RESEARCH *in* YOUR BACKYARD

Developing Cures, Creating Jobs

Pharmaceutical clinical trials in

PENNSYLVANIA

PhRMA

RESEARCH • PROGRESS • HOPE

Executive

Summary

Clinical trials in PENNSYLVANIA

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

Since 2004, biopharmaceutical research companies are conducting or have conducted more than 7,800 clinical trials of new medicines in Pennsylvania in collaboration with clinical research centers and hospitals. These clinical trials have investigated or are investigating some of Pennsylvania's biggest health care challenges, including asthma, arthritis, cancer, diabetes, cardiovascular disease and Alzheimer's disease.

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

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

Clinical tests of new drugs are conducted in three phases and, on average, account for nearly seven of the more than 10 years it takes to bring a new drug 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.

All clinical trials must be reviewed and approved by an Institutional Review Board (IRB) in advance; an independent committee of physicians, statisticians, local community advocates and others to ensure a trial is ethically conducted and patient rights are protected.

Clinical Trials in Pennsylvania since 2004—Completed and Open	
All Clinical Trials	**Open Clinical Trials**
7,893	1,178

Source: www.clinicaltrials.gov. Search criteria: Pennsylvania, United States; Phase: early 1, 1, 2, 3; Industry only, first received on or after 1/1/2004. Search performed 7/26/2017. Open clinical trials are recruiting, not yet recruiting, or expanded access.

Executive Summary (cont.)

"The liver wellness community in Pennsylvania is optimistic about the outlook for cures and treatments for patients with liver diseases like hepatitis C, cirrhosis, liver cancer and fatty liver disease. The clinical trials and research conducted at the world-class medical institutions in the commonwealth give hope to patients living with liver disease that they will soon have a cure."

Suzanna Masartis
Executive Director
Community Liver Alliance
We Work for Health PA Co-Chair

CLINICAL TRIALS OFFER IMPORTANT THERAPEUTIC OPTIONS FOR PATIENTS

For patients, clinical trials offer the potential for another therapeutic option. Clinical tests may provide a new avenue of care for some chronic disease sufferers who are still searching for the medicines that are best for them.

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

ECONOMIC IMPACT OF THE BIOPHARMACEUTICAL SECTOR IN PENNSYLVANIA

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

A study by TEConomy Partners found that in 2014, the industry supported more than 254,000 jobs throughout Pennsylvania. Wages and benefits for employees whose jobs were supported by the biopharmaceutical sector resulted in more than $3 billion in federal taxation and $469.6 million in state taxes.

Biopharmaceutical research companies supported the generation of $68.9 billion 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 Pennsylvania 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 PENNSYLVANIA

A separate study by Battelle Technology Partnership Practice found that in 2013 alone, there were 1,972 active industry-sponsored, site-based clinical trials in Pennsylvania, with an estimated enrollment of 47,538 Pennsylvania residents. Oncology had the leading clinical trial enrollment in the state.

The investment of these site-based clinical trials was more than $400 million and the estimated total economic impact was more than $1 billion.

Open Clinical Trials in Pennsylvania by Disease

Disease	Number of Trials
Allergy	2
Alzheimer's Disease	20
Arthritis/Musculoskeletal Disorders	51
Autoimmune Diseases	48
Bladder Disorders	5
Blood Disorders	18
Cancer	546
Cardiovascular Diseases	74
Diabetes	21
Eye Disorders	33
Gastrointestinal/Esophageal Diseases	39
Genetic Disorders	40
Growth Disorders	3
Infectious Diseases	44
Kidney Diseases	25
Liver Diseases	31
Mental Disorders	34
Neurological Disorders	50
Respiratory Diseases	26
Skin Diseases	19
Transplantation-Related	8
Other Diseases	41
Total	**1,178**

Source: www.clinicaltrials.gov. Search criteria: Pennsylvania, United States; Phase: early 1, 1, 2, 3; Industry only, first received on or after 1/1/2004. Search performed 7/26/2017. Open clinical trials are recruiting, not yet recruiting, or are expanded access.

"Over the past decade, the clinical trial landscape has changed significantly, but continues to grow and evolve for the benefit of patients and Pennsylvania's economy. With a diverse life science industry, including global pharmaceutical companies, contract research organizations, academic research institutions and premier healthcare systems, the Commonwealth has an ecosystem well-positioned to take advantage of those changes.

As the updated Research in Your Backyard study clearly points out, this innovative landscape has led to more than 7,800 clinical trials in Pennsylvania since 2004, and over 1,170 trials open this year. These trials are critical in ensuring that the research occurring at our institutions today, become the groundbreaking treatments and cures of tomorrow."

Christopher P. Molineaux,
President and CEO
Life Sciences Pennsylvania
We Work for Health PA Co-Chair

Patient Resources & Directory

WHAT IS THE CLINICAL TRIAL EXPERIENCE?

Clinical trials are research studies that generate data to support FDA approval of a new medicine or a new indication for an existing medication. They also grant participants early access to new medicines, which are being developed to help combat chronic and serious diseases. By volunteering for a clinical trial, patients take an active role in their health care by helping researchers test new treatments. In Pennsylvania, 7,893 clinical trials since 2004 have targeted diseases and conditions like asthma, arthritis, cancer, diabetes, cardiovascular disease and Alzheimer's disease.

PHASES OF CLINICAL TRIALS

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

PHASE I—Researchers test the drug 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 drug 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 drug 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 are 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. Patients can also use hospital and university websites to find the trials being conducted in their area. For instance, clinical trials being conducted at the University of Pennsylvania can be found at *clinicalresearch.itmat.upenn.edu/participant/*, for clinical trials at the University of Pittsburgh, go to *pittplusme.org* and for trials at Penn State University, go to *studyfinder.psu.edu/*. For cancer trials at Thomas Jefferson University, go to *www.jefferson.edu/cancer-center/research/clinical-trials.html*.

More information about clinical trials in Pennsylvania and how to volunteer for one can be found at *www.centerwatch.com*, a PhRMA-recommended website.

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. All prospective participants must sign an informed consent document saying they understand that the clinical trial is research, and that they can leave the trial at any time. After consulting with their health care providers, patients can volunteer to participate, leading to a pre-screening interview. If they fit the criteria and requirements of the test, they can be enrolled.

PATIENT EXPENSES

Patients should ask during pre-screening interviews what it will cost them to participate in a clinical trial. Clinical trial sponsors usually pay for all research-related expenses and additional testing or physician visits required by the trial. Patients or their insurance companies may be asked to pay for any routine treatments of their disease. And it's important to know some health plans do not pay for clinical trials.

Patients should make it a point to learn if they or their insurance company will be assessed any fees and should determine if their insurance company will cover the expense of routine examinations. Patients who live a distance from the trial site should learn the clinic's policy for covering travel costs and living expenses.

The National Cancer Institute, for example, makes patients responsible for their own travel costs for the initial screening visits. Once a patient is enrolled, the Institute will pay for transportation costs for all subsequent trial-related visits. These patients will receive a small per diem for food and lodging.

EXPANDED ACCESS

Successful completion of the clinical trials is required to demonstrate to the FDA that an investigational drug is safe and effective, so that it can be approved and made available to a broad patient population. Clinical trials are the primary route by which patients can participate in the drug development process, receive access to unapproved investigational drugs and contribute to the collection of safety and efficacy data necessary for FDA approval.

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 drug through an expanded access program may be an option. The current FDA process for a patient to gain access to an investigational drug through expanded access was established in 2009 in close consultation with patients, physicians and the biopharmaceutical industry. 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 Pennsylvania provide an exceptional resource for patients to connect and learn more about their condition and what treatment options are available in the state. These groups also provide an important voice on behalf of patients to protect their access to medicine and treatment.

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

Alzheimer's Association
Delaware Valley Chapter
399 Market Street, Suite 102
Philadelphia, PA 19106
(800) 272-3900

Alzheimer's Association
Lehigh Valley Branch Office
617 A Main Street
Hellertown, PA 18055
(800) 272-3900

Alzheimer's Association
Southwestern Pennsylvania Office
620 Freedom Business Drive
Suite 101
King of Prussia, PA 19406
(800) 272-3900

Alzheimer's Association
Berks County Branch Office
237 Court Street
Reading, PA 19601
(800) 272-3900

American Cancer Society
Bethlehem Office
3893 Adler Place, Suite 170
Bethlehem, PA 18017
(610) 882-5774

American Cancer Society
Blue Bell Office
480 Norristown Road, Suite 150
Blue Bell, PA 19422
(888) 202-1487

American Cancer Society
Clearfield Office
26 S. Second Street, Suite 102
Clearfield, PA 16830
(814) 765-1315

American Cancer Society
Erie Office
2115 W. 38th Street
Erie, PA 16508
(814) 866-5174

American Cancer Society
Frackville Office
101 W. Frack Street
Frackville, PA 17931
(570) 874-1413

American Cancer Society
Greensburg Office
510 Pellis Road, Suite 203
Greensburg, PA 15601
(724) 834-9081

American Cancer Society
Hershey Office
Route 422 & Sipe Avenue
Hershey, PA 17033
(717) 533-6144

American Cancer Society
Hollidaysburg Office
1004 N. Juniata Street
Hollidaysburg, PA 16648
(814) 695-9232

American Cancer Society
Lancaster Office
314 Good Drive
Lancaster, PA 17603
(717) 397-3745

American Cancer Society
Lebanon Office
855 Tuck Street, Suite 2
Lebanon, PA 17042
(717) 273-4740

American Cancer Society
Philadelphia Office
1626 Locust Street
Philadelphia, PA 19103
(215) 985-5400

American Cancer Society
Pittsburgh Office
320 Bilmar Drive
Pittsburgh, PA 15205
(412) 919-1100

American Cancer Society
Reading Office
498 Bellevue Avenue
Reading, PA 19605
(610) 921-2329

American Cancer Society
State College Office
1375 Martin Street, Suite 206
State College, PA 16801
(814) 234-1023

American Cancer Society
Stroudsburg Office
2158 W. Main Street
Stroudsburg, PA 18360
(570) 420-1685

American Cancer Society
Taylor Office
712 S Keyser Avenue
Taylor, PA 18517
(570) 562-9749

American Cancer Society
Washington Office
331 S. Main Street
Washington, PA 15301
(724) 222-6911

American Cancer Society
Williamsport Office
1948 E. 3rd Street
Williamsport, PA 17701
(570) 326-4149

American Cancer Society
York Office
924 N. Colonial Avenue
York, PA 17403
(717) 846-2561

American Diabetes Association
Philadelphia Office
150 Monument Road, Suite 10
Bala Cynwyd, PA 19004
(610) 828-5003

American Diabetes Association
Pittsburgh Office
2 Chatham Center, Suite 1520
112 Washington Place
Pittsburgh, PA 15219
(412) 824-1181

American Heart Association
Capital Region Office
1019 Mumma Road
Wormsleyburg, PA 17043
(717) 730-1783

American Heart Association
Lancaster Office
610 Community Way
Lancaster, PA 17603
(717) 393-0725

American Heart Association
Lehigh Valley and Northeast Division
968 Postal Road, Suite 110
Allentown, PA 18109
(610) 867-0583

American Heart Association
Mercer County Office
840 Southwestern Run
Youngstown, OH 44514
(330) 965-9230

American Heart Association
Pittsburgh Metro Office
Four Gateway Center
444 Liberty Avenue, Suite 1300
Pittsburgh, PA 15222-1207
(412) 208-3550

American Heart Association
Southeast Pennsylvania Office
One Penn Cntr. at Suburban Station
1617 JFK Boulevard, Suite 700
Philadelphia, PA 19103
(215) 575-5200

American Lung Association
Allentown Office
2200 W. Hamilton Street,
Suite 318
Allentown, PA 18104
(610) 253-5060, ext. 243

American Lung Association
Harrisburg Office
3001 Gettysburg Road
Camp Hill, PA 17011
(717) 541-5864

American Lung Association
Pittsburgh Office
810 River Avenue, Suite 140
Pittsburgh, PA 15212
(412) 321-4029

American Lung Association
Plymouth Meeting Office
527 Plymouth Road, Suite 415
Plymouth Meeting, PA 19462
(610) 941-9595

Arthritis Foundation
Harrisburg Office
4811 Jonestown Road, Suite 230
Harrisburg, PA 17109
(717) 763-0900

Arthritis Foundation
Pittsburgh Office
Foster Plaza, Suite 11
790 Holiday Drive
Pittsburgh, PA 15220
(412) 566-1645

NAMI Pennsylvania
Altoona Office
P.O. Box 3155
Altoona, PA 16603-3155
(814) 943-0414

NAMI Pennsylvania
Erie Office
1611 Peach Street, Suite 105
Erie, PA 16501-2120
(814) 456-1773

NAMI Pennsylvania
Harrisburg Office
2149 N. 2nd Street
Harrisburg, PA 17110
(717) 238-1514

NAMI Pennsylvania
Harrisburg Office
Polyclinic Campus, Landis Building
2501 N. 3rd Street
Harrisburg, PA 17106
(717) 233-1164

NAMI Pennsylvania
Philadelphia Office
520 N. Delaware Avenue,
7th Floor
Philadelphia, PA 19123
(267) 687-4381

NAMI Pennsylvania
Pittsburgh Office
105 Braunlich Drive
McKnight Plaza
Pittsburgh, PA 15237
(412) 366-3788

NAMI Pennsylvania
Scranton Office
846 Jefferson Avenue
Scranton, PA 18510-1032
(570) 342-1047

OTHER PATIENT RESOURCES

PARTNERSHIP FOR PRESCRIPTION ASSISTANCE (PPA): The Partnership for Prescription Assistance has helped more than 287,000 Pennsylvania patients access free or nearly free prescription medicines for residents who are underinsured or uninsured within the state. Patients should go to *www.pparx.org* for more information. The on-line process takes about 15 minutes, and you'll find out instantly if you're likely to be eligible for help.

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 was deployed in 11 states and the District of Columbia, and helped victims and evacuees who needed to fill or re-fill their prescriptions find open pharmacies. Healthcare Ready also helped emergency responders with critical information on the challenges facing supply chain partners relating to electricity, fuel and transportation issues. See more at *www.healthcareready.org.*

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 approximately 17 percent of all R&D spending by U.S. businesses. The industry and its large-scale research and manufacturing supply chain supports high-quality jobs across the U.S. economy.

Biopharmaceutical companies invest 12 times more in R&D per employee than manufacturing industries overall.

The biopharmaceutical industry supported more than 4.4 million jobs across the U.S. economy in 2014, according to a study by TEConomy Partners.

Since 2000, biopharmaceutical companies that are members of the Pharmaceutical Research and Manufacturers of America have invested more than $600 billion in R&D in the search for new treatments and cures.

ECONOMIC IMPACT OF THE BIOPHARMACEUTICAL SECTOR IN PENNSYLVANIA

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

- Supported more than 254,000 jobs throughout Pennsylvania in 2014.
- Supported the generation of $68.9 billion in economic activity in the state.
- Resulted in more than $3 billion in federal taxation and $469.6 million in state taxes through jobs supported by the biopharmaceutical sector.

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

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:

- **Abington Memorial Hospital,** Abington
- **Albert Einstein Medical Center,** Philadelphia
- **Chambersburg Hospital,** Chambersburg
- **Children's Hospital of Philadelphia,** Philadelphia
- **Children's Hospital of Pittsburgh of UPMC,** Pittsburgh
- **Cooper Institute for Reproductive Hormonal Diseases,** Melrose Park
- **Delmont Surgery Center,** Delmont
- **Donald Guthrie Foundation,** Sayre
- **Drexel University College of Medicine,** Philadelphia
- **Eastern Regional Medical Center,** Philadelphia
- **Fox Chase Cancer Center,** Philadelphia
- **Geisinger Medical Center,** Danville
- **Heritage Valley Health System,** Beaver
- **Lancaster General Hospital,** Lancaster
- **Lehigh Valley Hospital,** Allentown
- **Main Line Health Lankenau Heart Institute,** Wynnewood
- **Mount Nittany Medical Center,** State College
- **Nemours Children's at Jefferson University Hospital,** Philadelphia
- **Patterson Cancer Center at Cole Memorial Hospital,** Coudersport
- **Penn-Presbyterian Hospital,** Philadelphia
- **Penn State University Hershey Medical Center,** Hershey
- **Pennsylvania Hospital,** Philadelphia
- **Philadelphia College of Osteopathic Medicine,** Philadelphia
- **Philadelphia Institute of Dermatology,** Fort Washington
- **Pinnacle Health Hospitals,** Harrisburg
- **Pocono Medical Center,** East Stroudsburg
- **Pottstown Memorial Medical Center,** Pottstown
- **Reading Hospital and Medical Center,** West Reading
- **Riddle Memorial Hospital,** Media
- **Sacred Heart Hospital,** Allentown
- **Shriners Hospital for Children,** Philadelphia
- **Sidney Kimmel Cancer Center at Thomas Jefferson University,** Philadelphia
- **St. Christopher's Hospital for Children,** Philadelphia
- **St. Luke's Hospital & Health Network,** Bethlehem
- **St. Mary's Medical Center,** Langhorne
- **St. Vincent Hospital,** Abington
- **Temple University Hospital,** Philadelphia
- **Thomas Jefferson University,** Philadelphia
- **University of Pennsylvania,** Philadelphia
- **University of Pittsburgh Medical Center,** Pittsburgh
- **VA Pittsburgh Healthcare System,** Pittsburgh
- **WellSpan Ephrata Community Hospital,** Ephrata
- **WellSpan Health York Hospital,** York
- **West Penn Hospital,** Pittsburgh

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 7,100 open clinical trials[1] being sponsored by the biopharmaceutical industry, universities, individuals, and organizations combined. These trials represent studies being funded by industry, research collaboration studies, and research the other groups are undertaking on their own.

In Pennsylvania, of the 1,178 open clinical trials involving the biopharmaceutical research industry, the University of Pennsylvania is collaborating on more than 205 of the trials, the University of Pittsburgh on more than 142, Thomas Jefferson University on more than 120, Penn State University on more than 96, Temple University on more than 30, and Drexel University on more than 15 clinical trials.

THE STATE OF DISEASE IN PENNSYLVANIA

More than 12.7 million people live in Pennsylvania[1], and many are dealing with disease and disability from asthma to cancer and from diabetes to heart disease.

Selected Disease Statistics in Pennsylvania	
Disease	**Health Statistic**
Alzheimer's Deaths, 2015[2]	4,000
Asthma Prevalence-Adults, 2015[2]	955,374
Cancer New Cases, 2017[3]	77,710
Cancer Deaths, 2017[3]	28,510
Chronic Lower Respiratory Diseases, 2015[2]	6,655
Diabetes Prevalence-Adults, 2015[4]	10.4 percent
Diabetes Deaths, 2015[2]	3,765
Heart Disease Deaths, 2015[2]	31,875
HIV-Number Living with a Diagnosis, 2014[4]	33,593
HIV Deaths, 2015[2]	175
Influenza / Pneumonia Deaths, 2015[2]	2,892
Liver Disease/Cirrhosis Deaths, 2015[2]	1,433
Mental Illness-Adults, 2013-2014[4]	1,731,000
Multiple Sclerosis Deaths, 2015[2]	226
Nephritis Deaths, 2015[2]	3,022
Parkinson's Death, 2015[2]	1,348
Septicemia Deaths, 2015[2]	2,353
Stroke Deaths, 2015[2]	6,943

Source: 1. U.S. Census Bureau 2. Pennsylvania Department of Health 3. American Cancer Society 4. Kaiser Family Foundation, State Health Facts

[1] *Data collected from www.clinicaltrials.gov. Search criteria: United States, Phase early 1, 1, 2, 3; Industry and Other, first received on or after 1/1/2004. Search performed 7/26/2017. Open clinical trials are recruiting, not yet recruiting, or are expanded access.*

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

- Children under the age of 18 make up 20.9 percent of the population in Pennsylvania. Pediatric clinical trials are being conducted in the state for cystic fibrosis, diabetes, epilepsy, glioblastoma, hemophilia A, juvenile arthritis, leukemia, neuroblastoma, among others.
- Pennsylvanians aged 65 and older account for 17.4 percent of the states' population. In Pennsylvania, clinical trials are recruiting older people to study potential treatments for diseases such as Alzheimer's disease, chronic obstructive pulmonary disease, Crohn's disease, glaucoma, prostate cancer, heart failure, glaucoma and osteoarthritis, among others.
- Women and girls make up 51 percent of the population in Pennsylvania. Clinical trials are recruiting women for studies on medicines for breast cancer, endometriosis, ovarian cancer, postpartum depression and vaginal infections, among others.

Clinical Trials in Pennsylvania for Special Populations

Population	Number of Trials
Children (birth-17)	197
Seniors (66 and older)	983
Women (only)	65

Source: www.clinicaltrials.gov. Search criteria: Pennsylvania, United States; Phase: early 1, 1, 2, 3; Industry only, first received on or after 1/1/2004. Search performed 7/26/2017. Open clinical trials are recruiting, not yet recruiting, or expanded access.

SCIENCE AND CLINICAL TRIALS

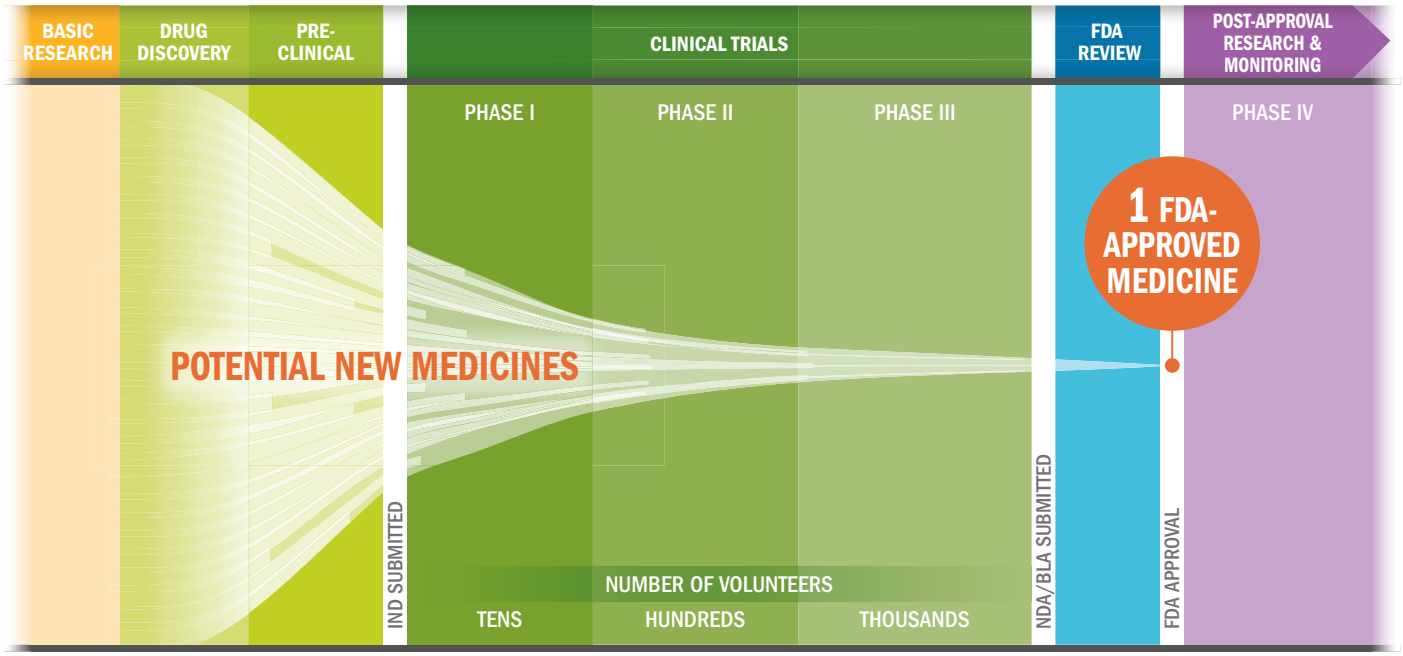
Some of the medicines in clinical testing in Pennsylvania feature revolutionary medical technologies. For example:

- A monoclonal antibody for the treatment of Duchenne muscular dystrophy is being studied in a clinical trial in the **Children's Hospital of Philadelphia**.
- A second-generation medicine for leukemia that blocks the activation of a receptor which is mutated in about one-third of all patients with acute myeloid leukemia is being tested in patients at **Penn State University Cancer Institute** in Hershey and the **University of Pennsylvania Cancer Center** in Philadelphia.
- A medicine for advanced acute myeloid leukemia that inhibits a mutated form of a gene that can lead to increased production of an oncometabolite that prevents immature white cells from developing into healthy infection-fighting cells is in clinical trials at **Penn State University Hershey Medical Center**.
- A monoclonal antibody for rheumatoid arthritis that may block the inflammatory process was studied in clinical trials in **Bethlehem**, **Duncansville** and **Reading**.
- A novel targeted therapy that combines recombinant interleukin-3 with truncated diphtheria toxin is in development for acute myeloid leukemia and blastic plasmacytoid dendritic cell neoplasm, a rare hematological disorder with high unmet medical need and no standard treatment, is in clinical trials at the **University of Pittsburgh Medical Center**.
- A medicine that targets a mutation in the gene that encodes BRAF kinase is being studied to treat melanoma at the **University of Pittsburgh Medical Center Hillman Cancer Center**.
- A monoclonal antibody is in development for the prevention of migraine by inhibiting calcitonin gene-related peptide (CGRP) activity. Anti-CGRP antibodies are thought to help inhibit the transmission of pain signals associated with migraines. The antibody is being studied in a clinical trial in **Philadelphia**.
- An investigational therapeutic using RNAi (RNA interference) is targeting the protein transthyretin (TTR) for the treatment of familial amyloid cardiomyopathy (FAC). RNAi is a biological process that can be used to silence a gene and, in turn, prevent production of the protein it encodes. The RNAi therapeutic just competed a clinical trial in **Philadelphia**.
- An anti-inflammatory medicine in development for acute coronary syndrome inhibits the activity of p38 mitogen activated protein (MAP) kinase, an enzyme associated with the acute inflammation that occurs in the blood vessels during and immediately following an acute coronary syndrome event. The medicine was studied in clinical trials in **Abington**, **Chambersburg**, **Danville**, **Doylestown**, **Wilkes-Barre** and **York**.
- A monoclonal antibody in development for osteoporosis bind to and inhibit the action of sclerostin, a protein encoded by the SOST gene. Mutations in sclerostin have been associated with abnormal bone growth. Inhibiting sclerostin may play a critical role in increasing bone formation and decreasing bone breakdown. It is being studied in clinical trials in **Duncansville**, **Pittsburgh** and **Wyomissing**.

The innovative treatments that are being developed today are helping to expand the frontiers of science and could lead to more and better treatments for patients in the future. In Pennsylvania, this innovation is the result of a successful collaboration between biopharmaceutical companies and local research institutions.

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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