

Developing Cures, Creating Jobs

**A Partnership Between Biopharmaceutical
Companies And State Research Institutions**

**PHARMACEUTICAL CLINICAL TRIALS IN
WEST VIRGINIA**



P/RMA

Executive Summary

This report shows that biopharmaceutical research companies continue to be vitally important to the economy and patient health in West Virginia, despite the recession.

At a time when the state still faces significant economic challenges, these companies are conducting or have conducted nearly 500 clinical trials of new medicines in collaboration with the state's university medical schools, hospitals and clinical research centers. Of the nearly 500 clinical trials, more than 200 target or have targeted the nation's six most debilitating chronic diseases—asthma, cancer, diabetes, heart disease, mental illnesses and stroke.

Institutions involved in clinical tests of treatments include the medical schools at West Virginia University in Morgantown and Marshall University in Huntington; such research centers as Marshall's Edwards Comprehensive Cancer Center, CAMC Health Education & Research Institute in Charleston and the Mary Robb Randolph Cancer Center at West Virginia University; and hospitals like Camden-Clark Memorial Hospital in Parkersburg, St. Mary's Medical Center in Huntington, Wheeling Hospital and the Charleston Area Medical Center.

The biopharmaceutical drug trials provide steady revenue for research facilities all over the state and what's more, nearly 40 of the clinical tests are in the initial stages of recruiting patients, which could be important to those still seeking effective treatments. In addition,

these tests—which are being conducted all over the Mountain State—are helping to advance science and patient care since many involve cutting-edge, new-generation biotechnology medications.

Earlier reports have shown the nation's biopharmaceutical companies are also an important source of jobs, research spending and tax revenue. A study by Archstone Consulting, for example, found that the industry supported nearly 12,600 West Virginia jobs in 2008 and was responsible for providing nearly \$3.1 billion in products and services.

Biopharmaceutical company employees in the state include life sciences researchers, management executives, office and administrative support workers, production workers, engineers, architects, computer and math experts and sales representatives.

In 2008, these workers were paid an estimated \$124.1 million, leading to more than \$4 million in state taxes and more than \$25 million in federal taxation. Biopharmaceutical research firms that year also invested \$144.8 million in research and development in the state. This new clinical trial report shows three years later, the trend continues: American biopharmaceutical research companies remain vitally important to the residents and economy of West Virginia.

The Need for New Chronic Disease Medicines

Chronic diseases pose the greatest threats to our nation's health and our ability to treat and prevent medical conditions. According to the Centers for Disease Control and Prevention, today, in the United States:

- Patients with chronic diseases account for 75 cents of every dollar spent on health care.
- Chronic diseases are the leading cause of death and disability.
- Chronic diseases are a leading driver of rising health care costs with expenses totaling billions of dollars every year.

With the stakes so high, America's biopharmaceutical research companies are developing new medicines to help treat those conditions that are taking an unprecedented toll on American lives.

Many of these medicines are being tested today in clinical trials throughout West Virginia.

At a time when tens of thousands of state residents are suffering from one or more chronic diseases, America's biopharmaceutical research companies are sponsoring or have sponsored more than 200 clinical trials of potential new medicines in the Mountain State alone for asthma, cancer, heart disease, stroke, diabetes and mental illnesses. Of the more than 200 trials, nearly 40 are either not yet



recruiting or are just now seeking West Virginia patients, thus giving those still searching for effective treatments potential new options.

Many of the state's clinical tests involve collaborations with such respected local institutions as **West Virginia University**, **Marshall University**, the **Edwards Comprehensive Cancer Center**, the **CAMC Health Education & Research Institute**, and the **Mary Robb Randolph Cancer Center**.

Clinical Trials in West Virginia

Table 1. Clinical Trials in West Virginia for Selected Chronic Diseases

Chronic Disease	All Clinical Trials	Clinical Trials Still Recruiting
Asthma	6	0
Cancer	114	28
Diabetes	30	3
Heart Disease	26	3
Mental Illness	23	1
Stroke	11	2

Source: www.clinicaltrials.gov

Note: Search criteria = West Virginia, Phase I, II, III; industry only.
Search performed 9/29/2011

Clinical tests of new medicines are a vitally important part of the drug development and approval process—they account for 45 to 75 percent of the \$1.2 billion average cost of developing a new drug and are conducted to determine the safety and effectiveness of that treatment in patients.

Some trials are also conducted to compare existing treatments and some are done to learn if a drug is appropriate for a different patient population, such as children. Still others are conducted to find ways to make existing ap-

proved drugs more effective and easier to use with fewer side effects.

It's essential that trials be conducted properly so that clinicians and drug reviewers can develop accurate assessments of the efficacy and safety of medicines when used by patients. The Food and Drug Administration (FDA) is a vigilant regulatory agency and its pharmaceutical review officers are effective in detecting flawed information.

Questionable or confusing data can lead to lengthy delays in product approval or outright FDA rejection of a new drug.

Biopharmaceutical research companies are looking for the best physicians and research institutions to meticulously help design and conduct their clinical trials to determine whether a medicine is safe and effective. Side effects must be painstakingly documented and a determination made as to whether they occur too often and are dangerous.

Clinical tests involve three phases and thousands of volunteer patients and are often conducted at multiple sites around the country. In West Virginia, biopharmaceutical companies have the luxury of having trials conducted at the states' well-respected university medical schools, comprehensive cancer centers and clinical trial research centers. According to *U.S. News and World Report*, West Virginia University Medical School ranked 90th among

this year's top 100 research-oriented medical schools in the United States.

Asthma is a debilitating condition for more than 23 million Americans, including 7 million children under the age of 18. The toll is also severe in West Virginia—in 2009, about 126,000 adults had asthma, according to the West Virginia Department of Health & Human Resources. While there are no currently active clinical trials for asthma in West Virginia, previous studies have been conducted in Charleston and Morgantown.

Cancer, the second leading cause of death in the United States, now afflicts nearly 12 million Americans, according to the National Cancer Institute. In West Virginia, more than 11,000 new cancer cases will be diagnosed this year and 4,680 victims in the state will die, according to the American Cancer Society.

Currently, nearly 30 clinical trials of new cancer medicines are recruiting patients in West Virginia.

Biopharmaceutical companies are collaborating on the tests with such prominent institutions as the **Davis Memorial Hospital's Cancer Care Center** in Elkins, the **Raleigh Regional Cancer Center** in Beckley, the **Schiffler Cancer Center of Wheeling Hospital** in Wheeling, and the **CAMC Health Education and Research Institute** in Charleston.

In Huntington, cancer research is taking place at the **Edwards Comprehensive Cancer Care** and **St. Mary's Medical Center**, both affiliated with the **Marshall University School of Medicine**.

In Morgantown, research is taking place at several sites affiliated with the **Robert C. Byrd Health Sciences Center at West Virginia University**, including the **Mary Babb Randolph Cancer Center**, and **West Virginia University Hospital**.

Diabetes affects more than 20 million Americans—about 8 percent of the U.S. population—and nearly

one-third are unaware they have the disease. In 2009, about 177,000 adults in West Virginia had diabetes, according to the West Virginia Department of Health & Human Resources.

Currently, three diabetes clinical tests are seeking patients in West Virginia. The trials are being conducted in Clarksburg, Huntington, and Morgantown.

Heart disease and stroke are the first and fourth leading causes of death in the United States and the first and third in West Virginia. According to the American Heart Association, more than 82 million Americans are affected by these diseases. In West Virginia, in 2008, more than 5,300 residents died from some form of heart disease and 1,047 died from a stroke.

Currently, three heart disease and two stroke clinical tests are seeking patients in West Virginia. The trials are being conducted in Clarksburg, Huntington, Morgantown and at the **Charleston Area Medical Center** in Charleston.

Mental illness affects nearly 60 million Americans suffering from some form of the disease—from anxiety to depression to schizophrenia to eating disorders. In West Virginia, about 81,000 adults live with serious mental illness and about 18,000 children live with serious mental health conditions, according to the National Alliance on Mental Illness.

Currently, one clinical trial for smoking cessation (smoking is classified under mental illness) is recruiting in Martinsburg. In 2009, 366,000 state residents were active smokers, according to the West Virginia Department of Health & Human Resources.

Physicians and patients can find out about clinical trials being conducted all over the state in collaboration with local institutions by accessing www.clinicaltrials.gov, a database sponsored by the National Institutes of Health. Information on medicines in development is also available on www.phrma.org, the website of the Pharmaceutical Research and Manufacturers of America (PhRMA).

New Generation Medicines in Development

Many of the medicines being tested in West Virginia are cutting-edge biotechnology drugs.

America's biopharmaceutical research companies are using biotechnology to develop hundreds of medicines and vaccines today. And West Virginia is one of the states where new-generation research and development work is being done.

Through biotechnology, new ways are being developed to not only more effectively treat disease, but also to predict, preempt and prevent it.

Biotechnology medicines are developed through biological processes using living cells or organisms, rather than traditional chemical synthesis, the mainstay of pharmaceutical development for decades.

Such novel treatments use a variety of new approaches to treat disease. For example, a monoclonal antibody is a laboratory-made version of the naturally occurring immune system protein that binds to and neutralizes foreign invaders. Interferons are proteins that interfere with the ability of a cell to reproduce.

Antisense drugs, meanwhile, are medicines that interfere with the communication process that tells a cell to pro-

duce an unwanted protein. In addition, nanotechnology is being used in biotechnology research to provide drug-delivery systems, new treatments and diagnostics.

Many of the medicines in clinical testing at West Virginia medical schools and research centers feature these technologies. For example:

- An antisense medicine for the treatment of cancer.
- A monoclonal antibody in the pipeline targets lupus and various types of cancer.
- A therapeutic vaccine, designed to jump-start the immune system to fight disease, is in development for lung cancer and melanoma.

These are only a portion of the examples of new ways the nation's biopharmaceutical companies and West Virginia research institutions are working together to attack disease. The biotechnology medicines and vaccines in development promise to push the frontiers of science and potentially bring more and better treatments to patients.

Conclusion

Biopharmaceutical companies' close collaboration with clinicians and research institutions in West Virginia benefits patients, the state's economy and the advancement of science and patient care. Clinical trial business is good business for the state's medical schools and clinical research centers and the medicines being tested are often cutting-edge cell and protein treatments with the potential to be safer and more effective than older chemical compound drugs.

What's more, West Virginians contemplating participation in clinical trials have a wide range of choices—nearly 40 tests of new medicines for the six most debilitating chronic diseases in America are underway in communities large and small all over the state and they need patient volunteers. Additional clinical trials recruiting patients target other diseases such as rheumatoid arthritis, chronic obstructive pulmonary disease, traumatic brain injury, cystic fibrosis, influenza, lupus, and psoriasis.

The Drug Discovery, Development and Approval Process

It takes 10-15 years on average for an experimental drug to travel from the lab to U.S. patients. Only five in 5,000 compounds that enter preclinical testing make it to human testing. One of these five tested in people is approved.

Clinical Trials							
Discovery/ Preclinical Testing		Phase I	Phase II	Phase III	FDA	Phase IV	
Years	6.5	1.5	2	3.5	1.5		
Test Population	Laboratory and animal studies	20 to 100 healthy volunteers	100 to 500 patient volunteers	1,000 to 5,000 patient volunteers	Review process/ approval	Additional post-marketing testing required by FDA	
Purpose	Assess safety, biological activity and formulations	Determine safety and dosage	Evaluate effectiveness, look for side effects	Confirm effectiveness, monitor adverse reactions from long-term use			
Success Rate	5,000 compounds evaluated		5 enter trials		1 approved		

The Drug Development and Approval Process

The U.S. system of new drug approvals is perhaps the most rigorous in the world.

It takes 10-15 years, on average, for an experimental drug to travel from lab to U.S. patients, according to the Tufts Center for the Study of Drug Development, based on drugs approved from 1994 through 1998. Only five in 5,000 compounds that enter preclinical testing make it to human testing. And only one of those five is approved for sale.

On average, it costs a company \$1.2 billion, including the cost of failures, to get one new medicine from the laboratory to U.S. patients, according to a 2007 study by the Tufts Center for the Study of Drug Development.

Once a new compound has been identified in the laboratory, medicines are developed as follows:

Preclinical Testing. A pharmaceutical company conducts laboratory and animal studies to show biological activity of the compound against the targeted disease, and the compound is evaluated for safety.

Investigational New Drug Application (IND). After completing preclinical testing, a company files an IND with the U.S. Food and Drug

Administration (FDA) to begin to test the drug in people. The IND shows results of previous experiments; how, where and by whom the new studies will be conducted; the chemical structure of the compound; how it is thought to work in the body; any toxic effects found in the animal studies; and how the compound is manufactured. All clinical trials must be reviewed and approved by the Institutional Review Board (IRB) where the trials will be conducted. Progress reports on clinical trials must be submitted at least annually to FDA and the IRB.

Clinical Trials, Phase I. These tests usually involve about 20 to 100 normal, healthy volunteers. The tests study a drug's safety profile, including the safe dosage range. The studies also determine how a drug is absorbed, distributed, metabolized, and excreted as well as the duration of its action.

Clinical Trials, Phase II. In this phase, controlled trials of approximately 100 to 500 volunteer patients (people with the disease) assess a drug's effectiveness and determine the early side effect profile.

Clinical Trials, Phase III. This phase usually involves 1,000 to 5,000 patients in clinics and

hospitals. Physicians monitor patients closely to confirm efficacy and identify adverse events.

New Drug Application (NDA)/Biologic License Application (BLA). Following the completion of all three phases of clinical trials, a company analyzes all of the data and files an NDA or BLA with FDA if the data successfully demonstrate both safety and effectiveness. The applications contain all of the scientific information that the company has gathered. Applications typically run 100,000 pages or more. The average review time for the 21 new therapeutics approved by the FDA in 2010 was 14.8 months.

Approval. Once FDA approves an NDA or BLA, the new medicine becomes available for physicians to prescribe. A company must continue to submit periodic reports to FDA, including any cases of adverse reactions and appropriate quality-control records. For some medicines, FDA requires additional trials (Phase IV) to evaluate long-term effects.

Discovering and developing safe and effective new medicines is a long, difficult, and expensive process. Pharmaceutical companies invested an estimated \$67.4 billion in research and development in 2010.

The Good News – Many Clinical Trials are Still Recruiting

There are nearly 70 clinical trials recruiting in West Virginia. These trials target the top six chronic diseases and other debilitating diseases affecting Americans and West Virginians.

Location	Asthma	Cancer	Diabetes	Heart Disease	Mental Illness	Stroke	Other Diseases
Beckley	0	5	0	0	0	0	4
Charleston	0	1	0	0	0	1	3
Clarksburg	0	1	1	2	0	1	12
Huntington	0	4	1	1	0	0	2
Morgantown	0	15	2	1	0	0	10
Wheeling	0	6	0	0	0	0	0

Source: www.clinicaltrials.gov

Note: Search criteria = West Virginia, Phase I, II, industry only. Search performed 9/29/2011. See appendices for detailed information about these clinical trials. Disease columns will not add to totals in Appendix because some clinical trials are recruiting in more than one city.

Cancer – Leading Institutions Conducting Clinical Trials

CAMC Health Education & Research Institute,
Charleston

Camden-Clark Memorial Hospital, Parkersburg

Davis Memorial Cancer Center, Elkins

Edwards Comprehensive Cancer Center,
Marshall University, Huntington

Mary Babb Randolph Cancer Center,

West Virginia University, Morgantown

Raleigh Regional Cancer Center, Beckley
Schiffler Cancer Center, Wheeling Hospital,
Wheeling

St. Mary’s Medical Center, Huntington

West Virginia University Hospital, Morgantown

Wheeling Hospital, Wheeling

Stroke – Leading Institutions Conducting Clinical Trials

Charleston Area Medical Center, Charleston

Appendix

The clinical trials listed here involve tests that have not yet started recruiting patients or are just now seeking volunteers to participate. This information is potentially valuable to patients still seeking effective treatments for their chronic diseases. It provides a new therapeutic option to discuss with physicians.

Those interested in obtaining more information about certain trials can use the URL code listed for each test to log onto www.clinicaltrials.gov, the clinical tests database of the National Institutes of Health.

Cancer

(28 clinical trials recruiting)

Study 1:

Study Evaluating The Effects Of Neratinib After Adjuvant Trastuzumab In Women With Early Stage Breast Cancer

URL: <http://ClinicalTrials.gov/show/NCT00878709>

Study 2:

Phase III Lucanix™ Vaccine Therapy in Advanced Non-small Cell Lung Cancer (NSCLC) Following Front-line Chemotherapy

URL: <http://ClinicalTrials.gov/show/NCT00676507>

Study 3:

A Study in Ovarian, Non-Small Cell Lung, Prostate, Colorectal, Gastroesophageal Cancers, and Squamous Cell Carcinoma of the Head and Neck

URL: <http://ClinicalTrials.gov/show/NCT01059643>

Study 4:

Cancer Vaccine Study for Unresectable Stage III Non-small Cell Lung Cancer

URL: <http://ClinicalTrials.gov/show/NCT00409188>

Study 5:

Trial of Gemcitabine/Carboplatin With or Without Iniparib (SAR240550) (a PARP1 Inhibitor) in Subjects With Previously Untreated Stage IV Squamous Non-Small-Cell Lung Cancer (NSCLC)

URL: <http://ClinicalTrials.gov/show/NCT01082549>

Study 6:

Study of Imprime PGG® in Combination With Cetuximab in Subjects With Recurrent or Progressive KRAS Wild Type Colorectal Cancer

URL: <http://ClinicalTrials.gov/show/NCT01309126>

Study 7:

Randomized Study to Compare CyberKnife to Surgical Resection In Stage I Non-small Cell Lung Cancer

URL: <http://ClinicalTrials.gov/show/NCT00840749>

Study 8:

Study of IMC-18F1 or Ramucirumab DP in Combination With Capecitabine or Capecitabine on Previously Treated Breast Cancer Patients

URL: <http://ClinicalTrials.gov/show/NCT01234402>

Study 9:

A Trial of E7080 in 131I-Refractory Differentiated Thyroid Cancer

URL: <http://ClinicalTrials.gov/show/NCT01321554>

Study 10:

Study of Immunotherapy to Treat Advanced Prostate Cancer

URL: <http://ClinicalTrials.gov/show/NCT00861614>

Study 11:

A Study of Pemetrexed, Carboplatin and Bevacizumab in Patients With Nonsquamous Non-Small Cell Lung Cancer

URL: <http://ClinicalTrials.gov/show/NCT00762034>

Study 12:

Chemotherapy and Radiation in Treating Patients With Stage 3 Non-Small Cell Lung Cancer

URL: <http://ClinicalTrials.gov/show/NCT00686959>

Study 13:

Study of Patients With Advanced Non-Small Cell Lung Cancer

URL: <http://ClinicalTrials.gov/show/NCT00948675>

Study 14:

A Study in Head and Neck Cancer

URL: <http://ClinicalTrials.gov/show/NCT01081041>

Study 15:

A Clinical Study Using MEDI-551 in Adult Subjects With Relapsed or Refractory Advanced B-Cell Malignancies

URL: <http://ClinicalTrials.gov/show/NCT00983619>

Study 16:

A Clinical Trial Comparing the Combination of TC Plus Bevacizumab to TC Alone and to TAC for Women With Node-Positive or High-Risk Node-Negative, HER2-Negative Breast Cancer

URL: <http://ClinicalTrials.gov/show/NCT00887536>

Study 17:

Phase 2 Study of Efficacy and Safety of Apricoxib/ Placebo With Either Docetaxel or Pemetrexed in Non-Small Cell Lung Cancer Patients

URL: <http://ClinicalTrials.gov/show/NCT00771953>

Study 18:

Study to Evaluate the Efficacy and Safety of Three Different Doses of SCV 07 in Attenuating Oral Mucositis in Subjects With Head and Neck Cancer

URL: <http://ClinicalTrials.gov/show/NCT01247246>

Study 19:

Panobinostat or Placebo With Bortezomib and Dexamethasone in Patients With Relapsed Multiple Myeloma

URL: <http://ClinicalTrials.gov/show/NCT01023308>

Study 20:

Aplidin—Dexamethasone in Relapsed/Refractory Myeloma

URL: <http://ClinicalTrials.gov/show/NCT01102426>

Study 21:

Assessment of Efficacy and Safety of Perifosine, Bortezomib and Dexamethasone in Multiple Myeloma Patients

URL: <http://ClinicalTrials.gov/show/NCT01002248>

Study 22:

Phase III Study of Lenalidomide and Dexamethasone With or Without Elotuzumab to Treat Newly Diagnosed, Previously Untreated Multiple Myeloma

URL: <http://ClinicalTrials.gov/show/NCT01335399>

Study 23:

Study of Vosaroxin or Placebo in Combination With Cytarabine in Patients With First Relapsed or Refractory Acute Myeloid Leukemia (AML)

URL: <http://ClinicalTrials.gov/show/NCT01191801>

Study 24:

Efficacy and Safety of CDP6038 in Patients With Rheumatoid Arthritis With an Unsuccessful Response to Anti-Tumor Necrosis Factor (Anti-TNF) Therapy

URL: <http://ClinicalTrials.gov/show/NCT01242488>

Study 25:

Ofatumumab and Bendamustine Combination Therapy Compared With Bendamustine Monotherapy in Indolent B-cell Non-Hodgkin's Lymphoma (NHL) Unresponsive to Rituximab or a Rituximab-Containing Regimen

URL: <http://ClinicalTrials.gov/show/NCT01077518>

Study 26:

PK-directed Dose Adjustment of IV Busulfan Conditioning Regimen for Autologous Stem Cell Transplant in Lymphoma Patients

URL: <http://ClinicalTrials.gov/show/NCT00948090>

Study 27:

Trial of Bendamustine, Bortezomib, and Rituximab in Patients With Previously Untreated Low Grade Lymphoma

URL: <http://ClinicalTrials.gov/show/NCT01029730>

Study 28:

Allogeneic HCT Using Nonmyeloablative Host Conditioning With TLI & ATG vs SOC in AML

URL: <http://ClinicalTrials.gov/show/NCT00568633>

Diabetes

(3 clinical trials recruiting)

Study 1:

BI 10773 Cardiovascular Outcome Event Trial in Type 2 Diabetes Mellitus Patients

URL: <http://ClinicalTrials.gov/show/NCT01131676>

Study 2:

A Study With Alogliptin in Patients With a Recent Acute Coronary Syndrome and Type 2 Diabetes Mellitus

URL: <http://ClinicalTrials.gov/show/NCT01042769>

Study 3:

Evaluation of Cardiovascular Outcomes in Patients With Type 2 Diabetes After Acute Coronary Syndrome During Treatment With AVE0010 (Lixisenatide)

URL: <http://ClinicalTrials.gov/show/NCT01147250>

Heart Disease

(3 clinical trials recruiting)

Study 1:

A Study With Alogliptin in Patients With a Recent Acute Coronary Syndrome and Type 2 Diabetes Mellitus

URL: <http://ClinicalTrials.gov/show/NCT01042769>

Study 2:

Evaluation of Cardiovascular Outcomes in Patients With Type 2 Diabetes After Acute Coronary Syndrome During Treatment With AVE0010 (Lixisenatide)

URL: <http://ClinicalTrials.gov/show/NCT01147250>

Study 3:

Cardiovascular Safety of Febuxostat and Allopurinol in Patients With Gout and Cardiovascular Comorbidities

URL: <http://ClinicalTrials.gov/show/NCT01101035>

Mental Illness

(1 clinical trial recruiting)

Study 1:

Study to Evaluate the Safety and Efficacy of Dietary Supplement Anatabloc in Reducing Daily Smokers' Urge to Smoke

URL: <http://ClinicalTrials.gov/show/NCT01428310>

Stroke

(2 clinical trials recruiting)

Study 1:

RESPECT PFO Clinical Trial

URL: <http://ClinicalTrials.gov/show/NCT00465270>

Study 2:

Cardiovascular Safety of Febuxostat and Allopurinol in Patients With Gout and Cardiovascular Comorbidities

URL: <http://ClinicalTrials.gov/show/NCT01101035>



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