, <https://www.phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/D-F/Economic-Impact-US-Biopharmaceutical-Industry-December-2019.pdf>

² *Biopharmaceutical Industry-Sponsored Clinical Trials: Growing State Economies*, TEconomy Partners, http://phrma-docs.phrma.org/files/dmfile/TEconomy_PhRMA-Clinical-Trials-Impacts.pdf

“Clinical trials in Montana allow patients access to cutting edge research, treatment and quality care. In addition to the human impact, they also have a significant economic impact that benefits all Montanans. Biopharmaceutical research companies support more than 4,256 direct jobs across Montana, while generating millions in economic activity in the state. These numbers reflect the fact that the clinical research not only shapes the future of disease treatment, but also, employs Montanans and stimulates our economy.”

Sharon Peterson
Montana BioScience Alliance

Open Clinical Trials in Montana by Disease	
Disease	Number of Trials
Arthritis/Musculoskeletal Disorders	4
Autoimmune Diseases	4
Cancer	64
Cardiovascular Diseases	7
Diabetes	5
Gastrointestinal/Esophageal Disorders	2
Infectious Diseases	23
Mental Disorders	2
Respiratory Diseases	9
Skin Diseases	3
Other Diseases	7
Total	130

Source: www.clinicaltrials.gov. Search criteria: Montana, United States; Phase: early 1, 1, 2, 3; Industry only, first posted on or after 1/1/2007. Search performed 10/6/2021. Open clinical trials are recruiting, not yet recruiting or are expanded access available.

Patient Resources & Directory

WHAT IS THE CLINICAL TRIAL EXPERIENCE?

Clinical trials are voluntary research studies conducted in people and designed to answer specific questions about the safety and effectiveness of drugs, vaccines, other therapies, or new ways of using existing treatments. Clinical trials can generate data to support FDA approval of a new medicine or a new indication for an existing medication. They may also grant participants early access to new medicines. By volunteering for a clinical trial, patients take an active role in their health care by helping researchers test new treatments. In Montana, 1,253 clinical trials since 2004 have targeted diseases and conditions like asthma, arthritis, cancer, diabetes and cardiovascular disease.

PHASES OF CLINICAL TRIALS

There are typically three phases of clinical testing used to evaluate potential new medicines:

PHASE I—Researchers test the medicine in a small group of people, usually between 20 and 100 healthy adult volunteers, to evaluate its initial safety and tolerability profile, determine a safe dosage range and identify potential side effects.

PHASE II—The medicine is given to volunteer patients, usually between 100 and 500 people, to study its efficacy, identify an optimal dose and to further evaluate its short-term safety.

PHASE III—The medicine is provided to a larger, more diverse patient population, often involving between 1,000 and 5,000 patients (but sometimes many more thousands), to generate statistically significant evidence to confirm its safety and effectiveness. They are the longest studies and usually take place in multiple sites around the world.

LEARNING ABOUT AND ACCESSING CLINICAL TRIALS

Patients can learn about clinical trials in several ways. Health care providers may be aware of clinical trials being conducted at hospitals, universities, and other leading health care facilities, and these institutions can be valuable sources of information for patients looking to participate.

For more information about clinical trials in Montana and how to participate in a clinical trial, visit www.centerwatch.com, or www.clinicaltrials.gov.

WHAT TO EXPECT

Since clinical trials are often conducted in a doctor's office, patients may need to devote more time to physician visits and physical examinations. They may also have additional responsibilities, like keeping a daily log of their health. Generally, prospective participants will receive information about the potential risks and benefits of participating in the trial and must sign an informed consent document saying, among other things, they understand that the clinical trial is research, and that they can leave the trial at any time. Patients can volunteer to participate, leading to a pre-screening interview. If they fit the criteria and requirements of the test, they may be enrolled.

PATIENT EXPENSES

As part of the informed consent process, clinical trial sponsors must disclose any additional costs to the subject that may result from participating in the research. During pre-screening discussions with the clinical trial investigator, the patient can also ask about associated costs to participate in the trial. Clinical trial sponsors usually pay for all research-related expenses and additional testing or physician visits required by the trial. Patients or their health insurance plan may be asked to pay for any routine treatments for their disease. However, it is important for the patient to know whether their health plans will pay for clinical trial participation or whether there will be out-of-pocket costs at the patient's expense.

Patients should learn whether they or their health insurance plan will be assessed any fees, and they should determine if their insurance will cover the expense of routine examinations. Patients who live a distance from the trial site should inquire whether the clinic has a policy for covering travel costs and living expenses. The National Cancer Institute, for example, makes patients cover for their own travel costs for the initial screening visits. Once a patient is enrolled in the trial, the Institute pays for transportation costs for all subsequent trial-related visits. These patients may also receive a small per diem for food and lodging.

EXPANDED ACCESS

For patients with a serious or life-threatening disease who are ineligible or unable to participate in a clinical trial, use of an unapproved investigational medicine through an expanded access program may be an option. Expanded access is the use of an unapproved investigational medicine outside of a clinical trial to treat a patient with a serious or immediately life-threatening disease or condition, when there are no other comparable or satisfactory alternative treatment options. Expanded access programs are part of many biopharmaceutical companies' commitment to patients.

*For more information about **the drug development and approval process in the United States**, see page 13.*

LOCAL PATIENT ADVOCACY GROUPS

Patient advocacy groups in Montana serve as an exceptional resource for patients, offering opportunities to connect and learn more about their condition and what treatment options are available locally. These groups also provide an important voice on behalf of patients to protect access to medicines and treatments.

The following are just a few major groups that work on behalf of patients in Montana and may provide more information to patients with further questions.

Alzheimer's Association

MONTANA CHAPTER
3010 11th Avenue, N
Billings, MT 59101
(406) 252-3053, (800) 272-3900

American Cancer Society

MONTANA CHAPTER
P.O. Box 20893
Billings, MT 59104
(800) 227-2345

American Diabetes Association

SERVING THE ROCKY MOUNTAIN AREA
P.O. Box 7023
Merrifield, VA 22116-7023
(720) 855-1102
ADARockyMtn@diabetes.org

American Heart Association

MONTANA CHAPTER
P.O. Box 8951
Missoula, MT 59807
(406) 273-8023

American Liver Foundation

MONTANA STATE RESOURCE CENTER
(800) 465-4837
info@liverfoundation.org

American Lung Association

SERVING MONTANA
822 John Street
Seattle, WA 98109
(206) 441-5100, (800) 586-4872

Arthritis Foundation

NATIONAL OFFICE
1355 Peachtree Street, NE,
Suite 600
Atlanta, GA 30309
(800) 283-7800

Epilepsy Foundation of Montana

3540 Crain Highway, Suite 675
Bowie, MD 20716
(800) 332-1000
montana@efa.org

NAMI Montana

NATIONAL ALLIANCE ON MENTAL ILLNESS
1331 Helena Avenue
Helena, MT 59601
(406) 443-7871
colleen@namimt.org

National Kidney Foundation

*SERVING COLORADO, NEW MEXICO,
MONTANA AND WYOMING*
1391 Speer Blvd., Suite 250
Boulder, CO 80204
(800) 596-7943
nkfconm@kidney.org

OTHER PATIENT RESOURCES

MEDICINE ASSISTANCE TOOL (MAT): The Medicine Assistance Tool is a PhRMA-sponsored search engine designed to help patients, caregivers and health care providers learn more about the resources available through the various biopharmaceutical industry programs. MAT is available to those who need financial support due to their lack of insurance or inadequate prescription medicine coverage. MAT is not its own patient assistance program, but rather, a search engine for many of the support programs and resources that the biopharmaceutical industry has offered for decades. The online process takes about 15 minutes, and patients can find out instantly if they are eligible for assistance. Patients can visit www.mat.org for more information.

HEALTHCARE READY: Healthcare Ready is a tool activated to help keep emergency responders informed on the status of the biopharmaceutical supply chain in the event of a natural disaster or emergency. Healthcare Ready's Rx Open tool has been deployed in 11 states and the District of Columbia and helps victims and evacuees who needed to fill or re-fill their prescriptions find open pharmacies. Healthcare Ready also helps emergency responders with critical information on the challenges facing supply chain partners relating to electricity, fuel and transportation issues. Patients can visit www.healthcareready.org for more information.

Clinical Trial Policy Resources

THE BIOPHARMACEUTICAL SECTOR'S ROLE IN THE ECONOMY

America's biopharmaceutical research companies serve as the foundation for one of the country's most dynamic innovation and business ecosystems. The biopharmaceutical industry is among the most research and development (R&D) intensive industries in the United States. In fact, the sector accounts for the single largest share of all U.S. business R&D, accounting for 17.7% of all R&D spending by U.S. businesses.¹ The industry and its large-scale research and manufacturing supply chain support high-quality jobs across the U.S. economy.

Biopharmaceutical companies invest on average six times more in R&D as a percentage of sales than all other manufacturing industries.

The biopharmaceutical industry supported more than 4 million jobs across the U.S. economy in 2017, according to a study by TEconomy Partners.¹

Since 2000, biopharmaceutical companies that are members of the Pharmaceutical Research and Manufacturers of America have invested more than \$1 trillion in the search for new treatments and cures, including an estimated \$91.1 billion in 2020 alone.

For more information on the economic impact of the biopharmaceutical industry in Montana, see page 2.

ECONOMIC IMPACT OF THE BIOPHARMACEUTICAL SECTOR IN MONTANA

Biopharmaceutical research companies have been and continue to be a source of quality jobs, tax revenue and research spending in Montana. A TEconomy Partners study¹ found that the biopharmaceutical sector:

- Supported more than 2,800 jobs throughout Montana in 2017.
- Supported the generation of \$574 million in economic activity in the state.
- Resulted in \$31 million in federal and state taxes through jobs supported by the biopharmaceutical sector.

¹ *The Economic Impact of the U.S. Biopharmaceutical Industry: 2017 National and State Estimates, TEconomy Partners, https://www.phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org_PDF/D-F/Economic-Impact-US-Biopharmaceutical-Industry-December-2019.pdf*

PUBLIC-PRIVATE PARTNERSHIPS AND LOCAL COLLABORATION

The following are just a few of the prominent institutions that biopharmaceutical research companies are collaborating with on clinical trials for new medicines:

Advanced Neurology Specialists, Great Falls

Benefis Hospital, Great Falls

Billings Clinic, Billings

Boeson Research, Butte, Great Falls, Helena, Kalispell, Missoula

Bozeman Deaconess Hospital, Bozeman

Community Hospital of Anaconda, Anaconda

Community Medical Center, Missoula

Community Physicians Group—Maternal Fetal Medicine, Missoula

Frontier Cancer Center and Blood Institute, Billings

Frontier Oncology, Billings

Glacier View Research Institute, Kalispell

Great Falls Clinic, Great Falls

Kalispell Regional Medical Center, Kalispell

Marcus Daly Memorial Hospital, Hamilton

Mercury Street Medical Group, Butte

Montana Medical Research, Missoula

Providence St. Patrick Hospital, Missoula

Sletten Cancer Institute, Great Falls

St. James Community Hospital, Butte

St. Luke Community Healthcare Hospital, Ronan

St. Patrick Hospital—Community Hospital, Missoula

St. Peter's Community Hospital, Helena

St. Vincent Frontier Cancer Center, Billings

The Birth Center, Missoula

UNIVERSITIES PLAY A KEY ROLE IN RESEARCH

Collaborations between the biopharmaceutical research industry and universities play an important role in the development of new medicines. In the United States, there are more than 8,800 open clinical trials¹ being sponsored by the biopharmaceutical industry, universities, individuals and organizations combined. These trials represent studies being funded by industry, research collaboration studies and research other groups are undertaking on their own.

Montana State University is a member of the WWAMI cooperative medical school program with the University of Washington School of Medicine and the states of Washington, Wyoming, Alaska and Idaho (the first letter of each state is where WWAMI gets its name). It gives students residing in Montana and other Northwestern states access to high-quality, cost-effective medical education by decentralizing the educational process and sharing existing facilities and personnel in universities and communities in the WWAMI states. Each year 30 qualified Montana residents are admitted to the WWAMI program.

THE STATE OF DISEASE IN MONTANA

More than 1 million people live in Montana¹, and many are dealing with disease and disability from asthma to cancer and from diabetes to heart disease.

Selected Disease Statistics in Montana	
Disease	Health Statistic
Alzheimer's Deaths 2020 ²	342
Asthma Prevalence—Adults 2020 ²	10%
Asthma Prevalence—Children 2020 ²	7%
Cancer New Cases 2021 ³	6,930
Cancer Deaths 2021 ³	2,150
Chronic Lower Respiratory Disease Deaths 2020 ²	653
COVID-19 Infections Deaths, 2020 ²	1,104
Diabetes Prevalence—Adults 2019 ²	64,000
Diabetes Deaths 2020 ²	337
Heart Disease Deaths 2020 ²	2,365
HIV—Number Living with a Diagnosis 2018 ⁴	644
Influenza/Pneumonia Deaths 2020 ²	119
Kidney Disease (Nephritis) Deaths 2020 ²	156
Liver Disease Deaths 2020 ²	231
Mental Illness—Adults 2018–2019 ⁴	171,000
Stroke Deaths 2020 ²	441

Source: 1. U.S. Census Bureau 2. Montana Department of Public Health and Human Services 3. American Cancer Society 4. Kaiser Family Foundation, State Health Facts

MONTANA CLINICAL TRIALS AND SPECIAL POPULATIONS: CHILDREN, OLDER AMERICANS AND WOMEN

- Children under the age of 18 make up 21.4%¹ of the population in Montana. Pediatric clinical trials are being conducted in the state for allergic rhinitis, asthma, atopic dermatitis, leukemia, lymphoma, meningococcal infections, respiratory syncytial virus infections and solid tumors, among others.²
- Montanans aged 65 and older account for 19.3%¹ of the states' population. In Montana, clinical trials are recruiting older people to study potential treatments for diseases such as asthma, coronary heart disease, colorectal cancer, chronic obstructive pulmonary disease,

heart failure, chronic lymphocytic leukemia, migraine, prostate cancer, type 2 diabetes and rheumatoid arthritis, among others.²

- Women and girls make up 49.7%¹ of the population in Montana. Clinical trials are recruiting women for studies on medicines for triple negative breast cancer, endometrial cancer, ovarian cancer, respiratory syncytial virus infections and urinary tract infections, among others.

¹ U.S. Census Bureau, ² www.clinicaltrials.gov

Open Clinical Trials in Montana for Special Populations

Population	Number of Trials
Children (birth–17)	19
Seniors (65 and older)	121
Women (only)	8

Source: www.clinicaltrials.gov. Search criteria: Montana, United States; Phase: early 1, 1, 2, 3; Industry only, first received on or after 1/1/2007. Search performed 10/6/2021. Open clinical trials are recruiting, not yet recruiting, or expanded access available.

SCIENCE AND CLINICAL TRIALS

Some of the medicines in clinical testing in Montana feature cutting-edge medical technologies. For example:

- A potential treatment for non-small cell lung cancer and melanoma, among others, is designed to stimulate cancer killing cells in the body by targeting CD122 on the surface of the immune cells. This experimental immune-therapy is being studied in combination with an approved immune checkpoint inhibitor which works by unleashing the body's own powerful immune system to target and kill cancer cells. The treatment works by increasing the number of tumor-infiltrating lymphocytes (TILs) which generate an immune response leading to increased therapeutic activity of the checkpoint inhibitor to attack cancer cells while leaving normal cells alone. Clinical trials are underway at the **Frontier Cancer Center and Blood Institute** in **Billings**.
- A synthetic analogue of human parathyroid hormone-related protein (hPTHrP) is in development for the treatment of postmenopausal osteoporosis. The protein hPTHrP is thought to be a critical cytokine for promoting new bone formation. The medicine, a transdermal patch, is designed to build bone rapidly without inducing hypercalcemia (too much calcium in the blood) or significant bone resorption. A clinical trial is being conducted at **Montana Medical Research** in **Missoula**.
- An antibacterial targeting *Clostridioides difficile* for the treatment of *Clostridium difficile* (C. difficile) infections is an orally administered, narrow-spectrum antibacterial to specifically target C. difficile at the infection site, without causing damage to the healthy gut flora, to reduce the risk of recurrent infection. Clinical trials are being conducted by the **Mercury Street Medical Group** in **Butte**.
- A novel bacterial topoisomerase II inhibitor is being developed to treat uncomplicated urinary tract infections. The drug has a dual mechanism of action, and works by selectively inhibiting two bacterial enzymes — DNA gyrase and topoisomerase IV — that play a role in bacterial replication. The drug may have activity against most target pathogens resistant to established antibiotics. A clinical trial was conducted in **Butte**.
- A potential first-in-class oral medicine in development provides a new way to address type 1 by acting on two different targets in the body. It is a dual inhibitor of both sodium-glucose co-transporter types 1 and 2 (SGLT1 and SGLT2), which are molecules that also help move glucose in and out of the body's cells independent of insulin. This movement is important for the absorption of glucose in the body both by the intestine (glucose absorption from food) and by the kidney (which determines how much glucose leaves the body via urine). A clinical trial was conducted in **Great Falls**.

Conclusion

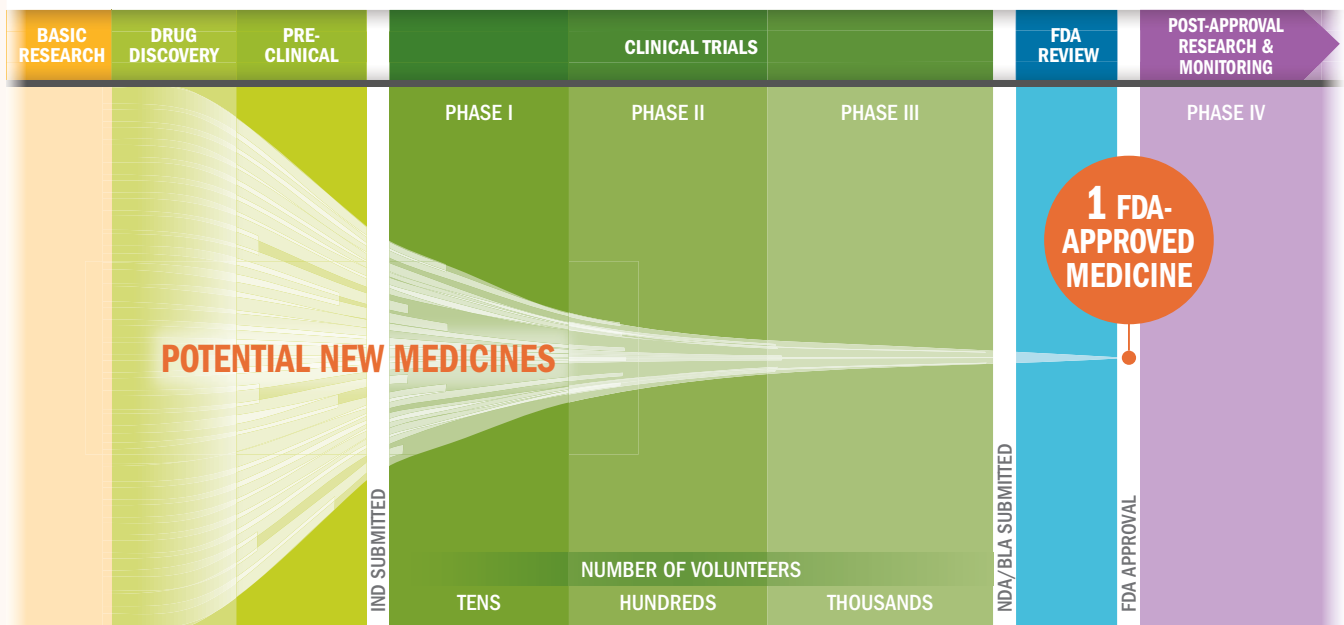
The Montana bioscience industry supports more than 2,800 jobs throughout Montana with wages and benefits supported by the sector, resulting in \$31 million in state and federal taxes paid. The industry is also driving innovation and additional economic activity in the state. Biopharmaceutical research companies supported the generation of \$574 million in direct and indirect economic activity in Montana.

Montanans are also positively impacted by the presence of a strong biopharmaceutical sector and clinical trials in the state. Innovative treatments developed today are helping to expand the frontiers of science and could lead to more and better treatments for patients in the future.

In Montana, this innovation is the result of a successful collaboration between biopharmaceutical companies and local research institutions. And the sector's growth and strength in Montana are driving our economy and communities forward.

THE BIOPHARMACEUTICAL RESEARCH AND DEVELOPMENT PROCESS

From drug discovery through FDA approval, developing a new medicine takes at least 10 years on average and costs an average of \$2.6 billion.* Less than 12% of the candidate medicines that make it into Phase I clinical trials will be approved by the FDA.



Key: IND: Investigational New Drug Application, NDA: New Drug Application, BLA: Biologics License Application

* The average R&D cost required to bring a new, FDA-approved medicine to patients is estimated to be \$2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.

Source: PhRMA adaptation based on Tufts Center for the Study of Drug Development (CSDD) Briefing: "Cost of Developing a New Drug," Nov. 2014. Tufts CSDD & School of Medicine and US FDA Infographic, "Drug Approval Process," <http://www.fda.gov/downloads/Drugs/ResourcesForYou/Consumers/UCM284393.pdf> (accessed Jan. 20, 2015).



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