MEDICINES IN DEVELOPMENT

Rare Diseases

A Report on Orphan Drugs in the Pipeline

PRESENTED BY AMERICA'S BIOPHARMACEUTICAL RESEARCH COMPANIES

2013 REPORT

Orphan Drugs in Development*

*Some medicines are listed in more than one category.

More Than 450 Medicines in Development for Rare Diseases

Rare diseases, when taken together, are not that rare at all. In fact, according to the National Institutes of Health (NIH), 30 million Americans have one of the nearly 7,000 diseases that are officially deemed "rare" because alone they each affect fewer than 200,000 people in the United States. Sometimes, only a few hundred patients are known to have a particular rare disease.

Simply receiving a diagnosis of a rare disease often becomes a frustrating quest, since many doctors may have never before heard of or seen the disease.

This is, however, a time of great progress and hope. Biopharmaceutical research is entering an exciting new era with a growing understanding of the human genome. Scientific advances have given researchers new tools to explore rare diseases, which are often more complex than common diseases.

In 2012 alone, 13 orphan drugs were approved for rare diseases, including therapies for Cushing disease, cystic fibrosis and Gaucher disease. America's biopharmaceutical research companies are continuing that progress with 452 medicines and vaccines in development for rare diseases. The medicines listed in this report are either in clinical trials or under review by the Food and Drug Administration (FDA).

A major area of this research targets rare cancers, accounting for more than one-third of all rare disease medicines in development. Other top research areas include genetic disorders, neurological conditions, infectious diseases and autoimmune disorders.

Despite some recent victories, research into treatments for rare diseases is a daunting quest. This ongoing innovation and the hundreds of new medicines in development now offer hope that physicians will have new treatment options for patients confronting a rare disease.

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PhRMA
RESEARCH • PROGRESS • HOPE

Innovative Orphan Drugs in the Pipeline for Rare Diseases

The 452 medicines and vaccines in development for rare diseases employ exciting new scientific and technical knowledge. Many of the medicines, which offer hope for those suffering from one of the nearly 7,000 rare diseases, represent innovative new ways to target disease, including:

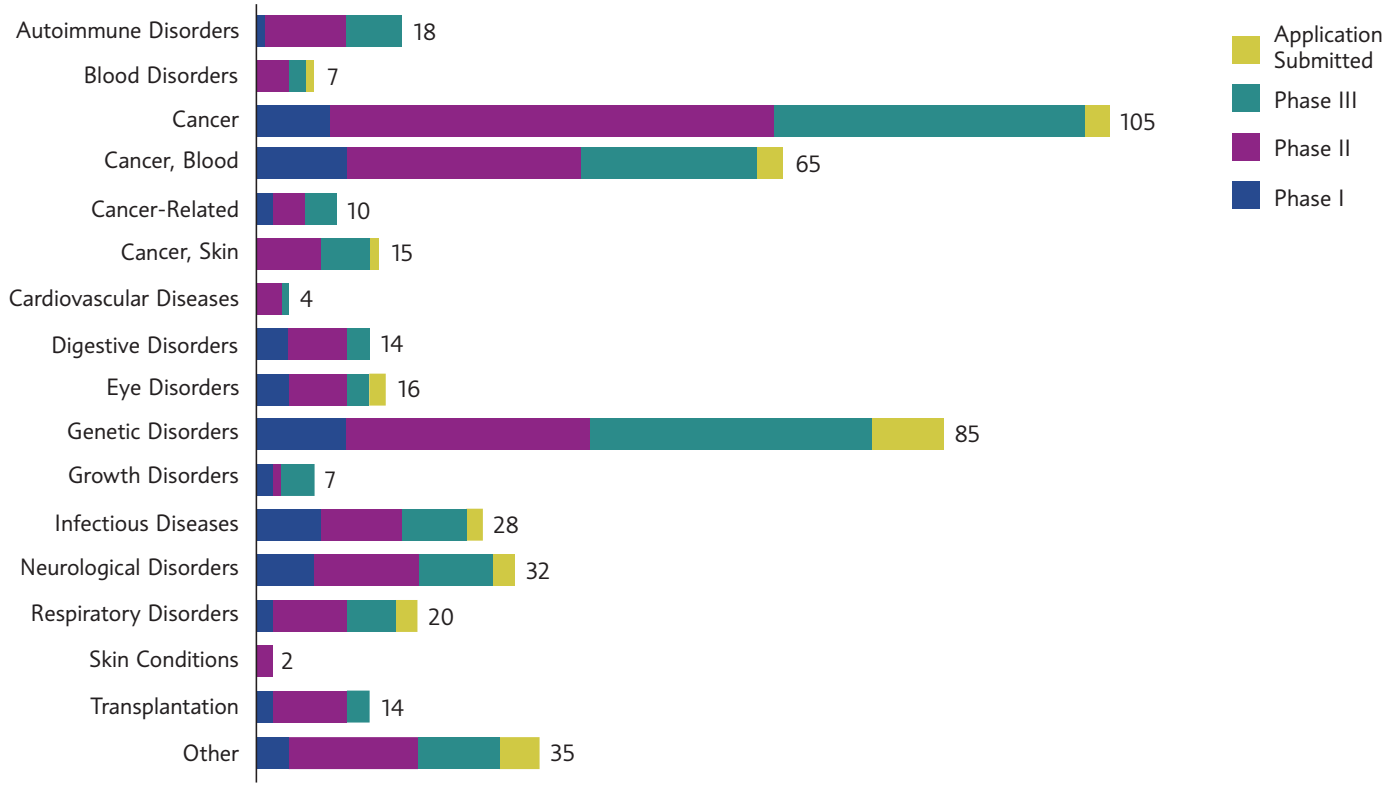
Targeted RNAi Therapy Approach for Duchenne Muscular Dystrophy—In clinical trials, RNAi therapies have shown potential in treating neuromuscular disorders such as Duchenne muscular dystrophy (DMD), which affects about 1 in every 3,500 to 6,000 male births each year in the United States. In DMD, DNA deletions cause mutations in important genes that encode for dystrophin, a structural protein found in normal muscle. The loss of this protein causes muscle fibers to disintegrate faster than they can be regenerated. One medicine in development targets restoration of dystrophin function and, as dystrophin expression increases, there have been demonstrated improvements in patients' ability to walk.

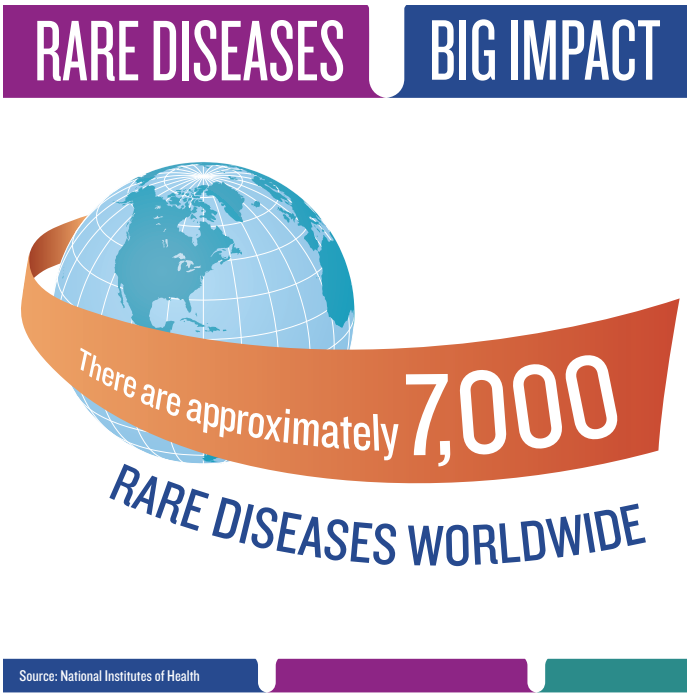
Potential New Treatment for a Genetic Disease in Infants—Hypophosphatasia is a rare inherited bone disease that results from a genetic mutation which hinders the formation of bones and teeth and can result in substantial skeletal abnormalities. Severely affected infants often have persistent bone disease or die from respiratory insufficiency due to progressive chest deformity from poorly developed bones. Currently, there are no approved medicines for this disease. One therapy in development delivers the enzyme necessary for proper bone growth that patients with hypophosphatasia are missing.

Treatments for Patients with Debilitating Lung Disease—Idiopathic pulmonary fibrosis (IPF) is a debilitating and almost uniformly fatal disease in which patients experience progressive difficulty breathing due to scarring of the lungs. There are currently no effective treatment options available, and

Medicines in Development By Disease and Phase

Some medicines are listed in more than one category.





the average patient with IPF dies within three years of diagnosis. A medicine in development targets connective tissue growth factor, which is elevated in the lungs of IPF patients. Researchers recently announced promising results from a Phase II trial in which 60 percent of IPF patients were able to stabilize their disease or experience improvement in lung function.

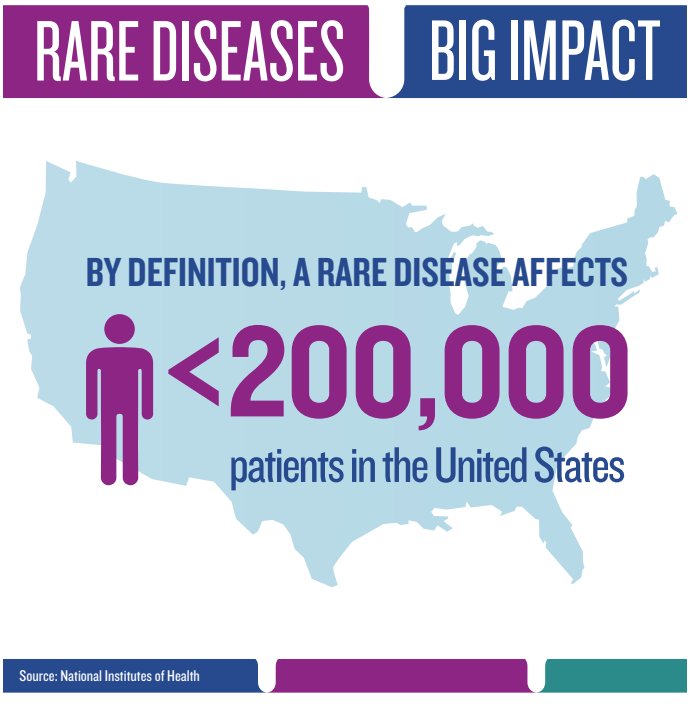
ORPHAN DRUG ACT OF 1983—A SUCCESS STORY

Recognizing the scarcity of medicines to treat rare diseases with very small patient populations, the Orphan Drug Act of 1983 provided incentives to companies developing rare disease treatments. Over the last 30 years, more than 400 medicines representing 447 separate indications have been approved to treat rare diseases, compared to fewer than 10 in the 1970s. As of September 15, 2013, the FDA has granted the orphan drug designation to 2,899 potential therapies.

ALS: Fighting a Devastating Disease—Amyotrophic lateral sclerosis (ALS), or Lou Gehrig's disease, is a progressive neurodegenerative disease that causes the brain to lose control over body movement, ultimately resulting in paralysis and death. The one drug approved to treat ALS can modestly slow progression of the disease, but new treatments are needed. As our scientific understanding of the disease has grown, researchers are pursuing many new approaches to halt or slow progression, including the use of the patient's own bone marrow stem-cells to create healthy neuron-like cells to replace diseased neurons. Other trials are studying ways to prompt the immune system to protect neurons affected by ALS.

Two Targets to Fight Leukemia—A potential first-in-class medicine for acute lymphoblastic leukemia (ALL) is a bispecific T-cell engager antibody designed to focus the body's cell destroying T-cells against cells expressing CD19, a protein found on the surface of B-cell-derived leukemia and lymphoma. The modified antibodies are designed to engage two different targets simultaneously, linking the T-cells to cancer cells.

Combination Vaccine Treatment for Pancreatic Cancer—A potential treatment for pancreatic cancer is a combination of two therapeutic vaccines. The treatment combines a Listeria-based vaccine that has been engineered to express the

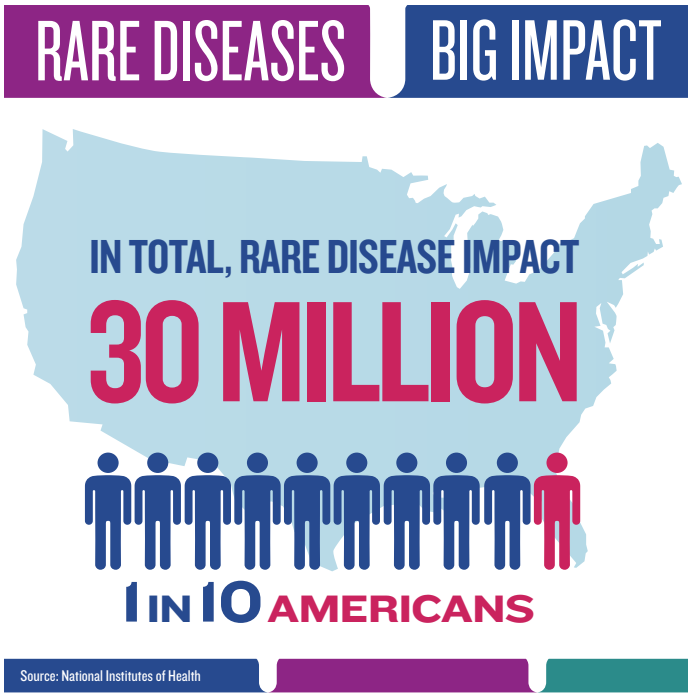


tumor-associated antigen mesothelin and allogeneic pancreatic cancer cells that are genetically-modified to secrete the immune-stimulant, granulocyte-macrophage colony stimulating factor (GM-CSF). The cells are irradiated to prevent further cell growth although they stay metabolically active. Sequential administration of the vaccines in animal studies have demonstrated enhanced tumor-specific T-cell and anti-tumor responses.

Orphan Drug Approvals for Rare Diseases

Since passage of the Orphan Drug Act in 1983, more than 400 medicines for rare diseases have been brought to market. Below are some key approvals for rare diseases in recent years that represent new technologies or a more defined patient population target.

Zelboraf® (vemurafenib), a personalized medicine, was approved for the treatment of unresectable or metastatic melanoma that expresses a gene mutation called BRAF V600E. It was approved with a first-of-its-kind companion diagnostic (4800 BRAF V600 Mutation Test) to help determine if a patient has the gene mutation. A normal BRAF protein is involved in regulating cell growth but a mutated form is found in about half of the late-stage melanoma cases. Zelboraf is able to block the function of the V600E-mutated BRAF protein.



SEEKING NEW TREATMENTS FOR CHILDREN WITH A RARE DISEASE

The National Institutes of Health estimates that 50 percent of people affected by rare diseases are children, making rare diseases a particularly deadly and debilitating concern for children worldwide. Rare diseases are responsible for 35 percent of deaths in the first year of life and 30 percent of children with a rare disease will not live to see their fifth birthday.

Encouragingly, one in three of the nearly 3,000 treatments with orphan designation are for children. In addition to the Orphan Drug Act, two other laws have made a significant impact on pediatric research. The Best Pharmaceuticals for Children Act (BPCA) *and the* Pediatric Research Equity Act (PREA) *have resulted in a wealth of useful information about dosing, safety, and efficacy. According to the FDA, BPCA and PREA have resulted in 467 pediatric labeling changes since 1988. Together, BPCA and PREA have helped foster research and greatly advanced our ability to treat pediatric patients.*

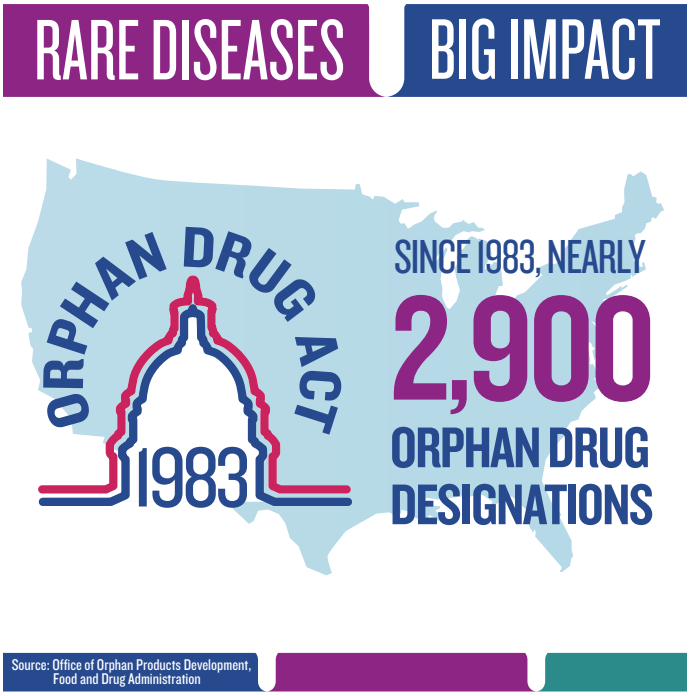
BPCA and PREA were permanently reauthorized by the Food and Drug Administration Safety and Innovation Act of 2012 (FDASIA). By providing a predictable regulatory environment, the permanent reauthorization will help ensure that pediatric research by biopharmaceutical companies continues to advance children's medical care. FDASIA also required the FDA to hold a public meeting and issue a report on encouraging and accelerating development of new therapies for pediatric rare diseases.

"In the last five years, one-third of all new drug approvals were for rare diseases"
—FDA

Adcetris® (brentuximab vedotin), the first in a new class of antibody-drug conjugates (ADCs), was approved to treat Hodgkin lymphoma and systemic anaplastic large cell lymphoma (ALCL), a rare type of lymphoma that represents only 3 percent of all non-Hodgkin lymphomas. ADCs combine a monoclonal antibody and a therapeutic drug, where the antibody directs the therapeutic to target the cancerous cells. It is also the first FDA-approved drug for Hodgkin lymphoma in more than 30 years and the first to specifically treat ALCL. Adcetris is composed of an anti-CD30 monoclonal antibody and a microtubule disrupting agent, allowing it to release its therapeutic drug once inside the CD30-expressing tumor cells.

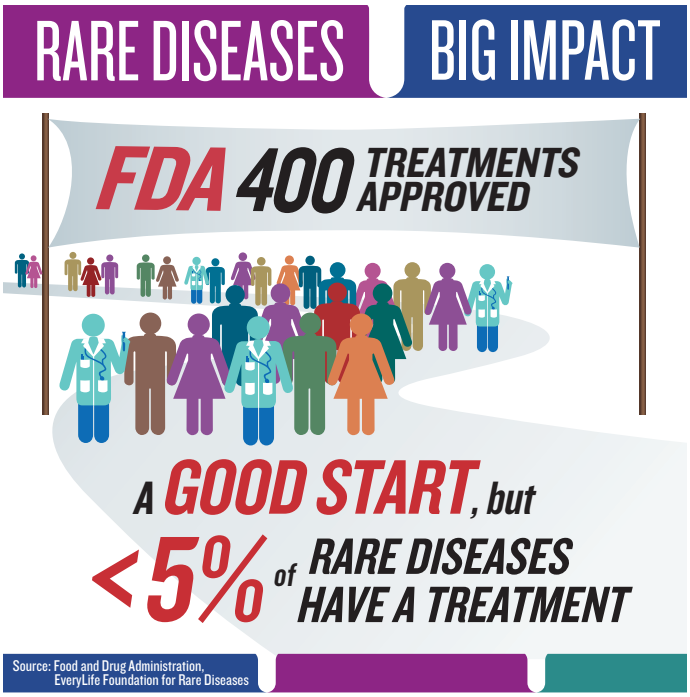
Ilaris® (canakinumab), a fully human monoclonal antibody, was approved to treat adults and children as young as age 4 with cryopyrin-associated periodic syndrome (CAPS). Specifically, Ilaris was approved to treat two types of CAPS: familial cold-auto-inflammatory syndrome (FACS) and Muckle-Wells syndrome (MWS). CAPS is a serious auto-inflammatory disease that lasts a lifetime with symptoms such as fever, headache, fatigue, skin rash, painful joints and muscles. CAPS is caused by a single gene mutation that leads to overproduction of interleukin-1 beta, which causes sustained inflammation and tissue damage. Ilaris rapidly and selectively blocks production of interleukin-1 beta.

Kalydeco™ (ivacaftor) was the first medicine approved to treat the underlying cause of cystic fibrosis (CF) and not just the symptoms of the disease. It targets a defective protein to help achieve sustained improvement in lung function. Kalydeco was approved for use in people with cystic fibrosis (ages 6 and older) who have at least one copy of the G551D mutation in the CF transmembrane conductance regulator (CFTR) gene. The defect affects a small portion of CF patients—about 5 percent or 1,200 of the 30,000 CF sufferers—but it also provides hope that knowledge gained will lead to treatments that will help even more CF patients.



PATIENT ENGAGEMENT

The biopharmaceutical research industry is committed to close collaboration and regular coordination with rare disease patient groups and other rare disease stakeholders. We must work together towards building a positive policy environment to help us meet the public health challenges that rare diseases present.



Challenges in Clinical Trials for Rare Diseases

Advances in science and technology, such as personalized medicine, are creating new opportunities to improve and expand research into rare diseases and the development of new treatments. While personalized medicine is just beginning to impact patients, the Personalized Medicine Coalition estimates that available personalized medicines, treatments and diagnostic products increased from 13 in 2006 to 72 by 2011. In just 10 years since the human genome was mapped, real progress has been made.

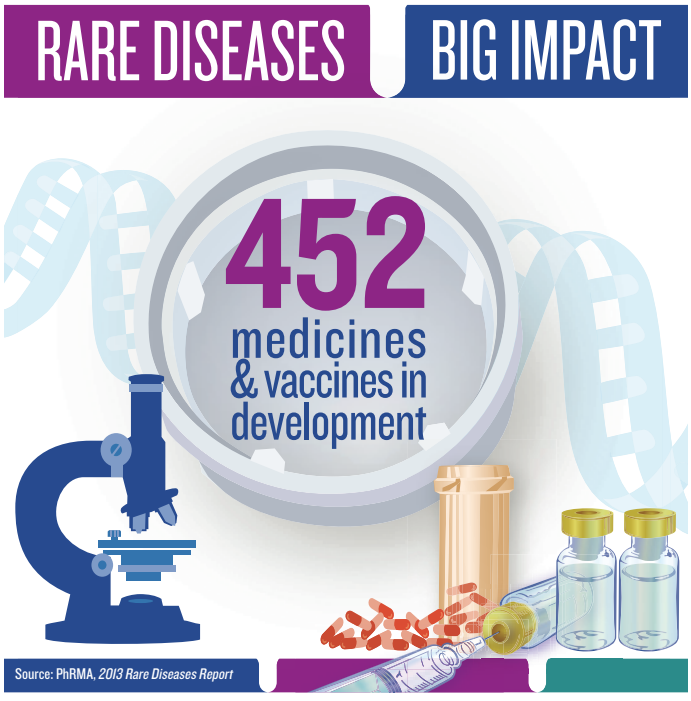
The sequencing of the human genome and the analysis of critical proteins in the blood have profoundly impacted biopharmaceutical research and are yielding important new tools for understanding and treating a wide range of conditions. These tools are proving critical for taking on rare diseases, which are often more complex than more common diseases. Many rare diseases will require new tactics to find effective treatments.

Researchers are increasingly able to identify much more targeted patient populations and this new knowledge is allowing clinicians to discover whether a patient is developing or will develop an illness much earlier. Already researchers have found genes associated with diseases such as myotonic dystrophy, ALS, cystic fibrosis, progeria and neurofibromatosis types 1 and 2. These breakthroughs are a crucial step toward new treatments.

Evolving genetic research may offer a solution to the challenges of clinical development. Genetic markers may make it possible to identify a patient population in advance and allow clinical trials with a smaller number of participants.

Before a potential new treatment can be approved, it must be tested in clinical trials. It is often difficult to find patients to volunteer in clinical trials, and rare diseases pose an even greater challenge. Specific rare disease patient populations are very small, geographically dispersed and often include children. The biopharmaceutical sector is working with patient advocacy organizations to identify and advance better ways to connect patients to biopharmaceutical and academic researchers conducting clinical trials.

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



Prescription Drug User Fee Act (PDUFA) Continues To be a Success for Patients and Medical Innovation

Since 1992, the Prescription Drug User Fee Act (PDUFA) has provided additional resources to FDA to help make safe and effective new medicines available to patients in a timely manner. In its first 20 years, PDUFA brought more than 1,500 new medicines to Americans; increased FDA's staffing and resources; helped improve the consistency, predictability and efficiency of FDA reviews; encouraged medical innovation and economic growth; and preserved and strengthened FDA's high safety standards. PDUFA has been reauthorized and amended four times since its original passage in 1992—in 1997, 2002, 2007, and most recently in 2012 as PDUFA V.

PDUFA V encompasses several provisions that will positively impact research and development of treatments for rare diseases.

PDUFA V Advances Regulatory Science

Through PDUFA V, FDA will have dedicated resources to develop and apply new scientific tools and approaches to assess the safety and effectiveness of new medicines.

- Additional FDA resources will help to better address submissions that involve pharmacogenomics and qualified biomarkers.
- FDA will support the development of treatments for rare diseases by increasing outreach to the patient community, providing specialized training for agency staff, and developing guidance and policy related to advancing and facilitating the development of medicines for rare diseases.
- Dedicated FDA staff will evaluate and define best practices for the conduct of meta-analyses and to inform guidance on commonly accepted and standardized methodologies to be used in drug review and safety monitoring.
- Dedicated FDA staff will support the advancement of the use of patient-reported outcomes and other outcomes assessment tools.

Rare Disease Facts and Statistics

Here are a few statistics and facts to illustrate the breadth of the rare disease challenge in the United States and worldwide.

- There are approximately 7,000 different types of rare diseases and disorders, with more being discovered each day.
- 30 million people in the United States are living with rare diseases. This equates to 1 in 10 Americans or 10 percent of the population.
- It is estimated that 350 million people worldwide suffer from rare diseases.
- If all of the people with rare diseases lived in one country, it would be the world's third most populous country.
- In the United States, a condition is considered "rare" if it affects fewer than 200,000 people.
- About 80 percent of rare diseases are genetic in origin, and thus are present throughout a person's life, even if symptoms do not immediately appear.
- The prevalence distribution of rare diseases is skewed— 80 percent of all rare disease patients are affected by approximately 350 rare diseases.
- According to the EveryLife Foundation for Rare Diseases, 95 percent of rare diseases lack a single FDA approved treatment.

Source: The Global Genes Project, a program of The R.A.R.E. Project

Autoimmune Disorders

Product Name	Sponsor	Official FDA Designation*	Development Status**
Actemra® tocilizumab	Genentech *South San Francisco, CA*	treatment of systemic sclerosis to be a separate disease or condition from localized scleroderma	Phase II www.gene.com
apremilast (CC-10004)	Celgene *Summit, NJ*	treatment of Behcet's disease	Phase II www.celgene.com
ARA290	Araim Pharmaceuticals *Ossining, NY*	treatment of neuropathic pain in patients with sarcoidosis	Phase II www.araim.org
ARG201 (type 1 native bovine skin collagen)	arGentis Pharmaceuticals *Memphis, TN*	treatment of diffuse systemic sclerosis	Phase II www.argentisrx.com
caplacizumab (ALX-0081)	Ablynx *Ghent, Belgium*	treatment of thrombotic thrombocytopenic purpura	Phase II www.ablynx.com
Diamyd® autoimmune diabetes vaccine	Diamyd Medical *Stockholm, Sweden*	treatment of type I diabetes with residual beta cell function	Phase III www.diamyd.com
DiaPep277® (peptide vaccine)	Andromeda Biotech *Ness Ziona, Israel*	for use in type 1 diabetic mellitus patients with residual beta-cell function	Phase III www.andromedabio.com
E5501 (avatrombopaq)	Eisai *Woodcliff Lake, NJ*	treatment of idiopathic thrombocytopenic purpura	Phase III www.eisai.com
Firdapse™ amifampridine	Catalyst Pharmaceutical *Coral Gables, FL*	treatment of Lambert-Eaton myasthenic syndrome (Breakthrough Therapy)	Phase III www.catalystpharma.com
gevokizumab (XOMA 052)	XOMA *Berkeley, CA*	treatment of Behcet's disease (see also eye)	Phase III www.xoma.com
Gilenya® fingolimod	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of chronic inflammatory demyelinating polyneuropathy	Phase III www.novartis.com
IBC-VS01 (insulin B-chain vaccine)	Orban Biotech *Brookline, MA*	treatment of type 1 diabetes patients with residual beta cell function	Phase I www.orbanbiotech.com
Oralgam™ human gammaglobulin oral	Latona Life Sciences *Scottsdale, AZ*	treatment for juvenile rheumatoid arthritis	Phase II completed www.latonalifesciences.com
Pomalyst® pomalidomide	Celgene *Summit, NJ*	treatment of systemic sclerosis (see also cancer, related)	Phase II www.celgene.com

*The designation is issued by the FDA's Office of Orphan Products Development while the drug is still in development. The designation makes the sponsor of the drug eligible for entitlements under the Orphan Drug Act of 1983. The entitlements include seven years of marketing exclusivity following FDA approval of the drug for the designated use.

**For more information about a specific medicine or company in the report, please use the website provided.

Autoimmune Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
Prochymal® remestemcel-L	Osiris Therapeutics *Columbia, MD*	treatment of type 1 diabetes patients with residual beta cell function (see also transplantation)	Phase II www.osiris.com
Simponi® golimumab	Janssen Biotech *Horsham, PA*	treatment of sarcoidosis (see also digestive)	Phase II www.janssenbiotech.com
Stelara® ustekinumab	Janssen Biotech *Horsham, PA*	treatment of chronic sarcoidosis --- treatment of primary biliary cirrhosis	Phase II www.janssenbiotech.com --- Phase II www.janssenbiotech.com
teplizumab (anti-CD3 mAb)	MacroGenics *Rockville, MD*	treatment of recent-onset type 1 diabetes	Phase III www.macrogenics.com

Blood Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
fedratinib	Sanofi US *Bridgewater, NJ*	treatment of secondary and primary myelofibrosis --- treatment of polycythemia vera	Phase III www.sanofi.com --- Phase II www.sanofi.com
human prothrombin complex concentrate	Octapharma USA *Hoboken, NJ*	reversal of anticoagulation therapy in patients needing treatment of serious or life-threatening bleeding and/or needing urgent surgery or invasive procedures	application submitted www.octapharma.us
Jakafi® ruxolitinib	Incyte *Wilmington, DE*	treatment of polycythemia vera (Fast Track) (see also cancer) --- treatment of essential thrombocythemia	Phase III www.incyte.com --- Phase II www.incyte.com
LY2784544 (gandotinib)	Eli Lilly *Indianapolis, IN*	treatment of myeloproliferative disorders (polycythemia vera, essential thrombocythemia and myelofibrosis)	Phase II www.lilly.com

Blood Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
pacritinib	Cell Therapeutics *Seattle, WA*	treatment of myeloproliferative disorders with the JAK2 V617F mutation	Phase III www.celltherapeutics.com
simtuzumab (anti-LOXL2 mAb)	Gilead Sciences *Foster City, CA*	treatment of myelofibrosis (see also cancer, respiratory)	Phase II www.gilead.com
SPD602 (iron chelator)	Shire *Wayne, PA*	treatment of chronic iron overload in patients with transfusion-dependent anemias	Phase II www.shire.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
Abraxane® paclitaxel protein-bound particles for injection suspension, (albumin-bound)	Celgene *Summit, NJ*	treatment of pancreatic cancer (see also cancer, skin)	Phase III www.celgene.com
ABT-414 (EGFR ADC)	AbbVie *North Chicago, IL*	treatment of glioblastoma multiforme (GBM)	Phase I www.abbvie.com
ADI-PEG20 (pegylated arginine deiminase)	Polaris Pharmaceuticals *San Diego, CA*	treatment of hepatocellular melanoma (see also cancer, skin)	Phase III www.polarispharma.com
ADXS-HPV	Advaxis *Princeton, NJ*	treatment of HPV-associated anal cancer	Phase I/II www.advaxis.com
AEZS-108 (zoptarelin doxorubicin)	AEterna Zentaris *South San Francisco, CA*	treatment of ovarian cancer	Phase II completed www.aezsinc.com
Afinitor® everolimus	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of hepatocellular carcinoma	Phase III www.novartis.com
aldoxorubicin	CytRx *Los Angeles, CA*	treatment of soft tissue sarcoma --- treatment of adenocarcinoma of the pancreas	Phase II www.cytrx.com --- Phase II www.cytrx.com
algenpantucel-L	NewLink Genetics *Ames, IA*	treatment of pancreatic cancer (Fast Track)	Phase III www.linkp.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
alisertib (MLN8237)	Millennium Pharmaceuticals *Cambridge, MA*	treatment of ovarian cancer (see also cancer, blood)	Phase II completed www.millennium.com
AMG 102 (rilotumumab)	Amgen *Thousand Oaks, CA*	treatment of gastric cancer including gastroesophageal junction adenocarcinoma	Phase III www.amgen.com
amrubicin	Celgene *Summit, NJ* Dainippon Sumitomo Pharma *Osaka, Japan*	treatment of small-cell-lung cancer	Phase III www.celgene.com
ARC-100 (abeotaxane inhibitor)	Archer Biosciences *New York, NY*	treatment of gliomas --- treatment of pediatric neuroblastoma	Phase II www.archerbiosciences.com --- Phase I/II www.archerbiosciences.com
Archexin® antisense oligonucleotide	Rexahn *Rockville, MD*	treatment of pancreatic cancer	Phase II www.rexahn.com
astuprotimut-R	GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of MAGE-A3 positive non-small-cell lung cancer (see also cancer, skin)	Phase III www.gsk.com
ATR-101	Atterocor *Ann Arbor, MI*	treatment of adrenocortical carcinoma	Phase I www.atterocor.com
AV0113 (dendritic cell cancer immunotherapy)	Activartis Biotech *Vienna, Austria*	treatment of malignant glioma	Phase II www.activartis.com
Avastin® bevacizumab	Genentech *South San Francisco, CA*	therapeutic treatment of patients with ovarian cancer	application submitted www.gene.com
Azedra™ iobenguane I-131	Progenics Pharmaceuticals *Tarrytown, NY*	treatment of neuroendocrine tumors (Fast Track)	Phase II www.progenics.com
BAY 94-9343 (anti-mesothelin-ADC)	Bayer HealthCare Pharmaceuticals *Wayne, NJ*	treatment of mesothelioma	Phase I www.bayerpharma.com
BC-819 (gene therapy)	BioCancell Therapeutics *Jerusalem, Israel*	treatment of pancreatic cancer (Fast Track)	Phase II www.biocancell.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
CBP501 (peptide mimetic)	CanBas *Shizouka, Japan*	for use in combination with cisplatin and pemetrexed for the treatment of patients with mesothelioma	Phase II www.canbas.co.jp
Ch14.18 mAb	United Therapeutics *Silver Spring, MD*	treatment of neuroblastoma	Phase III www.unither.com
CO-1686 (EGFR receptor antagonist)	Clovis Oncology *Boulder, CO*	treatment of non-small-cell lung cancer and mutations in the epidermal growth factor receptor	Phase I/II www.clovisoncology.com
Cotara® TNT-1B mAB	Peregrine Pharmaceuticals *Tustin, CA*	treatment of glioblastoma multiforme and anaplastic astrocytoma	Phase II www.peregrineinc.com
CP-613	Cornerstone Pharmaceuticals *Cranbury, NJ*	treatment of pancreatic cancer	Phase I/II www.cornerstonepharma.com
CPP-1X (eflornithine)	Cancer Prevention Pharmaceuticals *Tucson, AZ*	treatment of neuroblastoma	Phase II www.canprevent.com
crenolanib (CP-868-596)	AROG Pharmaceuticals *Dallas, TX*	treatment of malignant glioma (see also cancer, blood)	Phase I www.arogpharma.com
CRS-207/GVAX Pancreas (listeria monocytogenes)	Aduro Biotech *Berkeley, CA*	treatment of pancreatic cancer	Phase II www.adurobiotech.com
CVac™ intradermal cancer vaccine	Prima Biomed *Sydney, Australia*	treatment of ovarian cancer	Phase II/III www.primabiomed.com
DCVax®-Brain dendritic cell vaccine	Northwest Biotherapeutics *Bethesda, MD*	treatment of primary brain malignant cancer	Phase III www.nwbio.com
EGEN-001	Expression Genetics *Huntsville, AL*	treatment of ovarian cancer	Phase II www.egeninc.com
ENMD-2076	EntreMed *Rockville, MD*	treatment of ovarian carcinoma	Phase II www.entremed.com
ensituximab (NPC-1C)	Precision Biologics *Dallas, TX*	treatment of pancreatic cancer	Phase I/II www.precision-biologics.com
Estybon® rigosertib	Onconova Therapeutics *Newton, PA*	treatment of pancreatic cancer (see also cancer, blood)	Phase III www.onconova.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
etirinotecan pegol (NKTR-102)	Nektar Therapeutics *San Francisco, CA*	treatment of ovarian cancer	Phase II www.nektar.com
FANG™ Vaccine autologous tumor cell vaccine	Gradalis *Carrollton, TX*	treatment of ovarian cancer (see also cancer, skin)	Phase II www.gradalisinc.com
farletuzumab (MORAb-003)	Eisai *Woodcliff Lake, NJ*	treatment of ovarian cancer	Phase III www.eisai.com
G-202	GenSpera *San Antonio, TX*	treatment of of hepatocellular carcinoma	Phase II www.genspera.com
G-203-2c (synthesized peptide)	Genus Oncology *Vernon Hills, IL*	treatment of pancreatic cancer	Phase I www.genusoncology.com
G-series prophage vaccine (vitespan)	Agenus *Lexington, MA*	treatment of glioma	Phase II www.agenusbio.com
GL-0810 (HPV-16 cancer vaccine)	Gliknik *Baltimore, MD*	treatment of HPV-16-expressing head and neck squamous cell carcinoma	Phase II www.gliknik.com
GL-0817 (MAGE-A3 cancer therapeutic Trojan peptide vaccine)	Gliknik *Baltimore, MD*	treatment of MAGE-A3-expressing head and neck squamous cell carcinoma	Phase II www.gliknik.com
GliAtak® gene therapy	Advantagene *Auburndale, MA*	treatment of malignant brain tumors	Phase II www.advantagene.com
glufosfamide	Eleison Pharmaceuticals *St. Petersburg, FL*	for treatment of pancreatic cancer (Fast Track)	Phase III www.eleison-pharma.com
Havalen® eribulin	Eisai *Woodcliff Lake, NJ*	treatment of advanced soft tissue sarcoma	Phase III www.eisai.com
ICT-107 (dendritic cancer vaccine)	ImmunoCellular Therapeutics *Calabasas, CA*	treatment of glioblastoma or brain stem glioma	Phase II www.imuc.com
IMA901 (peptide vaccine)	immatics biotechnologies *Tuebingen, Germany*	treatment of renal cell carcinoma in HLA-A*2-positive patients	Phase III www.immatics.com
IMGN901 (maytansinoid DM-1-conjugated mAb)	ImmunoGen *Waltham, MA*	treatment of small-cell lung cancer (see also cancer, blood)	Phase II www.immunogen.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
IRX-2	IRX Therapeutics *New York, NY*	neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck (Fast Track)	Phase II completed www.irxtherapeutics.com
Jakafi® ruxolitinib	Incyte *Wilmington, DE*	treatment of pancreatic cancer (see also blood)	Phase II www.incyte.com
lenvatinib	Eisai *Woodcliff Lake, NJ*	treatment of follicullar, medullary, anaplastic cancer and metastatic or locally advanced papillary thyroid	Phase III www.eisai.com
Lutathera® DOTA-Tyr3 octreotide	Advanced Accelerator Applications *Saint Genis Pouilly, France*	treatment of gastro-entero-pancreatic neuroendocrine tumors	Phase III www.adacap.com
masitinib (AB-1010)	AB Science *Short Hills, NJ*	treatment of malignant gastrointestinal stromal tumors --- treatment of patients with pancreatic cancer	Phase III www.ab-science.com --- Phase III www.ab-science.com
mibefradil	Tau Therapeutics *Charlottesville, VA*	treatment of glioblastoma multiforme	Phase I www.tautherapeutics.com
mifamurtide	Millennium Pharmaceuticals *Cambridge, MA*	treatment of osteosarcoma	Phase III www.millennium.com
milciclib	Nerviano Medical Sciences *Nerviano, Italy*	treatment of thymic epithelial tumors	Phase II www.nervianoms.com
MK-1775 (WEE1 tyrosine kinase inhibitor)	Merck *Whitehouse Station, NJ*	treatment of ovarian cancer	Phase II www.merck.com
MM-111 (bispecific antibody mAb)	Merrimack Pharmaceuticals *Cambridge, MA*	treatment of HER2-expressing advanced adenocarcinoma of the stomach and gastroesophageal junction --- treatment of HER2-expressing adenocarcinoma of the esophagus	Phase II www.merrimackpharma.com --- Phase II www.merrimackpharma.com
MM-398 (nanoliposomal irinotecan)	Merrimack Pharmaceuticals *Cambridge, MA*	treatment of pancreatic cancer	Phase III www.merrimackpharma.com
MORAb-004	Eisai *Woodcliff Lake, NJ*	treatment of soft tissue sarcoma	Phase II www.eisai.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
MORAb-009 (amatuximab)	Eisai *Woodcliff Lake, NJ*	treatment of mesothelioma	Phase II www.eisai.com
MORAb-066	Eisai *Woodcliff Lake, NJ*	treatment of pancreatic cancer	Phase I www.eisai.com
Multikine® leukocyte interleukin	CEL-SCI *Vienna, VA*	neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck	Phase III www.cel-sci.com
Nexavar® sorafenib	Bayer HealthCare Pharmaceuticals *Wayne, NJ*	treatment of medullary thyroid cancer, anaplastic thyroid cancer, and recurrent or metastatic follicular or papillary thyroid cancer	application submitted www.bayerpharma.com
NGF-hTNF (recombinant fusion protein)	MolMed *Milan, Italy*	treatment of malignant pleural mesothelioma	Phase III www.molmed.com
niraparib (ADP-ribose PARP inhibitor)	TESARO *Waltham, MA*	treatment of ovarian cancer	Phase III www.tesarobio.com
onartuzumab	Genentech *South San Francisco, CA*	treatment of gastric cancer including gastroesophageal cancer	Phase III www.gene.com
Oncophage® prophage cancer vaccine	Agenus *Lexington, MA*	treatment of renal cell carcinoma (Fast Track)	Phase III www.agenusbio.com
Opaxio® paclitaxel poliglumex	Cell Therapeutics *Seattle, WA*	treatment of glioblastoma multiforme	Phase II www.celltherapeutics.com
oregovomab (B43.14 mAb)	Quest PharmaTech *Edmonton, Canada*	treatment of epithelial ovarian cancer	Phase II www.questpharmatech.com
OSE2101	OSE Pharma *Paris, France*	treatment of non-small-cell lung cancer in patients expressing HLA-A2	Phase III www.osepharma.com
perifosine	AEterna Zentaris *Basking Ridge, NJ*	treatment of neuroblastoma	Phase I www.aezsinc.com
Perjeta® pertuzumab	Genentech *South San Francisco, CA*	treatment of gastric cancer	Phase III www.gene.com
pexastimogene devacirepvec (Pexa-Vac; JX-594)	Jennerex, Inc. *San Francisco, CA*	treatment of hepatocellular carcinoma	Phase II www.jennerex.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
pimasertib (MEK inhibitor-1)	EMD Serono *Rockland, MA*	treatment of pancreatic cancer	Phase II www.emdserono.com
polyclonal antibody stimulator (G17DT immunogen)	Cancer Advances *Durham, NC*	treatment of gastric cancer --- treatment of adenocarcinoma of the pancreas	Phase III www.canceradvancesinc.com --- Phase III www.canceradvancesinc.com
Poly-ICLC	Emory University *Atlanta, GA* Oncovir *Washington, DC* University of California *San Diego, CA*	treatment of primary brain tumors	Phase II www.oncovir.com
PV-10 (rose bengal disodium)	Provectus Pharmaceuticals *Knoxville, TN*	treatment of hepatocellular carcinoma (see also cancer, skin)	Phase I www.provectus.com
ramucirumab	Eli Lilly *Indianapolis, IN*	treatment of gastric cancer --- treatment of hepatocellular carcinoma	application submitted www.lilly.com --- Phase III www.lilly.com
rindopepimut	Celldex Therapeutics *Phillipsburg, NJ*	treatment of EGFRvIII-expressing glioblastoma multiforme (Fast Track)	Phase II/III www.celldextherapeutics.com
rucaparib (PARP inhibitor)	Clovis Oncology *Boulder, CO*	treatment of ovarian cancer	Phase II www.clovisoncology.com
salirasib (KD032)	Kadmon *Warrendale, PA*	treatment of panreatic cancer	Phase II www.kadmon.com
simtuzumab (anti-LOXL2 mAb)	Gilead Sciences *Foster City, CA*	treatment of pancreatic cancer (see also blood, respiratory)	Phase II www.gilead.com
Somatuline® lanreotide acetate	Ipsen Biopharmaceuticals *Basking Ridge, NJ*	treatment of symptoms associated with carcinoid syndrome --- treatment of neuroendocrine tumors	Phase III www.ipsen.com --- Phase III www.ipsen.com
SP1049C	Supratek Pharma *Montreal, Canada*	treatment of gastric cancer	Phase II www.supratek.com
squalamine	Ohr Pharmaceutical *New York, NY*	treatment of ovarian cancer refractory or resistant to standard chemotherapy	Phase II www.ohrpharmaceutical.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
tegafur/gimeracil/oteracil	Taiho Pharma USA *Princeton, NJ*	treatment of gastric cancer	Phase III www.taiho.co.jp
telatinib (VEGFR/PDGFR/KIT inhibitor)	ACT Biotech *San Francisco, CA*	treatment of gastric cancer	Phase II www.actbiotech.com
telotristat etiprate (LX1032)	Lexicon Pharmaceuticals *The Woodlands, TX*	management of symptoms of carcinoid syndrome associated with carcinoid tumor	Phase III www.lexgen.com
TH-302 (hypoxia-activated prodrug)	EMD Serono *Rockland, MA* Threshold Pharmaceuticals *South San Francisco, CA*	treatment of soft tissue sarcoma	Phase III www.emdserono.com www.thresholdpharm.com
ThermoDox® lyso-thermosensitive liposomal doxorubicin	Celsion *Lawrenceville, NJ*	treatment of hepatocellular carcinoma (Fast Track)	Phase III www.celsion.com
Toca 511 & Toca FC combination	Tocagen *San Diego, CA*	treatment of glioblastoma multiforme	Phase I/II www.tocagen.com
trans sodium crocetinate (TSC)	Diffusion Pharmaceuticals *Charlottesville, VA*	treatment of glioblastoma in conjunction with radiotherapy	Phase I/II www.diffusionpharma.com
trebananib (AMG 386)	Amgen *Thousand Oaks, CA*	treatment of ovarian cancer	Phase III www.amgen.com
TVA-Brain-1	TVAX Biomedical *Lenexa, KS*	treatment of primary central nervous system malignancies	Phase II www.tvaxbiomedical.com
Tykerb® lapatinib	GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of ErbB2-positive esophageal cancer --- treatment of ErbB2-positive gastric cancer	Phase III www.gsk.com --- Phase III www.gsk.com
VAL-083	Del Mar Pharmaceuticals *Vancouver, Canada*	treatment of malignant gliomas	Phase I/II www.delmarpharma.com
VB-111	Vascular Biogenics *Or Yehuda, Israel*	treatment of malignant glioma	Phase I/II www.vblrx.com

Cancer

Product Name	Sponsor	Official FDA Designation	Development Status
VB4-845 (recombinant fusion protein)	Viventia Biotech *Winnipeg, Canada*	treatment of Ep-CAM-positive squamous cell carcinoma of the head and neck (Fast Track)	Phase II www.viventia.com
veliparib	AbbVie *North Chicago, IL*	treatment of epithelial ovarian cancer in combination with DNA-damaging agents (see also cancer, skin)	Phase II completed www.abbvie.com
Votrient® pazopanib	GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of ovarian cancer	Phase III www.gsk.com
VS-6063 (defactinib)	Verastem *Cambridge, MA*	treatment of mesothelioma	Phase II www.verastem.com
Y-90 clivatuzumab	Immunomedics *Morris Plains, NJ*	treatment of pancreatic cancer (Fast Track)	Phase II www.immunomedics.com
Yondelis® trabectedin	Janssen Research & Development *Raritan, NJ*	treatment of patients with ovarian cancer --- treatment of soft tissue sarcoma	Phase III www.janssenrnd.com --- Phase III www.janssenrnd.com
Zybrestat™ fosbreyabulin	OXiGENE *South San Francisco, CA*	treatment of ovarian cancer (Fast Track)	Phase II www.oxigene.com

Cancer, Blood

Product Name	Sponsor	Official FDA Designation	Development Status
ACE-536 (recombinant fusion protein)	Acceleron Pharma *Cambridge, MA* Celgene *Summit, NJ*	treatment of myelodysplastic syndromes (see also genetic)	Phase II www.acceleronpharma.com www.celgene.com
Adcetris® brentuximab vedotin	Seattle Genetics *Bothell, WA*	treatment of patients with peripheral T-cell lymphoma, not otherwise specified (Fast Track) (see also cancer, skin)	Phase III www.seattlegenetics.com

Cancer, Blood

Product Name	Sponsor	Official FDA Designation	Development Status
AFM-13 (anti-CD30/CD16A mAb)	Affimed Therapeutics *Heidelberg, Germany*	treatment of Hodgkin lymphoma	Phase I www.affimed.com
alisertib (MLN8237)	Millennium Pharmaceuticals *Cambridge, MA*	treatment of peripheral T-cell lymphoma (see also cancer)	Phase III www.millennium.com
allogeneic mesenchymal precursor cells (stem cell therapy)	Mesoblast *New York, NY*	treatment of insufficient hematopoietic stem cell production in patients with hematologic malignancies who have failed treatment with conventional chemotherapy	Phase III www.mesoblast.com
AT-101 (R-(-)-gossypol)	Ascenta Therapeutics *Malvern, PA*	treatment of chronic lymphocytic leukemia	Phase II www.ascenta.com
AT9183	Astex Therapeutics *Dublin, CA*	treatment of acute myeloid leukemia	Phase I/II completed www.astx.com
belinostat	Spectrum Pharmaceuticals *Henderson, NV*	treatment of peripheral T-cell lymphoma (Fast Track)	Phase II www.sppirx.com
BI-505 (anti-cellular adhesion molecule-1 mAb)	BioInvent International *Lund, Sweden*	treatment of multiple myeloma	Phase I www.bioinvent.com
BiovaxID® dasiprotimut-T	Biovest International *Tampa, FL*	treatment of follicular lymphoma (Fast Track) --- treatment of mantle cell lymphoma	Phase III www.biovest.com --- Phase II www.biovest.com
BL-8040 (CXCR4 chemokine receptor)	BioLineRx *Jerusalem, Israel*	treatment of acute myeloid leukemia	Phase II www.biolinerx.com
blinatumomab	Amgen *Thousand Oaks, CA*	treatment of acute lymphocytic leukemia	Phase II www.amgen.com
BP 100-1-01 (liposomal Grb-2)	Bio-Path *Houston, TX*	treatment of chronic myelogenous leukemia	Phase I www.biopathholdings.com
BT-062 (indatuximab ravtansine)	Biotest Pharmaceuticals *Boca Raton, FL*	treatment of multiple myeloma	Phase I/II www.biotestpharma.com

Cancer, Blood

Product Name	Sponsor	Official FDA Designation	Development Status
CNDO-109 (activated allogeneic natural killer cells)	Coronado Biosciences *Burlington, MA*	treatment of acute myeloid leukemia	Phase I/II www.coronadobiosciences.com
CPX-351 (cytarabine/daunorubicin liposome injection)	Celator Pharmaceuticals *Ewing, NJ*	treatment of acute myeloid leukemia	Phase III www.celatorpharma.com
crenolanib (CP-868-596)	AROG Pharmaceuticals *Dallas, TX*	treatment of acute myelogenous leukemia (see also cancer)	Phase II www.arogpharma.com
Dacogen® decitabine	Eisai *Woodcliff Lake, NJ*	treatment of acute myeloid leukemia	Phase II www.eisai.com
daratumumab	Janssen Research & Development *Raritan, NJ*	treatment of multiple myeloma (Fast Track) (Breakthrough Therapy)	Phase I/II www.janssenrnd.com
darinaparsin (ZIO-101)	Solasia *Tokyo, Japan* ZIOPHARM Oncology *Boston, MA*	treatment of peripheral T-cell lymphoma	Phase I www.ziopharm.com
dinaciclib (MK-7965)	Merck *Whitehouse Station, NJ*	treatment of chronic lymphocytic leukemia	Phase III www.merck.com
elotuzumab	AbbVie *North Chicago, IL* Bristol-Myers Squibb *Princeton, NJ*	treatment of multiple myeloma	Phase III www.abbvie.com www.bms.com
epratuzumab	Immunomedics *Morris Plains, NJ*	treatment of non-Hodgkin lymphoma	Phase II www.immunomedics.com
EPZ-5676 (DOT1L protein inhibitor)	Epizyme *Cambridge, MA*	treatment of acute lymphoblastic leukemia (ALL)	Phase I www.epizyme.com
Estybon™ rigosertib	Onconova Therapeutics *Newton, PA*	treatment of myelodysplastic syndromes (see also cancer)	Phase III www.onconova.com
forodesine (PNP inhibitor)	BioCryst Pharmaceuticals *Durham, NC* Mundipharma *Cambridge, United Kingdom*	treatment of T-cell non-Hodgkin lymphoma	Phase II www.biocryst.com www.mundipharma.com

Cancer, Blood

Product Name	Sponsor	Official FDA Designation	Development Status
ibrutinib	Janssen Biotech *Horsham, PA* Pharmacyclics *Sunnyvale, CA*	treatment of chronic lymphocytic leukemia (Fast Track) (Breakthrough Therapy) --- treatment of mantle cell lymphoma (Breakthrough Therapy) --- treatment of multiple myeloma	application submitted www.janssenbiotech.com www.pharmacyclics.com --- Phase III www.janssenbiotech.com www.pharmacyclics.com --- Phase II www.janssenbiotech.com www.pharmacyclics.com
idelalisib (PI3K delta inhibitor)	Gilead Sciences *Foster City, CA*	treatment of chronic lymphocytic leukemia	Phase III www.gilead.com
IMGN901 (maytansinoid DM1-conjugated mAb)	ImmunoGen *Waltham, MA*	treatment of multiple myeloma (see also cancer)	Phase I www.immunogen.com
inotuzumab ozogamicin (CD22-targeted cytotoxic agent)	Pfizer *New York, NY*	treatment of B-cell acute lymphoblastic leukemia	Phase III www.pfizer.com
interleukin-21 (rIL-21)	Bristol-Myers Squibb *Princeton, NJ*	treatment of stage II (T4), III or IV malignant melanoma	Phase I www.bms.com
IPI-145 (PI3K δ/γ inhibitor)	Infinity Pharmaceuticals *Cambridge, MA*	treatment of follicular lymphoma	Phase II www.infi.com
ISF35 (gene encoding chimeric CD40 ligand)	Memgen *San Diego, CA*	treatment of chronic lymphocytic leukemia	Phase II www.memgen.com
lestaurtinib (CEP-701)	Teva Pharmaceutical *North Wales, PA*	treatment of acute myeloid leukemia	Phase II www.tevapharm.com
midostaurin (PKC412)	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of acute myeloid leukemia (see also other)	Phase III www.novartis.com
milatuzumab	Immunomedics *Morris Plains, NJ*	treatment of chronic lymphocytic leukemia	Phase I/II www.immunomedics.com
MLN4924 (NAE inhibitor)	Millennium Pharmaceuticals *Cambridge, MA*	treatment of acute myelogenous leukemia --- treatment of myelodysplastic syndromes	Phase I www.millennium.com --- Phase I www.millennium.com
MLN9708 (ixazomib citrate)	Millennium Pharmaceuticals *Cambridge, MA*	treatment of multiple myeloma (see also other)	Phase III www.millennium.com

Cancer, Blood

Product Name	Sponsor	Official FDA Designation	Development Status
mocetinostat (MGCD0103)	Mirati Therapeutics *San Diego, CA*	treatment of Hodgkin lymphoma	Phase II www.mirati.com
mogamulizumab	Kyowa Hakko Kirin Pharma *Princeton, NJ*	treatment of patients with cutaneous T-cell lymphoma --- treatment of adult T-cell leukemia/ lymphoma (ATLL) --- treatment of peripheral T-cell lymphoma	Phase III www.kyowa-kirin-pharma.com --- Phase II www.kyowa-kirin-pharma.com --- Phase II www.kyowa-kirin-pharma.com
moxetumomab pasudotox	AstraZeneca *Wilmington, DE* MedImmune *Gaithersburg, MD*	treatment of hairy cell leukemia	Phase III www.astrazeneca.com
NiCord® stem cell therapy	Gamida Cell *Jerusalem, Israel*	for use as hematopoietic support in patients with myelodysplastic syndromes	Phase I/II www.gamida-cell.com
obinutuzumab	Genentech *South San Francisco, CA*	treatment of chronic lymphocytic leukemia (Breakthrough Therapy) --- treatment of diffuse large B-cell lymphoma	application submitted www.gene.com --- Phase III www.gene.com
ocaratuzumab	MENTRIK Biotech *Dallas, TX*	treatment of follicular lymphoma	Phase II www.mentrik.com
otlertuzumab (anti-CD37 mAb) (TRU-016)	Emergent BioSolutions *Rockville, MD*	treatment of chronic lymphocytic leukemia	Phase II www.emergentbiosolutions.com
OXi4503 (combretastatin A 1 diphosphate)	OXiGene *South San Francisco, CA*	treatment of acute myelogenous leukemia	Phase I www.oxigene.com
panobinostat	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of multiple myeloma	Phase III www.novartis.com
plitidepsin (cyclic depsipeptide)	PharmaMar USA *New York, NY*	treatment of multiple myeloma	Phase III www.pharmamar.com

Cancer, Blood

Product Name	Sponsor	Official FDA Designation	Development Status
quizartinib (FLT3 inhibitor)	Ambit Biosciences *San Diego, CA*	treatment of acute myeloid leukemia	Phase II www.ambitbio.com
Revlimid® lenalidomide	Celgene *Summit, NJ*	treatment of chronic lymphocytic leukemia --- treatment of diffuse large B-cell lymphoma --- treatment of follicular lymphoma	Phase III www.celgene.com --- Phase III www.celgene.com --- Phase III www.celgene.com
sapacitabine	Cyclacel *Berkeley Heights, NJ*	treatment of acute myelogenous leukemia --- treatment of myelodysplastic syndromes	Phase III www.cyclacel.com --- Phase II www.cyclacel.com
SHP-141 (HDACi inhibitor)	Shape Pharmaceuticals *Cambridge, MA*	treatment of cutaneous T-cell lymphoma	Phase I www.shapepharma.com
siltuximab	Janssen Research & Development *Raritan, NJ*	treatment of Castleman's disease --- treatment of multiple myeloma	application submitted www.janssenrnd.com --- Phase II www.janssenrnd.com
SL-401 (recombinant fusion protein)	Stemline Therapeutics *New York, NY*	treatment of acute myeloid leukemia	Phase I/II www.stemline.com
SNS01-T (DNA plasmid vector)	Senesco Technologies *Bridgewater, NJ*	treatment of diffuse large B-cell lymphoma --- treatment of mantle cell lymphoma --- treatment of multiple myeloma	Phase I/II www.senesco.com --- Phase I/II www.senesco.com --- Phase I/II www.senesco.com
StemEx® carlecortemcel-L	Gamida Cell *Jerusalem, Israel*	for use as hematopoietic support in patients with relapsed or refractory hematologic malignancies who are receiving high-dose therapy (Fast Track)	Phase III www.gamida-cell.com

Cancer, Blood

Product Name	Sponsor	Official FDA Designation	Development Status
Synribo® omacetaine mepesuccinate	Teva Pharmaceutical *North Wales, PA*	treatment of myelodysplastic syndromes	Phase II www.tevapharm.com
tabalumab	Eli Lilly *Indianapolis, IN*	treatment of multiple myeloma	Phase II/III www.lilly.com
Telintra® ezatiostat	Telik *Palo Alto, CA*	treatment of myelodysplastic syndromes	Phase II www.telik.com
tosedostat	Cell Therapeutics *Seattle, WA* Chroma Therapeutics *Oxon, United Kingdom*	treatment of acute myeloid leukemia	Phase II www.celltherapeutics.com
ublituximab (TG-1101)	TG Therapeutics *New York, NY*	treatment of chronic lymphocytic leukemia --- treatment of nodal marginal zone lymphoma --- treatment of extranodal marginal zone lymphoma (mucosa-associated lymphatic tissue, MALT)	Phase I/II www.tgtherapeutics.com --- Phase /II www.tgtherapeutics.com --- Phase I/II www.tgtherapeutics.com
veltuzumab	Immunomedics *Morris Plains, NJ*	treatment of chronic lymphocytic leukemia	Phase I/II www.immunomedics.com
Vidaza® azacitidine	Celgene *Summit, NJ*	treatment of acute myeloid leukemia	Phase III www.celgene.com
vosaroxin (SNS-595)	Sunesis Pharmaceuticals *South San Francisco, CA*	treatment of acute myeloid leukemia (Fast Track)	Phase III www.sunesis.com
Xalkori® crizotinib	Pfizer *New York, NY*	treatment of anaplastic large-cell lymphoma	Phase I www.pfizer.com

Cancer, Related Conditions

Product Name	Sponsor	Official FDA Designation	Development Status
Captisol-Enabled® melphalan	Spectrum Pharmaceuticals *Henderson, NV*	high dose conditioning treatment prior to hematopoietic progenitor (stem) cell transplantation	Phase II/III www.sppirx.com
E-0316 (naloxone topical)	Elorac *Vernon Hills, IL*	topical treatment of pruritus associated with mycosis fungoides (Fast Track)	Phase II www.eloracpharma.com
FT-1050 (stem cell stimulant)	Fate Therapeutics *San Diego, CA*	enhancement of stem cell engraftment through ex-vivo treatment of human allogeneic hematopoietic stem cells (treatment of neutropenia, thrombocytopenia, lymphopenia, and anemia)	Phase II www.fatetherapeutics.com
GC-4419 (superoxide dismutase mimetic)	Galera Therapeutics *Malvern, PA*	prevention of radiation- or chemotherapy-induced oral mucositis in cancer patients	Phase I completed www.galeratx.com
LG631 (stem cell gene therapy)	Lentigen *Gaithersburg, MD*	for bone marrow protection in the treatment of glioblastoma multiforme	Phase I www.lentigen.com
momelotinib (JAK inhibitor)	Gilead Sciences *Foster City, CA*	treatment of myelofibrosis	Phase II www.gilead.com
Pomalyst® pomalidomide	Celgene *Summit, NJ*	treatment of persons with myelo-proliferative neoplasm-associated myelofibrosis and anemia who are red blood cell tranfusion dependent (see also blood)	Phase III www.celgene.com
sodium thiosulfate (STS)	Adherex Technologies *Rsch. Triangle Park, NC*	prevention of platinum-induced ototoxicity in pediatric patients	Phase III www.adherex.com
Xerecept® corticotropin-releasing factor	Celtic Pharma *Hamilton, Bermuda*	treatment of peritumoral brain edema	Phase III www.celticpharma.com
Xgeva® denosumab	Amgen *Thousand Oaks, CA*	treatment of hypercalcemia of malignancy	Phase II www.amgen.com

Cancer, Skin

Product Name	Sponsor	Official FDA Designation	Development Status
Abraxane® paclitaxel protein-bound particles for injection suspension (albumin-bound)	Celgene *Summit, NJ*	treatment of stage IIB to IV melanoma (see also cancer)	Phase III www.celgene.com
Adcetris® brentuximab vedotin	Seattle Genetics *Bothell, WA*	treatment of mycosis fungoides (see also cancer, blood)	Phase III www.seattlegenetics.com
ADI-PEG20 (pegylated arginine deiminase)	Polaris Pharmaceuticals *San Diego, CA*	treatment of invasive malignant melanoma (see also cancer)	Phase II www.polarispharma.com
astuprotimut-R	GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of MAGE-A3-positive stages IIB to IV malignant melanoma (see also cancer)	Phase III www.gsk.com
Cavatak™ coxsackievirus A21	Viralytics *Sydney, Australia*	treatment of stage II (T4), stage III, and stage IV melanoma	Phase II www.viralytics.com
FANG™ Vaccine autologous tumor cell vaccine	Gradalis *Carrollton, TX*	treatment of stage IIB to IV melanoma (see also cancer)	Phase II www.gradalisinc.com
Marqibo® vincristine liposomal	Spectrum Pharmaceuticals *Henderson, NV*	treatment of metastatic uveal melanoma	Phase II www.sppirx.com
melapuldencel-T (autologous dendritic cell vaccine)	California Stem Cell *Irvine, CA*	treatment of stage IIIB through IV metastatic melanoma	Phase II www.californiastemcell.com
MK-3475 (lambrolizumab)	Merck *Whitehouse Station, NJ*	treatment of stage IIB through IV malignant melanoma (Breakthrough Therapy)	Phase II www.merck.com
nivolumab (anti-PD-1 mAb)	Bristol-Myers Squibb *Princeton, NJ*	treatment of stage IIB to IV melanoma (Fast Track)	Phase III www.bms.com
POL-103A (polyvalent melanoma vaccine)	Polynoma *San Diego, CA*	treatment of stage IIB to stage IV melanoma	Phase III www.polynoma.com
PV-10 (rose bengal disodium)	Provectus Pharmaceuticals *Knoxville, TN*	treatment of metastatic melanoma (see also cancer)	Phase II www.pvct.com
talimogene laherparepvec	Amgen *Thousand Oaks, CA*	treatment of stage IIB-stage IV melanoma	Phase III www.amgen.com
trametinib and dabrafenib	GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of stage IIB through IV melanoma	application submitted www.gsk.com

Cancer, Skin

Product Name	Sponsor	Official FDA Designation	Development Status
veliparib	AbbVie *North Chicago, IL*	treatment of malignant melanoma stages IIB through IV (see also cancer)	Phase II www.abbvie.com

Cardiovascular Diseases

Product Name	Sponsor	Official FDA Designation	Development Status
ixmyelocel-T	Aastrom Biosciences *Ann Arbor, MI*	treatment of dilated cardiomyopathy	Phase II www.aastrom.com
plasmin (human)	Grifols Therapeutics *Los Angeles, CA*	treatment of acute peripheral arterial occlusion	Phase II www.grifolsusa.com
SRM003	Shire *Wayne, PA*	prevention of arteriovenous fistula or arteriovenous graft failure in patients with end-stage renal disease receiving hemodialysis or preparing for hemodialysis (Fast Track)	Phase II www.shire.com
tafamidis meglumine	Pfizer *New York, NY*	treatment of symptomatic transthyretin (TTR) amyloid cardiomyopathy (see also genetic)	Phase III www.pfizer.com

Digestive Diseases

Product Name	Sponsor	Official FDA Designation	Development Status
Aes-210 (clotrimazole)	AesRx *Newton, MA*	topical treatment of children and adults with pouchitis	Phase II www.aesrx.com
alicaforsen	Atlantic Healthcare *Essex, United Kingdom*	treatment of pouchitis (Fast Track)	Phase III www.atlantichc.com
budesonide oral suspension	Meritage Pharma *San Diego, CA*	treatment of patients with eosinophilic esophagitis	Phase II www.meritagepharma.com
EUR-100 (fluticasone propionate)	Aptalis Pharma US *Bridgewater, NJ*	treatment of pediatric and adult eosinophilic esophagitis	Phase I/II www.aptalispharma.com

Digestive Diseases

Product Name	Sponsor	Official FDA Designation	Development Status
Humira® adalimumab	AbbVie *North Chicago, IL*	treatment of pediatric Crohn's disease	Phase III www.abbvie.com
Lialda® mesalamine controlled release	Shire *Wayne, PA*	treatment of ulcerative colitis in pediatric patients	Phase I www.shire.com
metronidazole 10% topical ointment	SLA Pharma *Liestal, Switzerland*	topical treatment of active perianal Crohn's disease	Phase II www.slapharma.com
naltrexone low-dose	TNI BioTech *Bethesda, MD*	treatment of Crohn's disease in pediatric patients	Phase II www.tnibiotech.com
orBec® beclomethasone oral	Soligenix *Princeton, NJ*	treatment of gastrointestinal symptoms with chronic graft versus host disease in patients undergoing allogeneic hematopoietic cell transplantation	Phase I www.soligenix.com
reslizumab	Teva Pharmaceutical *North Wales, PA*	treatment of children with eosinophilic esophagitis	Phase III www.tevapharm.com
SGX203	Soligenix *Princeton, NJ*	treatment of pediatric patients with ulcerative colitis	Phase I www.soligenix.com
Simponi® golimumab	Janssen Biotech *Horsham, PA*	treatment of pediatric ulcerative colitis (see also autoimmune)	Phase I www.janssenbiotech.com
Soliris® eculizumab	Alexion Pharmaceuticals *Cheshire, CT*	treatment of Shiga toxin-producing *Escherichia coli* hemolytic uremic syndrome (see also eye)	Phase II www.alxn.com
Uceris® budesonide	Santarus *San Diego, CA*	treatment of ulcerative colitis in pediatric patients aged 0 through 16 years	Phase II www.santarus.com

Eye Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
CODA-001 (antisense oligonucleotide)	CoDa Therapeutics *San Diego, CA*	treatment of persistent corneal epithelial defects	Phase II www.codatherapeutics.com
EryDex erythrocyte-encapsulated dexamethasone	EryDel *Urbino, Italy*	treatment of ataxia-telangiectasia	Phase I www.erydel.com
gevokizumab (XOMA 052)	XOMA *Berkeley, CA*	treatment of non-infectious intermediate, posterior or pan uveitis, or chronic non-infectious anterior uveitis (see also autoimmune)	Phase III www.xoma.com
LIPO-102 (salmeterol/fluticasone)	Lithera *San Diego, CA*	treatment of symptomatic exophthalmos associated with thyroid related eye disease	Phase II www.lithera.com
Mitosol® mitomycin	Mobius Therapeutics *St. Louis, MO*	prevention of recurrence of pterygium after its surgical excision	application submitted www.mobiustherapeutics.com
NT-501 CNTF (ciliary neurotrophic growth factor)	Neurotech USA *Cumberland, RI*	treatment of retinitis pigmentosa (Fast Track) --- treatment of macular telangiectasia type 2 (MacTel)	Phase II/III www.neurotechusa.com --- Phase I www.neurotechusa.com
plasminogen concentrate (human)	Kedrion *Barga, Italy*	treatment of ligneous conjunctivitis	Phase II/III www.kedrion.com
QLT091001 (synthetic retinoid)	QLT *Menlo Park, CA*	treatment of retinitis pigmentosa (see also genetic)	Phase I www.qltinc.com
QPI-1007 (synthetic double-stranded siRNA)	Quark Pharmaceuticals *Fremont, CA*	treatment of ischemic optic neuropathy	Phase I www.quarkpharma.com
retinal pigment epithelium cell therapy	Advanced Cell Technology *Santa Monica, CA*	treatment of Stargardt's macular dystrophy	Phase I/II www.advancedcell.com
RV001 (teprotumumab)	River Vision *New York, NY*	treatment of active (dynamic) phase Grave's orbitopathy	Phase II
sirolimus opthalmic (DE-109)	Santen *Emeryville, CA*	treatment of chronic/refractory anterior noninfectious uveitis, noninfectious intermediate uveitis, noninfectious panuveitis and non-infectious uveitis affecting the posterior of the eye (NICUPS)	Phase III www.santen.com

Eye Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
Soliris® eculizumab	Alexion Pharmaceuticals *Cheshire, CT*	treatment of neuromyelitis optica (see also digestive)	Phase II www.alxn.com
StarGen™ gene therapy	Sanofi US *Bridgewater, NJ*	treatment of Stargardt disease	Phase I/II www.sanofi.com
UshStat® gene therapy	Sanofi US *Bridgewater, NJ*	treatment of retinitis pigmentosa associated with Usher syndrome 1B gene defect	Phase I/II www.sanofi.com
Vibex™/KXL™ System riboflavin ophthalmic solution	Avedro *Waltham, MA*	treatment of corneal ectasia following refractive surgery --- treatment of keratoconus	application submitted www.avedro.com --- application submitted www.avedro.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
AAV1-FS344 (gene therapy-delivered myostatin inhibitor)	Milo Biotechnology *Cleveland, OH*	treatment of Duchenne and Becker muscular dystrophy	Phase I/II www.milobiotechnology.com
ABH001 human fibroblast-derived dermal substitute	Shire *Wayne, PA*	treatment of epidermolysis bullosa (Fast Track)	Phase III www.shire.com
Abilify® aripiprazole	Otsuka Pharmaceutical *Rockville, MD*	treatment of Tourette's syndrome	Phase III www.otsuka.com
ACE-536 (recombinant fusion protein)	Acceleron Pharma *Cambridge, MA* Celgene *Summit, NJ*	treatment of B-thalassemia (see also cancer, blood)	Phase II www.acceleronpharma.com www.celgene.com
Aeroquin® levofloxacin	Aptalis Pharmaceuticals *Birmingham, AL*	treatment of pulmonary infections due to *Pseudomonas aeruginosa* and other bacteria in patients with cystic fibrosis	Phase III www.aptalispharma.com
Aes-103	AesRx *Newton, MA*	treatment of sickle cell disease	Phase II www.aesrx.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
afamelanotide	Clinuvel Pharmaceuticals *New York, NY*	treatment of erythropoietic porphyrias	Phase III www.clinuvel.com
ALN-TTR02/GENZ438027 (siRNA oligonucleotide)	Alnylam Pharmaceuticals *Cambridge, MA* Sanofi US (Genzyme) *Bridgewater, NJ*	treatment of familial amyloidotic polyneuropathy	Phase II www.alnylam.com www.sanofi.com
alpha1-proteinase inhibitor (human)	Grifols Therapeutics *Los Angeles, CA*	treatment of cystic fibrosis	Phase II www.grifols.com
Alprolix™ recombinant factor IX fusion protein	Biogen Idec *Weston, MA*	for the control and prevention of hemorrhagic episodes in patients with hemophilia B (congenital factor IX deficiency or Christmas disease) (Fast Track)	application submitted www.biogenidec.com
ALXN1101 (cPMP replacement therapy)	Alexion Pharmaceuticals *Cheshire, CT*	treatment of molybdenum cofactor deficiency type A (MoCD)	Phase I www.alxn.com
AMG 145 (evolocumab)	Amgen *Thousand Oaks, CA*	treatment of homozygous familial hypercholesterolemia	Phase III www.amgen.com
AMT060 (factor IX gene therapy)	uniQure *Amsterdam, Netherlands*	treatment of hemophilia B	Phase I/II www.uniqure.com
arbaclofen (STX209)	Seaside Therapeutics *Cambridge, MA*	treatment of the behavioral abnormalities associated with fragile X syndrome	Phase III www.seasidetherapeutics.com
Arikace® liposomal amikacin for inhalation	Insmed *Monmouth Junction, NJ*	treatment of bronchopulmonary *Pseudomonas aeruginosa* infections in cystic fibrosis patients (see also infectious)	Phase III www.insmed.com
asfotase alfa	Alexion Pharmaceuticals *Cheshire, CT*	treatment of hypophosphatasia (Fast Track) (Breakthrough Therapy)	Phase II/III www.alxn.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
ataluren (PTC124)	PTC Therapeutics *South Plainfield, NJ*	treatment of muscular dystrophy resulting from premature stop mutations in the dystrophin gene (Fast Track) --- for use in the treatment of cystic fibrosis resulting from a nonsense (premature stopcodon) mutation in the cystic fibrosis transmembrane conductance regulatory gene (Fast Track)	Phase III www.ptcbio.com --- Phase III www.ptcbio.com
BAX111 (rhVWF)	Baxter International *Deerfield, IL*	treatment of von Willebrand disease	Phase III www.baxter.com
beloranib	Zafgen *Cambridge, MA*	treatment of Prader-Willi syndrome	Phase II www.zafgen.com
BMN-701 (IGF2-GAA)	BioMarin Pharmaceutical *San Rafael, CA*	treatment of Pompe disease	Phase II www.bmrn.com
Bronchitol® mannitol inhalation	Pharmaxis *Exton, PA*	for use to facilitate clearance of mucus in patients with bronchiectasis and in patients with cystic fibrosis at risk for bronchiectasis (Fast Track)	application submitted www.pharmaxis.com
CEQ508 (RNA interference)	Marina Biotech *Bothell, WA*	treatment of familial adenomatous polyposis	Phase I/II www.marinabio.com
ciprofloxacin dry powder inhalation (DPI)	Bayer HealthCare Pharmaceuticals *Wayne, NJ* Novartis Pharmaceuticals *East Hanover, NJ*	management of pulmonary infection due to *Pseudomonas aeruginosa* in cystic fibrosis patients	Phase II www.bayerpharma.com www.novartis.com
CPP-IX/sul (eflornithine plus sulindac)	Cancer Prevention Pharmaceuticals *Tucson, AZ*	treatment of familial adenomatous polyposis	Phase II www.canprevent.com
CSL654 (rIX-FP)	CSL Behring *King of Prussia, PA*	treatment of patients with congenital factor IX deficiency (hemophilia B)	Phase II/III www.cslbehring.com
CSL689 (rVIIa-FP)	CSL Behring *King of Prussia, PA*	treatment and prophylaxis of bleeding episodes in patients with congenital hemophilia and inhibitors to coagulation factor VIII or IX --- treatment of congenital factor VII deficiency which includes treatment and prophylaxis of bleeding episodes in patients with congenital factor VII deficiency	Phase I www.cslbehring.com --- Phase I www.cslbehring.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
drisapersen (GSK2402968)	GlaxoSmithKline *Rsch. Triangle Park, NC* Prosensa Therapeutics *Leiden, Switzerland*	treatment of Duchenne muscular dystrophy (Breakthrough Therapy)	Phase III www.gsk.com
duvoglustat (AT2220)	Amicus Therapeutics *Cranbury, NJ*	treatment of Pompe disease	Phase II www.amicusrx.com
ecopipam (PSYRX101)	Psyadon Pharmaceuticals *Germantown, MD*	symptomatic treatment of self injurious behaviors in patients with Lesch-Nyhan disease ---------- treatment of Tourette's syndrome in children 0-16 years old	Phase III www.psyadonrx.com ---------- Phase II www.psyadonrx.com
EDI1200	Edimer Pharmaceuticals *Cambridge, MA*	treatment of X-linked hypohidrotic ectodermal dysplasia (Fast Track)	Phase II www.edimerpharma.com
eliglustat tartrate (glucosylceramide synthase inhibitor)	Sanofi US (Genzyme) *Bridgewater, NJ*	treatment of Gaucher disease	Phase III www.sanofi.com
Eloctate™ recombinant human factor VIII-FC	Biogen Idec *Weston, MA*	treatment of hemophilia A (Fast Track)	application submitted www.biogenidec.com
EPI-743 (vatiquinone)	Dainippon Sumitomo Pharma *Osaka, Japan* Edison Pharmaceuticals *Mountain View, CA*	treatment of inherited mitochondrial respiratory chain diseases	Phase II/III www.ds-pharma.com www.edisonpharma.com
eteplirsen (antisense oligonucleotide)	Sarepta Therapeutics *Cambridge, MA*	treatment of Duchenne muscular dystrophy (Fast Track)	Phase II www.sareptatherapeutics.com
EXR-101 (pyrimethamine)	ExSAR *Monmouth Junction, NJ*	treatment of GM-2 gangliosidoses (Tay-Sachs disease and Sandhoff disease)	Phase II www.exsar.com
EXR-202 (ambroxol)	ExSAR *Monmouth Junction, NJ*	treatment of Gaucher disease	Phase I www.exsar.com
GNE lipoplex	Gradalis *Carrollton, TX*	treatment of hereditary inclusion body myopathy-2	Phase I www.gradalisinc.com
GSK2696273 (ex-vivo stem cell gene therapy)	GlaxoSmithKline *Rsch. Triangle Park, NC* MolMed *Milan, Italy*	treatment of severe combined immunodeficiency due to adenosine deaminase deficiency	Phase III www.gsk.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
GZ402665 (rhASM)	Sanofi US (Genzyme) *Bridgewater, NJ*	treatment of acid sphingomyelinase deficiency (Niemann-Pick disease)	Phase I www.sanofi.com
halofuginone hydrobromide	Halo Therapeutics *Newton, MA*	treatment of Duchenne muscular dystrophy	Phase I/II www.halotherapeutics.com
HGT1110 (cerebroside sulfatase)	Shire *Wayne, PA*	treatment of metachromatic leukodystrophy	Phase I/II www.shire.com
HGT1410 (sulfamidase enzyme replacement therapy)	Shire *Wayne, PA*	for treatment of Sanfilippo syndrome (MPS IIIA)	Phase I/II www.shire.com
HQK-1001	HemaQuest Pharmaceuticals *San Diego, CA*	treatment of beta thalassemia --- treatment of sickle cell disease	Phase II www.hemaquest.com --- Phase II www.hemaquest.com
human-cl rhFVIII	Octapharma USA *Hoboken, NJ*	immune tolerance induction in hemophilia A patients with inhibitors	Phase III www.octapharma.com
human coagulation factor X	Bio Products Laboratory *Herst, United Kingdom*	treatment of hereditary factor X deficiency	Phase III www.bpl.co.uk
idebenone	Santhera Pharmaceuticals *Liestal, Switzerland*	treatment of Duchenne muscular dystrophy --- treatment of mitochondrial myopathy, encephalopathy, lactic acidosis with stroke-like episodes syndrome (MELAS)	Phase III www.santhera.com --- Phase II www.santhera.com
ISIS-TTRRX (antisense oligonucleotides)	Isis Pharmaceuticals *Carlsbad, CA*	treatment of familial amyloid polyneuropathy (Fast Track)	Phase III www.isispharm.com
L-glutamine	Emmaus Medical *Torrance, CA*	treatment of sickle cell disease (Fast Track)	Phase III www.emmausmedical.com
LCI699 (aldosterone synthase inhibitor)	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of Cushing's disease	Phase II www.novartis.com
LentiD® gene therapy	bluebird bio *Cambridge, MA*	treatment of andrenoleukodystrophy	Phase II/III www.bluebirdbio.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
LentiGlobin® gene therapy	bluebird bio *Cambridge, MA*	treatment of B-thalassemia major and intermediate	Phase I/II www.bluebirdbio.com
LUM001 (sodium bile acid cotransporter inhibitor)	Lumena Pharmaceuticals *San Diego, CA*	treatment of alagille syndrome --- treatment of primary biliary cirrhosis --- treatment of primary sclerosing cholangitis --- treatment of progresssive familial intrahepatic cholestasis	Phase II www.lumenapharma.com --- Phase II www.lumenapharma.com --- Phase I www.lumenapharma.com --- Phase I www.lumenapharma.com
mavoglurant (AFQ056)	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of fragile X syndrome	Phase II/III www.novartis.com
migalastat	Amicus Therapeutics *Cranbury, NJ* GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of Fabry disease	Phase III www.amicusrx.com www.gsk.com
Mirapex® pramipexole	Boehringer-Ingelheim Pharmaceuticals *Ridgefield, CT*	treatment of Tourette syndrome in pediatric patients	Phase III completed www.boehringer-ingelheim.com
MST-188 (purified poloxamer 188)	Mast Therapeutics *San Diego, CA*	treatment of sickle cell anemia (Fast Track)	Phase III www.masttherapeutics.com
NKTT120 (rhIgG1k mAb)	NKT Therapeutics *Waltham, MA*	treatment of sickle cell disease	Phase I www.nktrx.com
NN7999 (N9-GP)	Novo Nordisk *Princeton, NJ*	routine prophylactic administration for prevention of bleeding in patients with hemophilia B (Christmas disease)	Phase III www.novonordisk.com
NovoThirteen® catridecacog (rFXIII)	Novo Nordisk *Princeton, NJ*	for the prevention of bleeding associated with congenital FXIII deficiency --- treatment of congenital FXIII deficiency	application submitted www.novonordisk.com --- application submitted www.novonordisk.com
OB-1 (recombinant porcine factor VIII)	Baxter International *Deerfield, IL*	treatment and prevention of episodic bleeding in patients with inhibitor antibodies to human coagulation factor VIII (Fast Track)	Phase III www.baxter.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase)	BioMarin Pharmaceutical *San Rafael, CA*	treatment of hyperphenylalaninemia	Phase III www.bmrn.com
pegylated carboxyhemoglobin	Sangart *San Diego, CA*	treatment of acute painful sickling crises in patients with sickle cell disease	Phase I www.sangart.com
PF-05280602 (rhFVIIa)	Catalyst Biosciences *South San Francisco, CA* Pfizer *New York, NY*	routine prophylaxis to prevent bleeding episodes in patients with hemophilia A and B patients with inhibitors	Phase I www.catalystbiosciences.com www.pfizer.com
pradigastat (LCQ908)	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of hypertriglyceridemia in the setting of type I hyperlipoproteinemia, also known as familial chylomicronemia syndrome	Phase III www.novartis.com
pridopidine	Teva Pharmaceutical *North Wales, PA*	treatment of Huntington's disease	Phase II/III www.tevapharm.com
QLT091001 (synthetic retinoid)	QLT *Menlo Park, CA*	treatment of Leber congenital amaurosis (LCA) due to inherited mutations in RPE65 (encoding the protein retinal pigment epithelial protein 65) or LRAT (encoding the enzyme lecithin:retinol acyltransferase) genes (see also eye)	Phase I www.qltinc.com
rAAV1-CB-hAAT (adeno-associated virus vector-mediated gene therapy)	AGTC *Alachua, FL*	treatment of alpha1-antitrypsin deficiency	Phase II www.agtc.com
rAAV2-CB-hRPE65 (gene therapy)	AGTC *Alachua, FL*	treatment of type II Leber's congenital amaurosis	Phase II www.agtc.com
RG2833 (HDAC3 inhibitor)	Repligen *Waltham, MA*	treatment of Friedreich's ataxia	Phase I www.repligen.com
RG7090	Roche *Nutley, NJ*	treatment of fragile X syndrome	Phase II www.roche.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
RGN-137 topical (thymosin beta 4)	RegeneRx Biopharmaceuticals *Rockville, MD*	treatment of epidermolysis bullosa	Phase II completed www.regenerx.com
rivipansel (GMI-1070)	GlycoMimetics *Gaithersburg, MD* Pfizer *New York, NY*	treatment of vaso-occlusive crisis in patients with sickle cell disease (Fast Track)	Phase II www.glycomimetics.com www.pfizer.com
Ruconest® conestat alfa	Santarus *San Diego, CA*	treatment of (acute attacks of) angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency --- prophylactic treatment of angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency	application submitted www.santarus.com --- Phase II www.santarus.com
SD-101 (allantoin)	Scioderm *Durham, NC*	treatment of skin blistering and erosions associated with inherited epidermolysis bullosa (Breakthrough Therapy)	Phase II www.sderm.com
sebelipase alfa	Synageva BioPharma *Lexington, MA*	treatment of lysosomal acid lipase deficiency (Fast Track)	Phase III www.synageva.com
SelG2 (humanized IgG2 antibody)	Selexys Pharmaceuticals *Oklahoma City, OK*	treatment of vaso-occlusive crisis in patients with sickle cell disease	Phase II www.selexys.com
S-nitroglutathione (N6022)	N30 Pharmaceuticals *Boulder, CO*	management of cystic fibrosis patients to improve airway clearance and to improve or stabilize pulmonary function	Phase I www.n30pharma.com
Spiriva® HandiHaler® tiotropium bromide	Boehringer Ingelheim Pharmaceuticals *Ridgefield, CT*	to improve pulmonary function in conjunction with standard therapy in the management of patients with cystic fibrosis	Phase III www.boehringer-ingelheim.com
tafamidis meglumine	Pfizer *New York, NY*	treatment of familial amyloid polyneuropathy (see also cardiovascular)	application submitted www.pfizer.com
TD-101 (small interfering RNA)	TransDerm *Santa Cruz, CA*	treatment of pachyonychia congenita	Phase I www.transderm.com

Genetic Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
UX001 (sailic acid extended release)	Ultragenyx Pharmaceutical *Novato, CA*	treatment of hereditary inclusion body myopathy	Phase II www.ultragenyx.com
Vimizim™ elosulfase alfa	BioMarin Pharmaceutical *Novato, CA*	treatment of mucopolysacharidosis type IV A (Morquio A syndrome)	application submitted www.bmrn.com
VX-809/ivacaftor	Vertex Pharmaceuticals *Cambridge, MA*	treatment of cystic fibrosis (Fast Track) (Breakthrough Therapy)	Phase III www.vrtx.com
Xenobilox® chenodeoxycholic acid	Sigma-Tau Pharmaceuticals *Gaithersburg, MD*	treatment of cerebrotendinous xanthomatosis	application submitted www.sigmatau.com
Zavesca® miglustat	Actelion Pharmaceuticals *South San Francisco, CA*	treatment of the neurological manifestations of Niemann-Pick disease, type C	application submitted www.actelion.com

Growth Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
BMN-111 (modified recombinant human C-type natriuretic peptide)	BioMarin Pharmaceutical *San Rafael, CA*	treatment of achondroplasia	Phase I www.bmrn.com
macimorelin acetate (AEZS-130)	AEterna Zentaris *South San Francisco, CA*	diagnosis of growth hormone deficiency	Phase III www.aezsinc.com
MOD-4023 (hGH-CTP)	PROLOR Biotech *Ness Ziona, Israel*	treatment of growth hormone deficiency	Phase III www.prolor-biotech.com
octreotide (oral)	Chiasma *Jerusalem, Israel*	for the oral treatment of acromegaly	Phase I www.chiasmapharma.com
Signifor® LAR pasireotide	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of acromegaly	Phase III www.novartis.com
testosterone undecanoate (oral)	SOV Therapeutics *Morrisville, NC*	treatment of constitutional delay in growth and puberty in adolescent boys (14-17 yrs of age)	Phase II www.sovtherapeutics.com

Growth Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
triptorelin pamoate (Debio 8206 CPP)	Debiopharm *Lausanne, Switzerland*	treatment of central precocious puberty	Phase III www.debiopharm.com

Infectious Diseases

Product Name	Sponsor	Official FDA Designation	Development Status
AB-103 (synthetic peptide)	Atox Bio *Ness Ziona, Israel* Fast Track Drugs and Biologics *North Potomac, MD*	treatment of necrotizing soft tissue infections (NSTI) (Fast Track)	Phase II www.atoxbio.com
anthrax immune globulin (human)	Cangene *Winnipeg, Canada*	treatment of toxemia associated with inhalational anthrax	Phase III www.cangene.com
Anthrivig™ anthrax immune globulin	Emergent Biosolutions *Rockville, MD*	treatment of inhalation anthrax disease (Fast Track)	Phase III www.emergentbiosolutions.com
Arestvyr™ tecovirimat	SIGA *New York, NY*	treatment of orthopoxvirus infections (Fast Track)	Phase II www.siga.com
AriKace® liposomal amikacin for inhalation	Insmed *Monmouth Junction, NJ*	treatment of bronchiectasis in patients with *Pseudomonas aeruginosa* or other susceptible microbial pathogens (see also genetic) --- treatment of infections caused by non-tuberculous mycobacteria (Fast Track)	Phase II completed www.insmed.com --- Phase II www.insmed.com
ASP0113 (cytomegalovirus DNA vaccine) (VCL-CB01)	Astellas Pharma US *Northbrook, IL*	prevention of clinically significant cytomegalovirus (CMV) viremia, CMV disease and associated complications in at-risk hematopoietic cell transplant populations --- prevention of clinically significant cytomegalovirus (CMV) viremia, CMV disease and associated complications in solid transplant populations	Phase III www.astellas.com --- Phase II www.astellas.com

Infectious Diseases

Product Name	Sponsor	Official FDA Designation	Development Status
AVI-7288 (antisense oligonucleotide)	Sarepta Therapeutics *Cambridge, MA*	prophylaxis following documented or suspected exposure to Marburg virus (Fast Track)	Phase I www.sareptatherapeutics.com
Cayston® aztreonam	Gilead Sciences *Foster City, CA*	improvement of respiratory symptoms in patients with bronchiectasis and gram-negative bacteria in the airways	Phase III completed www.gilead.com
Civacir® hepatitis C virus immune globulin (human)	Biotest Pharmaceuticals *Boca Raton, FL*	prophylaxis of hepatitis C infection in liver transplant recipients	Phase III www.biotestpharma.com
delamanid (OPC-67683)	Otsuka Pharmaceutical *Rockville, MD*	treatment of pulmonary tuberculosis	Phase III www.otsuka.com
Dificid® fidaxomicin	Optimer Pharmaceuticals *Jersey City, NJ*	treatment of pediatric *Clostridium difficile* infection	Phase II www.optimerpharma.com
ETI-204 (recombinant chimeric mAb)	Elusys Therapeutics *Pine Brook, NJ*	treatment of exposure to *B. anthracis* spores (Fast Track)	Phase I www.elusys.com
isavuconazole	Astellas Pharma US *Northbrook, IL* Basilea Pharmaceutica *Basel, Switzerland*	treatment of invasive aspergillosis	Phase III www.astellas.com
maribavir	ViroPharma *Exton, PA*	treatment of clinically significant cytomegalovirus viremia and disease in at-risk patients (Fast Track)	Phase II www.viropharma.com
MBX-400/cyclopropavir (nucleoside DNA polymerase inhibitor)	Microbiotix *Worcester, MA*	treatment of active cytomegalovirus infections	Phase I www.microbiotix.com
miltefosine	Paladin Therapeutics *St-Laurant, Canada*	treatment of leishmaniasis	application submitted www.paladin-labs.com
MK-8228 (letermovir)	Merck *Whitehouse Station, NJ*	prevention of human cytomegalovirus viremia and disease in at-risk populations (Fast Track)	Phase II www.merck.com
Nabi-HB® hepatitis B immune globulin (human)	Biotest Pharmaceuticals *Boca Raton, FL*	prophylaxis against hepatitis B virus reinfection liver transplant patients	application submitted www.biotestpharma.com

Infectious Diseases

Product Name	Sponsor	Official FDA Designation	Development Status
nifurtimox	Bayer HealthCare Pharmaceuticals *Wayne, NJ*	treatment of Chagas disease (American typanosomiasis) caused by *T. cruzi*	Phase I www.bayerpharma.com
Nuartez™ artesunate injection	Sigma-Tau Pharmaceuticals *Gaithersburg, MD* US Army Medical Materiel Development Activity *Ft. Detrick, MD*	immediate treatment of malaria	Phase III completed www.sigmatau.com
RiVax™ ricin toxin pre-exposure vaccine	Soligenix *Princeton, NJ*	prevention of ricin intoxication	Phase I www.soligenix.com
SQ109 (ethylenediamine)	Sequella *Rockville, MD*	treatment of tuberculosis (Fast Track)	Phase II www.sequella.com
sutezolid	Sequella *Rockville, MD*	treatment of tuberculosis	Phase II www.sequella.com
tafenoquine (8-aminoquinoline)	GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of malaria	Phase II www.gsk.com
TCN-202 (mAb)	Theraclone Sciences *Seattle, WA*	prevention of congenital cytomegalovirus (CMV) infection following primary CMV infection in pregnant women	Phase I www.theraclone-sciences.com
Thravixa™ human anthrax mAb	Emergent BioSolutions *Rockville, MD*	for post-exposure prophylaxis and treatment of inhalation anthrax	Phase I www.emergentbiosolutions.com
Valtorim® anthrax anti-toxin mAb	PharmAthene *Annapolis, MD*	treatment of anthrax infection (Fast Track)	Phase I www.pharmathene.com
vancomycin inhalation powder	Savara Pharmaceuticals *Austin, TX*	treatment of persistent methicillin-resistant *S. aureus* lung infection in patients with cystic fibrosis	Phase II www.savarapharma.com

Neurological Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
ADX-N05 (neurotransmitter modulator)	Aerial BioPharma *Morrisville, NC*	treatment of narcolepsy	Phase II www.aerialbio.com
AmiKet™ amitriptyline/ketamine	Immune Pharmaceuticals *Tarrytown, NY*	treatment of postherpetic neuralgia	Phase II completed www.immunepharmaceuticals.com

Neurological Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
arimoclomol	ALS Association *Richmond, VA* FDA Office of Orphan Products Development *Silver Spring, MD* University of Miami *Miami, FL*	treatment of amyotrophic lateral sclerosis	Phase II/III www.alsa.org
ATI355 (anti-Nogo-A mAb)	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of acute spinal cord injury	Phase I www.novartis.com
autologous bone marrow-derived mesenchymal stem cells	TCA Cellular Therapy *Covington, LA*	treatment of amyotrophic lateral sclerosis	Phase I www.tcacellulartherapy.com
BA-210 (recombinant fusion protein)	BioAxone BioSciences *Cambridge, MA*	treatment of acute spinal cord injury (Fast Track)	Phase II www.bioaxonebio.com
Captisol-enabled™ topiramate injection	CURx Pharmaceuticals *San Diego, CA* Ligand Pharmaceuticals *La Jolla, CA*	treatment of partial onset or primary generalized tonic-clonic seizures for hospitalized epilepsy patients who are unable to take oral topiramate	Phase I www.ligand.com
Circadin® melatonin controlled-release	Neurim Pharmaceuticals *Tel-Aviv, Israel*	treatment of non-24-hour sleep-wake disorder in blind individuals without light perception	Phase III www.neurim.com
civamide	Winston Laboratories *Vernon Hills, IL*	treatment of postherpetic neuralgia --- treatment of postherpetic neuralgia of the trigeminal nerve	Phase III www.winstonlabs.com --- Phase II www.winstonlabs.com
CPP-115 (GABA-AT inhibitor)	Catalyst Pharmaceutical Partners *Coral Gables, FL*	treatment of infantile spasms	Phase I www.catalystpharma.com
diazepam intranasal spray	Acorda Therapeutics *Ardsley, NY*	management of patients with acute repetitive seizures	Phase III www.acorda.com
Duopa® levodopa/carbidopa intranuodenal	AbbVie *North Chicago, IL*	treatment of late-stage Parkinson's disease (Fast Track)	application submitted www.abbvie.com
Eladur™ bupivacaine transdermal	DURECT *Cupertino, CA*	relief of persistent pain associated with postherpetic neuralgia	Phase II www.durect.com
ganaxolone	Marinus Pharmaceuticals *New Haven, CT*	treatment of infantile spasms	Phase II www.marinuspharma.com

Neurological Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
H.P. Acthar® Gel repository corticotropin injection subcutaneous	Questcor Pharmaceuticals *Anaheim Hills, CA*	treatment of amyotrophic lateral sclerosis	Phase II www.questcor.com
intravenous carbamazepine	Lundbeck *Deerfield, IL*	treatment of epilepsy patients who cannot take anything by mouth (NPO)	Phase III www.lundbeck.com
ISIS-SMNRX (antisense oligonucleotide)	Biogen Idec *Weston, MA* Isis Pharmaceuticals *Carlsbad, CA*	treatment of spinal muscular atrophy	Phase II www.biogenidec.com www.isispharm.com
midazolam intranasal (USL-261)	Upsher-Smith Laboratories *Maple Grove, MN*	rescue treatment of seizures in patients who require control of intermittent bouts of increased seizure activity (e.g., acute repetitive seizures, seizure clusters) (Fast Track)	Phase III www.upsher-smith.com
NH001 (apomorphine subcutaneous)	NeuroHealing Pharmaceuticals *Waban, MA*	for the treatment of patients in a vegetative state orminimally conscious state for up to 12 months following a severe traumatic brain injury (traumatic or spontaneous) (Fast Track)	Phase II www.neurohealing.com
Northera® droxidopa	Chelsea Therapeutics *Charlotte, NC*	treatment of neurogenic symptomatic orthostatic hypotension in patients with primary autonomic failure (Parkinson's disease, multiple system atrophy, and pure autonomic failure), dopamine-beta-hydroxylase deficiency, and nondiabetic autonomic neuropathy (Fast Track)	application submitted www.chelseatherapeutics.com
NP001	Neuraltus Pharmaceuticals *Palo Alto, CA*	for slowing the progression of amyotrophic lateral sclerosis	Phase II www.neuraltus.com
NSI-566 (human spinal cord-derived neural stem cells)	Neuralstem *Rockville, MD*	treatment of amyotrophic lateral sclerosis	Phase II www.neuralstem.com
NurOwn™ (GDNF-producing stem cell therapy)	BrainStorm Cell Therapeutics *New York, NY*	treatment of amyotrophic lateral sclerosis	Phase II www.brainstormcell.com
ozanezumab (anti-nogo-A mAb)	GlaxoSmithKline *Rsch. Triangle Park, NC*	treatment of amyotrophic lateral sclerosis	Phase II www.gsk.com

Neurological Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
PF-06687859 (mRNA decapping enzyme inhibitor)	Pfizer *New York, NY*	treatment of spinal muscular atrophy (Fast Track)	Phase I www.pfizer.com
progesterone infusion (BHR-100)	BHR Pharma *Herndon, VA*	for early intervention in the treatment of moderate to severe closed-head traumatic brain injury (Fast Track)	Phase III www.bhrpharma.com
RP103 (cysteamine)	Raptor Therapeutics *Novato, CA*	treatment of Huntington's disease	Phase II/III www.raptorpharma.com
RTL-1000 (recombinant T-cell receptor ligand)	Artielle ImmunoTherapeutics *San Mateo, CA*	treatment of multiple sclerosis patients who are both HLA-DR2-positive and autoreactive to myelin oligodendrocyte glycoprotein residues 35-57	Phase I completed www.artielle.com
SEN196 (SIRT-1 inhibitor)	Siena Biotech *Siena, Italy*	treatment of Huntington's disease	Phase I www.sienabiotech.com
tasimelteon	Vanda Pharmaceuticals *Washington, DC*	treatment of non-24 hour sleep/wake disorder in blind individuals without light perception	application submitted www.vandapharma.com
tirasemtiv	Cytokinetics *South San Francisco, CA*	treatment of amyotrophic lateral sclerosis (ALS) (Fast Track)	Phase II www.cytokinetics.com
Vanquix® diazepam auto-injector	Pfizer *New York, NY*	management of selected, refractory patients with epilepsy on stable regimens of antiepileptic drugs, who require intermittent use of diazepam to control bouts of increased seizure activity	Phase III www.pfizer.com

Respiratory Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
Adempas™ riociguat	Bayer HealthCare Pharmaceuticals *Wayne, NJ*	treatment of pulmonary arterial hypertension	application submitted www.bayerpharma.com
AIR001 (sodium nitrite)	Aires Pharmaceuticals *San Diego, CA*	treatment of pulmonary arterial hypertension	Phase II www.airespharma.com

Respiratory Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
ARD-3150 (liposomal ciprofloxacin)	Aradigm *Hayward, CA*	management of bronchiectasis	Phase III www.aradigm.com
aviptadil (vasoactive intestinal peptide)	THERAMetrics *Stans, Switzerland*	treatment of acute respiratory distress syndrome	Phase I www.therametrics.com
beraprost 314d	United Therapeutics *Silver Spring, MD*	treatment of pulmonary arterial hypertension	Phase III www.unither.com
BMS-986202 (LPA1 receptor antagonist)	Bristol-Myers Squibb *Princeton, NJ*	treatment of idiopathic pulmonary fibrosis	Phase II www.bms.com
Esbriet® pirfenidone	InterMune *Brisbane, CA*	treatment of idiopathic pulmonary fibrosis (Fast Track)	Phase III www.intermune.com
FG-3019 (CTGF mAb inhibitor)	FibroGen *San Francisco, CA*	treatment of idiopathic pulmonary fibrosis	Phase II www.fibrogen.com
GeNosyl™ nitric oxide	GeNO *Waltham, MA*	treatment of persistent pulmonary hypertension in newborns	application submitted www.genollc.com
INOmax® nitric oxide inhalation	Ikaria *Hampton, NJ*	to reduce the risk of chronic lung disease in premature neonates --- treatment of pulmonary arterial hypertension	Phase III www.ikaria.com --- Phase I www.ikaria.com
IW001 (purified bovine type V collage)	ImmuneWorks *Indianapolis, IN*	treatment of idiopathic pulmonary fibrosis	Phase I www.immuneworks.com
nintedanib (triple kinase inhibitor)	Boehringer Ingelheim Pharmaceuticals *Ridgefield, CT*	treatment of patients with idiopathic pulmonary fibrosis	Phase III www.boehringer-ingelheim.com
Opsumit® macitentan	Actelion Pharmaceuticals *South San Francisco, CA*	treatment of pulmonary arterial hypertension	application submitted www.actelion.com
PRM-151 (recombinant human pentraxin-2 protein)	Promedior *Lexington, MA*	treatment of idiopathic pulmonary fibrosis	Phase II www.promedior.com
SAR156597 (IL4/IL13 bi-specific antibody)	Sanofi US *Bridgewater, NJ*	treatment of idiopathic pulmonary fibrosis	Phase II www.sanofi.com
selexipag	Actelion Pharmaceuticals *South San Francisco, CA*	treatment of pulmonary arterial hypertension	Phase III www.actelion.com

Respiratory Disorders

Product Name	Sponsor	Official FDA Designation	Development Status
simtuzumab (anti-LOXL2 mAb)	Gilead Sciences *Foster City, CA*	treatment of idiopathic pulmonary fibrosis (see also blood, cancer)	Phase II www.gilead.com
STX-100 (anti-integrin alphaVbeta6 mAb)	Biogen Idec *Weston, MA*	treatment of idiopathic pulmonary fibrosis	Phase II www.biogenidec.com
Surfaxin® lucinactant	Discovery Laboratories *Warrington, PA*	treatment of acute respiratory distress syndrome in adults (Fast Track)	Phase II completed www.discoverylabs.com
tralokinumab (anti-interleukin-13 mAb)	AstraZeneca *Wilmington, DE* MedImmune *Gaithersburg, MD*	treatment of idiopathic pulmonary fibrosis	Phase II www.astrazeneca.com

Skin

Product Name	Sponsor	Official FDA Designation	Development Status
Debrase® bromelain topical	MediWound *Yvane, Israel*	debridement of acute, deep dermal burns in hospitalized patients	Phase II completed www.mediwound.com
StrataGraft™ skin replacement therapy	Stratatech *Madison, WI*	treatment of hospitalized patients with complex skin defects resulting from partial and full thickness skin burns requiring excision and grafting	Phase I/II www.stratatechcorp.com

Transplantation

Product Name	Sponsor	Official FDA Designation	Development Status
anti-T-lymphocyte immune globulin	Fresenius Biotech *Waltham, MA*	prevention of graft versus host disease (GVHD)	Phase III www.fresenius-biotech.com
ASC-101	America Stem Cell *Floresville, TX*	to improve homing to bone (treatment of myeloablation) in patients receiving hematopoietic stem cell transplantation	Phase I/II www.americastemcell.com

Transplantation

Product Name	Sponsor	Official FDA Designation	Development Status
BB3 (HGF mimetic)	Angion Biomedica *Uniondale, NY*	to improve renal function and prevent delayed graft function following renal transplantation (Fast Track)	Phase II www.angion.com
emricasan	Conatus Pharmaceuticals *San Diego, CA*	treatment of patients undergoing solid organ transplantation (hepatic fibrosis)	Phase II www.conatuspharma.com
HSV-TK cell therapy	MolMed *Milan, Italy*	immunotherapy for acceleration of T-cell reconstitution in patients undergoing allogeneic hematopoietic stem cell transplantation	Phase III www.molmed.com
humanized IgG4 mAb (OPN-305)	Opsona Therapeutics *Dublin, Ireland*	prevention of ischemia/reperfusion injury associated with solid organ transplantation	Phase II www.opsona.com
liposomal alpha galactosylceramide (RGI-2001)	REGiMMUNE *Santa Clara, CA*	prevention of graft versus host disease	Phase I/II www.regimmune.com
Multistem® stem cell therapy	Athersys *Cleveland, OH*	prophylaxis of graft versus host disease	Phase I www.athersys.com
NX001	NephRx *Kalamazoo, MI*	treatment of delayed graft function in renal transplant recipients --- prevention of delayed graft function in renal transplant recipients	Phase I www.nephrx.com --- Phase I www.nephrx.com
Prochymal® remestemcel-L	Osiris Therapeutics *Columbia, MD*	treatment of acute graft versus host disease (Fast Track) (see also autoimmune)	Phase III www.osiris.com
QPI-1002 (siRNA oligonucleotide)	Quark Pharmaceuticals *Fremont, CA*	prophylaxis of delayed graft function in renal transplant patients	Phase II www.quarkpharma.com
reparixin (IL-8A/B receptor antagonist)	Dompe *Milan, Italy*	prevention of graft loss in pancreatic islet transplantation	Phase II www.dompe.com

Transplantation

Product Name	Sponsor	Official FDA Designation	Development Status
TOL101 (anti T-cell receptor murine mAb)	Tolera Therapeutics *Kalamazoo, MI*	prophylaxis of acute rejection of solid organ transplantation	Phase I/II www.tolera.com
TXA127 (angiotensin 1-7)	Tarix Pharmaceuticals *Cambridge, MA*	to accelerate engraftment of hematopoietic cells (treatment of neutropenia, thrombocytopenia, lymphoma, and anemia) in hematopoietic stem cell transplants	Phase II www.tarixpharma.com
		treatment of patients requiring stem cell transplantation to accelerate the mobilization of hematopoietic stem cells (CD34+) from the bone marrow to the peripheral blood when combined with a granulocyte colony-stimulating factor	Phase I completed www.tarixpharma.com

Other

Product Name	Sponsor	Official FDA Designation	Development Status
Ampligen® rintatolimid	Hemispherx Biopharma *Philadelphia, PA*	treatment of chronic fatigue syndrome	Phase III completed www.hemispherx.net
BIO300	Humanetics *Minneapolis, MN*	prevention of acute radiation syndrome	Phase I www.humaneticscorp.com
BMN-190 (rhTPP1)	BioMarin Pharmaceutical *San Rafael, CA*	treatment of neuronal ceroid lipofutscinosis type 2	Phase I/II www.bmrn.com
BYM338 (bimagrumab)	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of inclusion body myositis (Breakthrough Therapy)	Phase II www.novartis.com
CYT-107 (glycosylated recombinant human interleukin-7)	Cytheris *Rockville, MD*	treatment of progressive multifocal leukoencephalopathy	Phase II www.cytheris.com
Defitelo™ defibrotide	Gentium *Villa Guardia, Italy* Sigma-Tau Pharmaceuticals *Gaithersburg, MD*	for the treatment of hepatic veno-occlusive disease (Fast Track)	Phase III www.sigmatau.com

Other

Product Name	Sponsor	Official FDA Designation	Development Status
DigiBind® digoxin immune fab (ovine)	Glenveigh Medical *Chattanooga, TN*	treatment of severe preeclampsia and eclampsia (Fast Track)	Phase II www.glenveigh.com
ELAD® immortalized human liver cells/ extracorporeal liver assist device	Vital Therapies *San Diego, CA*	treatment of fulminant hepatic failure (acute liver failure)	Phase III www.vitaltherapies.com
entolimod	Cleveland BioLabs *Buffalo, NY*	prevention of death following a potentially lethal dose of total body irradiation during or after a radiation disaster	Phase I www.cbiolabs.com
fresolimumab (TGF-beta antagonist)	Sanofi US *Bridgewater, NJ*	treatment of primary focal segmental glomerulosclerosis	Phase II www.sanofi.com
human heterologous liver cells	Cytonet *Durham, NC*	treatment of urea cycle disorders	Phase II www.cytonetllc.com
Ilaris® canakinumab	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of TNF receptor-associated periodic syndrome (TRAPS)	Phase II www.novartis.com
Kiacta™ eprodisate	Auven Therapeutics *New York, NY*	treatment of secondary amyloidosis	Phase III www.auventx.com
Lucassin® terlipressin	Ikaria *Lebanon, NJ*	treatment of hepatorenal syndrome (Fast Track)	application submitted www.ikaria.com
metreleptin	AstraZeneca *Wilmington, DE* Bristol-Myers Squibb *Princeton, NJ*	treatment of metabolic disorders secondary to lipodystrophy (Fast Track) --- treatment of leptin deficiency secondary to generalized lipodystrophy and partial familial lipodystrophy (Fast Track)	application submitted www.astrazeneca.com www.bms.com --- application submitted www.astrazeneca.com www.bms.com
midostaurin (PKC412)	Novartis Pharmaceuticals *East Hanover, NJ*	treatment of mastocytosis (see also cancer, blood)	Phase II www.novartis.com
MLN9708 (ixazomib citrate)	Millennium Pharmaceuticals *Cambridge, MA*	treatment of systemic light chain (AL) amyloidosis (see also cancer, blood)	Phase III www.millennium.com
Natpara® recombinant human parathyroid hormone (rhPTH)	NPS Pharmaceuticals *Bedminster, NJ*	treatment of hypoparathyroidism	Phase III www.npsp.com

Other

Product Name	Sponsor	Official FDA Designation	Development Status
Neo-Urinary Conduit™	Tengion *Winston-Salem, NC*	treatment of bladder dysfunction requiring incontinent urinary diversion	Phase I www.tengion.com
NT-100 (rhGCSF)	Nora Therapeutics *Palo Alto, CA*	prevention of implantation failure	Phase II www.noratherapeutics.com
obeticholic acid (OCA)	Intercept Pharmaceuticals *New York, NY*	treatment of primary biliary cirrhosis	Phase III www.interceptpharma.com
OCR-002 (ornithine phenylacetate)	Ocera Therapeutics *San Diego, CA*	treatment of hyperammonemia and resultant hepatic encephalopathy (HE) in patients with acute liver failure (Fast Track)	Phase II www.ocerainc.com
Octaplas LG® human coagulation active plasma, solvent/detergent treated	Octapharma USA *Hoboken, NJ*	treatment of thrombotic thrombocytopenic purpura	application submitted www.octapharma.com
OrbeShield™ beclomethasone dipropionate	Soligenix *Princeton, NJ*	prevention of death following a potentially lethal dose of total body irradiation during or after a radiation disaster (Fast Track)	Phase I/II www.soligenix.com
Oxabact® oxalobacter formigenes	OxThera *Stockholm, Sweden*	treatment of primary hyperoxaluria	Phase II/III www.oxthera.com
PRT-201 (recombinant human type I pancreatic elastase)	Proteon Therapeutics *Waltham, MA*	prevention of arteriovenous fistula maturation and arteriovenous graft failure in patients with endstage renal disease who are receiving hemodialysis or preparing for hemodialysis (Fast Track)	Phase II www.proteontherapeutics.com
Ravicti™ glycerol	Hyperion Therapeutics *South San Francisco, CA*	for intermittent or chronic treatment of patients with cirrhosis and any grade hepatic encephalopathy	Phase II www.hyperiontx.com
recilisib	Onconova Therapeutics *Newton, PA*	treatment of acute radiation syndrome	Phase I www.onconova.com
Samsca™ tolvaptan	Otsuka Pharmaceuticals *Rockville, MD*	treatment of autosomal dominant polycystic kidney disease (Fast Track)	application submitted www.otsuka.com
SAR100842 (LPA-1/LPA-3 antagonist)	Sanofi US *Bridgewater, NJ*	treatment of patients with systemic sclerosis	Phase II www.sanofi.com

Other

Product Name	Sponsor	Official FDA Designation	Development Status
Serelsa™ fluoxetine rapid dissolve (AT001)	Autism Therapeutics *New York, NY*	treatment of autism (Fast Track)	Phase III www.autismtherapeutics.com
triheptanoin	Ultragenyx *Novato, CA*	treatment of fatty acid disorders	Phase II www.ultragenyx.com
uridine triacetate (PN401)	Wellstat Therapeutics *Gaithersburg, MD*	an antidote in the treatment of 5-fluorouracil poisoning	Phase III www.wellstattherapeutics.com
XEN402 (sodium channel inhibitor)	Teva Pharmaceutical *North Wales, PA* Xenon Pharmaceuticals *Burnaby, Canada*	treatment of erythromelalgia	Phase II www.tevapharm.com www.xenon-pharma.com
Xiaflex® collagenase clostridium hystolyticum	Auxilium Pharmaceuticals *Chesterbrook, PA*	treatment of Peyronie's disease	application submitted www.auxilium.com

The content of this report has been obtained through public, government (FDA's Orphan Drug Product designation database) and industry sources, and the Adis "R&D Insight" database based on the latest information. **Report current as of September 23, 2013.** *The medicines in this report include medicines being developed by U.S. based companies conducting trials in the United States and abroad, PhRMA-member companies conducting trials in the United States and abroad, and foreign companies conducting clinical trials in the United States. The information in this report may not be comprehensive. For more specific information about a particular product, contact the individual company directly or go to* ***www.clinicaltrials.gov****. The entire series of Medicines in Development is available on PhRMA's website.*

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Glossary

adenocarcinoma—Cancer of glandular tissue, or tumor of which gland-derived cells form gland-like structures.

adjuvant—A substance or drug that aids another substance in its action.

alpha 1-proteinase inhibitor deficiency—Although it is a rare condition, some people are congenitally deficient in alpha 1-proteinase inhibitor (or alpha 1-trypsin, a glycoprotein), which predisposes them to pulmonary emphysema early in life, even in the absence of exposure to substances (like cigarette smoke) that interfere with lung-defense mechanisms.

amyotrophic lateral sclerosis (ALS)—Also known as Lou Gehrig's disease, the most common of the motor neuron diseases, a group of rare disorders in which the nerves that control muscular activity degenerate within the brain and spinal cord causing weakness and wasting of the muscles.

anaplastic thyroid carcinoma—An aggressive, invasive form of cancer of the thyroid gland. It occurs most often in people over age 60. The cause is unknown. Anaplastic cancer accounts for only about 1 percent of all thyroid cancers and is a very rare disease.

application submitted—An application for marketing has been submitted by the company to the Food and Drug Administration (FDA).

aspergillosis—Infection caused by aspergillus, a fungus sometimes found in old buildings or decaying plant matter.

B-cell—A class of white blood cells important to the body's immune system.

Becker muscular dystrophy (BMD)—One of nine types of muscular dystrophy, a group of genetic, degenerative diseases primarily affecting voluntary muscles. It's caused by an insufficient production of dystrophin, a protein that helps keep muscle cells intact. Onset can occur during adolescence or adulthood. Symptoms include generalized weakness and wasting, which first affects the muscles of the hips, pelvic area, thighs and shoulders. BMD is similar to Duchenne MD but often much less severe. The disease progresses slowly and with variability but can affect all voluntary muscles. BMD primarily affects boys and men, who inherit the disease through their mothers. Most with BMD survive well into mid- to late adulthood.

Breakthrough therapy—A designation assigned by the U.S. Food and Drug Administration that is intended to expedite the development and review of drugs for serious or life-threatening conditions. The criteria for breakthrough therapy designation require preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy. A breakthrough therapy designation conveys all of the **Fast Track** designation features, as well as more intensive FDA guidance on an efficient drug development program.

carcinoma—Cancer. **Squamous cell carcinoma** is one of the three most common types of skin cancer, arising from the flattened, scale-like cells in the skin and resulting primarily from long-term exposure to the sun.

chronic fatigue syndrome—The symptoms of this illness include debilitating fatigue, interference with the ability to concentrate, and, in some cases, a low-grade fever and swelling of the lymph nodes. Many possible causes have been implicated, but the true cause remains unknown.

Clostridium difficile—A bacterium that produces an irritating toxin that causes a form of colitis characterized by profuse, watery diar¬rhea with cramps and low-grade fever.

Crohn's disease—A subacute chronic gastro¬intestinal disorder, involving the small intestine, characterized by patchy deep ulcers that may cause fistulas and a narrowing and thickening of the bowel.

cutaneous—Pertaining to the skin.

cystic fibrosis—A genetic disorder of the exocrine glands (such as sweat glands or kidneys) that causes abnormal mucous secretions that obstruct glands and ducts in various organs.

cytomegalovirus (CMV)—A DNA virus that can cause infection without symptoms or with mild flu-like symptoms.

diabetes—A chronic disease in which the body does not produce or properly use insulin, a hormone that is needed to convert sugar, starches and other food into energy needed for daily life. Symptoms may include excessive thirst, hunger, urination and weight loss. The cause of diabetes continues to be a mystery, although both genetics and environmental factors such as obesity and lack of exercise appear to play roles. Type 1 diabetes, the more severe form, results from the body's failure to produce insulin, which "unlocks" the cells of the body, allowing glucose to enter and fuel them. It is estimated that 5 percent to 10 percent of Americans who are diagnosed with diabetes have type 1, which requires insulin treatment.

Duchenne muscular dystrophy—An inherited disorder that involves rapidly worsening muscle weakness. Other muscular dystrophies get worse much more slowly. Duchenne's is caused by a defective gene. Because of the way the disease is inherited, males are more likely to develop symptoms than are women.

epidermolysis bullosa—A rare, inherited condition in which blisters appear on the skin after minor damage. It mainly affects young children and has a wide range of severity.

Fabry disease—A genetic metabolic disorder that causes build-up of certain lipids. It becomes clinically apparent in childhood and adolescence with fever, pain and small vascular tumors. It progresses to central nervous system disturbances and renal and cardiac failure in mid-life.

Glossary

Fast Track—A process designed to facilitate the development and expedite the review of drugs to treat serious diseases and fill an unmet medical need. The status is assigned by the U.S. Food and Drug Administration. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious diseases. Generally, determining factors include whether the drug will have an impact on such factors as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one. Filling an unmet medical need is defined as providing a therapy where none exists or providing a therapy which may be potentially superior to existing therapy. Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

fragile X syndrome—One of the most common causes of inherited mental retardation and neuropsychiatric disease in human beings, affecting as many as 1 in 2,000 males and 1 in 4,000 females. The syndrome is also known as FRAXA (the fragile X chromosome itself) and as the Martin-Bell syndrome. However, the preferred name is fragile X syndrome. The characteristic features of the fragile X syndrome in boys include prominent or long ears, a long face, delayed speech, large testes, hyperactivity, tactile defensiveness, gross motor delays, and autistic-like behaviors. Much less is known about girls with fragile X syndrome. Only about half of all females who carry the genetic mutation have symptoms themselves. Of those, half are of normal intelligence, and only one-fourth have an IQ under seventy. Few fragile X girls have autistic symptoms, although they tend to be shy and quiet.

Friedreich's ataxia—An inherited disease that causes progressive damage to the nervous system resulting in symptoms ranging from gait disturbance and speech problems to heart disease. "Ataxia," which refers to coordination problems such as clumsy or awkward movements and unsteadiness, occurs in many different diseases and conditions. The ataxia of Friedreich's ataxia results from the degeneration of nerve tissue in the spinal cord and of nerves that control muscle movement in the arms and legs. The spinal cord becomes thinner and nerve cells lose some of their myelin sheath—the insular covering on all nerve cells that helps conduct nerve impulses. The condition, although rare, is the most prevalent inherited ataxia, affecting about 1 in every 50,000 people in the United States.

FXIII deficiency (congenital)—A rare disease that affects 1 out of every 3-5 million people in the United States, or approximately 150 people. The condition is characterized by blood that clots normally, but the clots are unstable, so bleeding recurs. FXIII deficiency can cause umbilical cord bleeding in some newborns, soft tissue bruising, mucosal bleeding and potentially fatal intracranial hemorrhage (ICH). Studies have shown that up to 60 percent of people with FXIII deficiency experience at least one ICH during their lifetime.

Gaucher disease—An inherited disease caused by a lack or deficiency of an enzyme (glucocerebrosidase). Primarily affects the liver, spleen and bone marrow.

glioblastoma multiforme—The most common and most malignant of the astrocytomas. The tumor grows so fast that it increases pressure in the brain, producing headaches, slowed thinking, and if severe enough, sleepiness and coma.

glioma—A type of brain tumor arising from the supporting glial cells within the brain. Gliomas make up about 60 percent of all primary brain tumors and are frequently malignant.

graft versus host disease—In bone marrow transplantation, normal bone marrow is used to replace malignant or defective marrow. In an **allogeneic** transplantation, healthy marrow is taken from a donor; in an **autologous** transplantation, the patient's own healthy marrow is used. In graft vs. host disease, a complication of such transplants, immune system cells attack the transplant recipient's tissues.

hematopoietic support—Helping the body to form blood or blood cells.

hemophilia A and B—Hemophilia A, the "clas¬sic" hemophilia, is a genetic bleeding disorder due to deficiency of the coagulation factor VIII. Hemophilia B, or "Christmas" disease, is caused by deficiency of coagulation factor IX.

hepatic—Related to the liver.

hepatitis—Inflammation of the liver with accompanying liver cell damage or death, caused most often by viral infection (e.g., **types B** and **C**), but also by certain drugs, chemicals or poisons. Hepatitis may be either acute (of limited duration) or chronic (continuing).

hepatocellular—Pertaining to the cells in the liver.

hepatocellular cancer/carcinoma—A cancer that begins in the liver cells.

hereditary angioedema—A rare but serious problem with the immune system that is passed down through families. It is caused by low levels or improper functioning of a protein called C1 inhibitor, which affects the blood vessels. People with hereditary angioedema can develop rapid swelling of the hands, feet, limbs, face, intestinal tract, or airway (larynx or trachea).

HPV (human papillomavirus)—Viral agent of warts, believed to be contagious and usually harm-less, but it can lead to cervical cancer.

Huntington's disease—Huntington's chorea is an uncommon, inherited disease in which degeneration of the basal

ganglia (structures deep in the brain) results in chorea (rapid, jerky, involuntary movements) and dementia (progressive mental impairment). Symptoms do not usually appear until the age of 35 to 50.

hypercholesterolemia (homozygous familial)—An inherited metabolic disorder resulting in an abnormal amount of cholesterol in the blood. It can lead to accelerated atherosclerosis and early heart attack. Dietary treatment seldom helps in these cases.

hypophosphatasia—A rare, inherited disease that results in decreased activity of the enzyme alkaline phosphatase, which assists in the metabolism of phosphate that is present in many tissues, including bones and teeth. The illness may occur during infancy or as an adult. The infantile form of hypophosphatasia is fatal in 50 percent of cases. Symptoms of hypophosphatasia in infants include poor feeding, failure to gain weight, failure to thrive, delayed development, loss of teeth, and bone pain. Adults who develop hypophosphatasia have a normal life expectancy. Symptoms in adults include premature loss of teeth, fractures, and bone pain.

idiopathic thrombocytopenia purpura—A condition in which there is destruction of blood platelets by the immune system. The reduced number of platelets may result in abnormal bleeding into the skin (purpura) and other parts of the body.

inherited mitochondrial diseases—A group of systemic diseases caused by inherited or acquired damage to the mitochondria, which are small, energy-producing structures found in every cell in the body that serve as the cells' "power plants." When the mitochondria are not working properly, there is an energy shortage within those areas of the body that consume large amounts of energy such as the muscles, brain, and heart. The result is often muscle weakness, fatigue, and problems with the heart, eyes, and various other systems.

Juvenile rheumatoid arthritis—Refers to arthritis or an arthritis-related condition (rheumatic disease) that occurs by age 15 or younger.

Leber congenital amaurosis (LCA)—An inherited retinal degenerative disease characterized by severe loss of vision at birth. A variety of other eye-related abnormalities including roving eye movements, deep-set eyes, and sensitivity to bright light also occur with this disease. Some patients with LCA also experience central nervous system abnormalities.

Lesch-Nyhan syndrome (LNS)—A rare, inherited disorder caused by an enzyme (HPRT) deficiency. LNS is present at birth in baby boys. The lack of HPRT causes a build-up of uric acid in all body fluids, leading to symptoms such as severe gout, poor muscle control, and moderate retardation, which appear in the first year of life. A striking feature of LNS is self-mutilating behaviors–characterized by lip and finger biting–that begin in the second year of life. Abnormally high uric acid levels can cause sodium urate crystals to form in the joints, kidneys, central nervous system, and other tissues of the body, leading to gout-like swelling in the joints and severe kidney problems. Neurological symptoms include facial grimacing, involuntary writhing, and repetitive movements of the arms and legs similar to those seen in Huntington's disease.

leukemia—A form of cancer involving abnormally growing white blood cells, which dominate the bone marrow and prevent it from making enough normal blood cells. This leaves the patient highly susceptible to serious infections, anemia and bleeding episodes. The cells increase in the blood, interfering with the function of other organs.

lymphoma—Cancers in which the cells of lymphoid tissue, found mainly in the lymph nodes and spleen, multiply unchecked. Lymphomas fall into two categories: One is called Hodgkin's disease, characterized by a particular kind of abnormal cell. All others are called non-Hodgkin's lymphomas, which vary in their malignancy according to the nature and activity of the abnormal cells. **B and T-cell lymphomas** are caused by proliferation of the two principal types of white blood cells, called B- and T-lymphocytes. **Mycosis fungoides** is a type of lymphoma that primarily affects the skin of the buttocks, back or shoulders but can also occur in other sites. The cause is unknown.

melanoma—A cancer made up of pigmented skin cells.

metastatic—Secondary cancers that have spread from the primary or original cancer site.

mucositis—The swelling, irritation, and ulceration of the mucosal cells that line the digestive tract. Mucositis can occur anywhere along the digestive tract from the mouth to the anus. It can be a very troublesome and painful side effect of chemotherapy.

multiple myeloma—A malignant condition characterized by the uncontrolled proliferation and disordered function of plasma cells (a type of white blood cell) in the bone marrow. It occurs in middle to old age and leaves patients vulnerable to increased infections and anemia.

multiple sclerosis (MS)—Progressive disease of the central nervous system in which scattered patches of the covering of nerve fibers (myelin) in the brain and spinal cord are destroyed. Symptoms range from numbness and tingling to paralysis and incontinence.

neuroblastoma—A tumor of the adrenal glands or sympathetic nervous system (the part of the nervous system responsible for certain automatic body functions, such as the control of heart rate). Neuroblastomas are the most common

extra-cranial (outside the skull) solid tumors of childhood.

neuropathic pain—Caused by disease, inflammation, or damage to the peripheral nerves, which connect the central nervous system (brain and spinal cord) to the sense organs, muscles, glands, and internal organs.

neuropathy—Disease, inflammation, or damage to the peripheral nerves, which connect the central nervous system to the sense organs, muscles, glands, and internal organs.

Parkinson's disease—Chronic neurologic disease of unknown cause, characterized by tremors, rigidity and an abnormal gait. The most common variety is idiopathic Parkinson's disease.

Phase 0—First-in-human trials conducted in accordance with FDA's 2006 guidance on exploratory Investigational New Drug (IND) studies designed to speed up development of promising drugs by establishing very early whether the tested compound behaves in human subjects as was anticipated from preclinical studies.

Phase I—Safety testing and pharmacological profiling in humans.

Phase II—Effectiveness and safety testing in humans.

Phase III—Extensive clinical trials to demonstrate safety and efficacy in humans.

Pompe disease—A rare (estimated at 1 in every 40,000 births), inherited and often fatal disorder that disables the heart and muscles. It is caused by mutations in a gene that makes an enzyme called alpha-glucosidase (GAA). Normally, the body uses GAA to break down glycogen, a stored form of sugar used for energy. But in Pompe disease, mutations in the GAA gene reduce or completely eliminate this essential enzyme. Excessive amounts of glycogen accumulated everywhere in the body, but the cells of the heart and skeletal muscles are the most seriously affected. The symptoms of Pompe disease can vary widely in terms of age of onset and severity depending on the degree of enzyme deficiency.

postherpetic neuralgia—A burning pain that may recur at the site of an attack of shingles months or even years after the illness.

prophylaxis—Treatment intended to preserve health and prevent the spread of disease.

pulmonary—Pertaining to the lungs.

pulmonary arterial hypertension—High blood pressure in the arteries supplying the lungs due to increased resistance to blood flow through the lungs.

renal—Relates to kidneys.

respiratory distress syndrome (RDS)—Lung disorder of premature infants characterized by respiratory distress and cyanosis (lack of oxygen in blood). RDS is caused by a deficiency of surfactant, a substance that coats the inner lining of the lungs and prevents them from collapsing during exhalation.

retinitis pigmentosa—Degeneration in both eyes of the rods and cones of the retina—the light-sensitive membrane that lines the inside of the back of the eye on which images are cast by the cornea and lens. Usually has a genetic basis. The first symptom is usually night blindness, progressing to a ring-shaped area of blindness that gradually extends to lessen the field of vision.

sickle cell anemia/disease—Inherited blood disorder in which red cells are abnormal in shape and contain an abnormal oxygen-carrying pigment called hemoglobin S, resulting in chronic, severe anemia and the characteristic sickle shape of the red cell. Caused by mutation of the gene that codes for hemoglobin.

spinal cord injury—Damage to the spinal cord which can cause loss of sensation, muscle weakness or paralysis.

systemic—Affecting the whole body.

thalassemia—Not just one disease but rather a complex series of genetic (inherited) disorders all of which involve underproduction of hemoglobin, the indispensable molecule in red blood cells that carries oxygen.

Tourette syndrome (TS)—A neurological disorder characterized by repetitive, involuntary movements and vocalizations called tics. The early symptoms of TS are typically noticed first in childhood, with the average onset between the ages of 3 and 9. TS occurs in people from all ethnic groups; males are affected about three to four times more often than females. It is estimated that 200,000 Americans have the most severe form of TS, and as many as 1 in 100 exhibit milder and less complex symptoms such as chronic motor or vocal tics. Although TS can be a chronic condition with symptoms lasting a lifetime, most people with the condition experience their worst tic symptoms in their early teens, with improvement occurring in the late teens and continuing into adulthood.

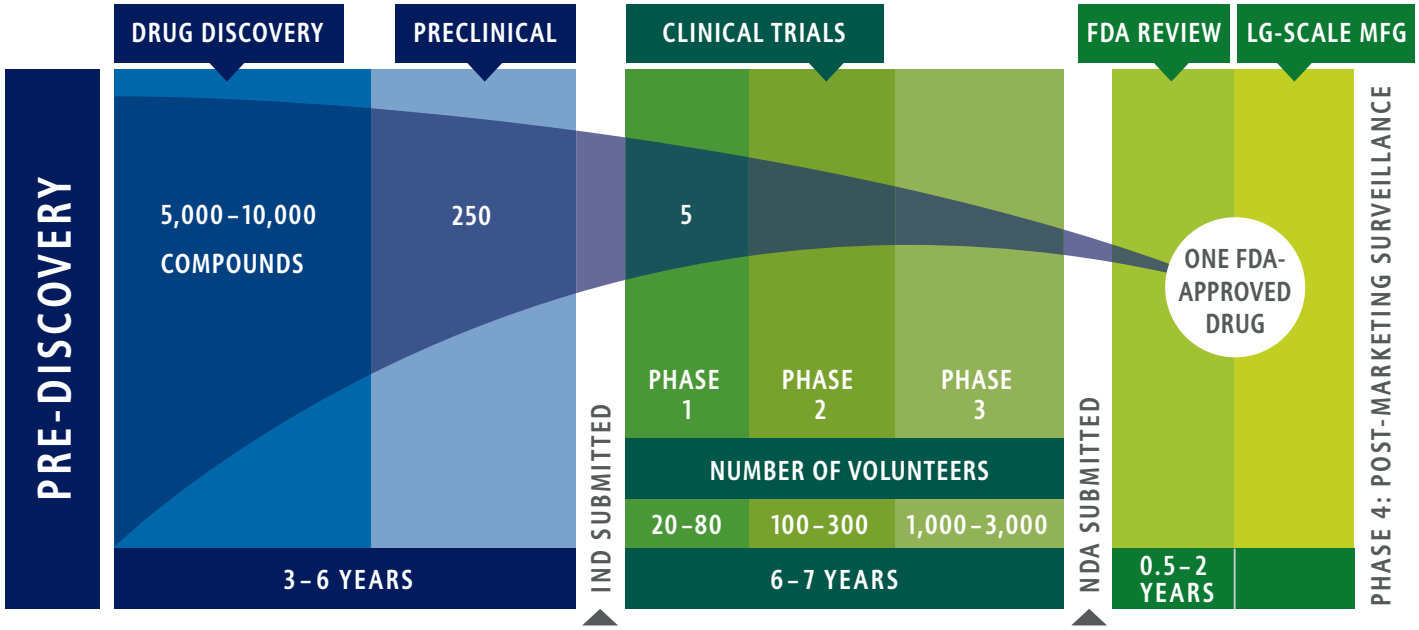
ulcerative colitis—A chronic inflammation and ulceration of the lining of the colon and rectum. It causes bloody diarrhea and mainly involves the left colon.

uveitis—Inflammation of the uvea, the middle layer of the eye.

The Drug Discovery, Development and Approval Process

Developing a new medicine takes an average of 10-15 years;
For every 5,000-10,000 compounds in the pipeline, only 1 is approved.

Drug Discovery and Development: A LONG, RISKY ROAD



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. 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 recent study by the Tufts Center for the Study of Drug Development.

Once a new compound has been identified in the laboratory, medicines are usually 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—Researchers test the drug in a small group of people, usually between 20 and 80 healthy adult volunteers, to evaluate its initial safety and tolerability profile, determine a safe dosage range, and identify potential side effects.

Clinical Trials, Phase II—The drug is given to volunteer patients, usually between 100 and 300, to see if it is effective, identify an optimal dose, and to further evaluate its short-term safety.

Clinical Trials, Phase III—The drug is given to a larger, more diverse patient population, often involving between 1,000 and 3,000 patients (but sometime 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.

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.

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. PhRMA member companies invested an estimated $48.5 billion in research and development in 2012.