

# Rare Diseases

PRESENTED BY AMERICA'S BIOPHARMACEUTICAL RESEARCH COMPANIES

## More Than 400 Medicines in Development to Treat or Prevent Rare Diseases

As we issue our report on rare diseases, I can't help but think of the words of a mother whose daughter has Lennox-Gastaut Syndrome, which causes multiple, daily seizures. In a documentary about the disease, she gives the following advice to other parents: "Don't try looking at tomorrow. Don't even think of the next hour. Just look for the next minute. And if the next minute is too far away, look for the next second. We've had to do this at times. But there is hope."

Remembering her, I was gratified to find that among the 460 medicines in development for rare diseases, one is for Lennox-Gastaut Syndrome. With great science, and some good fortune, we hope it will prove a solution. Meanwhile, much work remains.

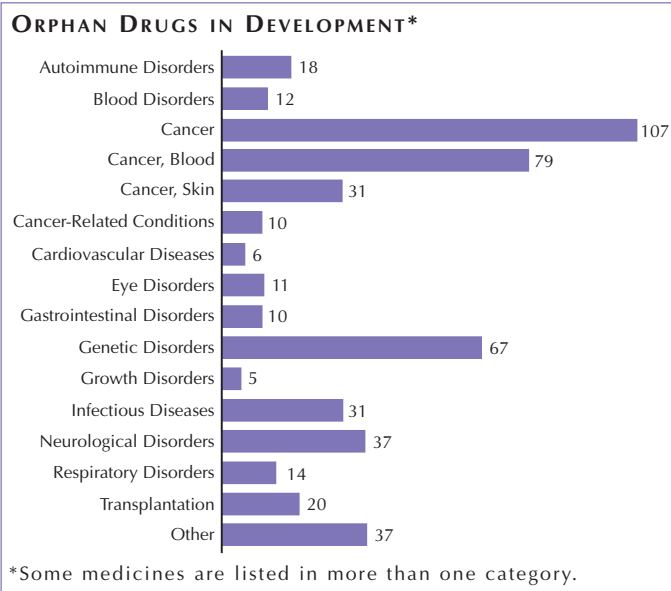
Rare diseases, taken together, aren't rare at all. In fact, according to the National Institutes of Health, 25-30 million Americans have one of the nearly 7,000 diseases that are officially deemed "rare" because alone they each affect fewer than 200,000 people. Sometimes, only a few hundred Americans are known to have a particular rare disease.

Simply getting a diagnosis often becomes a quest, since a long series of doctors may have never heard of or seen the disease. Sometimes, patients and parents of a sick child are initially relieved when told they or their child has a disease they've never heard of, because finally they have a name to give the suffering. With new optimism in their voices they often ask: "What medicines or surgeries can help them?" Too often, the terrible answer is, "None."

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

Since 1983, more than 350 medicines have been approved to treat rare diseases, compared to fewer than 10 in the 1970s. Moreover, the Orphan Drug Act of 1983 provided incentives, for example a longer period of marketing exclusivity, for drugs that are not expected to recoup their development costs or that are targeted at diseases affecting fewer than 200,000 people. Under the Orphan Drug Act, 2,313 medicines have been designated orphan drugs by the U.S. Food and Drug Administration (FDA) as of January 24, 2011. These medicines are in all stages of development and some will eventually gain approval.

Over the last few years, new medicines have been approved for Pompe disease, which causes among other symptoms an engorged heart. Other recently approved treatments are for rare cancers and myelodysplastic syndromes, which are pre-leukemia diseases affecting blood marrow.



America's biopharmaceutical research companies are continuing that progress. The 460 medicines for rare diseases in this report are all in later stages of the pipeline, meaning in clinical trials or under review by the FDA. A major area of research involves rare cancers. Solid tumors of the liver and thyroid, cancer of the blood and melanoma, or skin cancer, account for more than one-third of all rare disease drugs under development.

Other major areas of research include: genetic disorders, such as cystic fibrosis, with 67 medicines in development; neurologic disorders, such as multiple sclerosis and muscular dystrophy, with 37 medicines in development; infectious diseases, such as anthrax and West Nile virus, with 31 medicines in development.

Other examples include a medicine in development for epidermolysis bullosa, a group of inherited disorders where skin blisters develop in response to minor trauma, and one for Friedrich's ataxia, a genetic disease that causes degenerative nerve damage in children.

With continued innovation, we hope that one day, whenever the question is asked, "How can we help them?" there always will be an encouraging answer.

Sincerely,  
John J. Castellani  
President and CEO  
PhRMA

# Orphan Drugs in Development

## AUTOIMMUNE DISORDERS

Product Name	Sponsor	FDA Official Designation*	Development Status**
3,4-diamino-pyridine	Jacobus Pharmaceutical <i>Princeton, NJ</i>	treatment of Lambert-Eaton myasthenic syndrome	in clinical trials (609) 921-7447
amifampridine	BioMarin Pharmaceutical <i>Novato, CA</i>	treatment of Lambert-Eaton myasthenic syndrome	Phase III (415) 506-6700
<b>Ampligen®</b> rintatolimod	Hemispherx Biopharma <i>Philadelphia, PA</i>	treatment of chronic fatigue syndrome (see also infectious diseases)	application submitted (215) 988-0080
ARG201	arGentis Pharmaceuticals <i>Memphis, TN</i>	treatment of diffuse systemic sclerosis	Phase II (901) 818-3262
autoimmune diabetes vaccine	Diamyd <i>Pittsburgh, PA</i>	treatment of type I diabetes with residual beta cell function	Phase III (412) 770-1310
gevokizumab	XOMA <i>Berkeley, CA</i>	treatment of Behcet's disease	Phase I (510) 204-7200
<b>Ilaris®</b> canakinumab	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of systemic onset juvenile idiopathic arthritis	Phase III (888) 669-6682
MEDI-551	MedImmune <i>Gaithersburg, MD</i>	treatment of scleroderma	Phase I (301) 398-0000
<b>Oralgam™</b> human gammaglobulin	Latona Life Sciences <i>Phoenix, AZ</i>	treatment for juvenile rheumatoid arthritis	Phase II completed (520) 844-1187
otelixizumab (TRX4)	Tolerx <i>Cambridge, MA</i>	treatment of new-onset type 1 diabetes mellitus	Phase III (617) 354-8100
<b>Prochymal®</b> human mesenchymal stem cell therapy	Osiris Therapeutics <i>Columbia, MD</i>	treatment of type 1 diabetes patients with residual beta cell function (see also cancer-related conditions)	Phase II (443) 545-1800
recombinant human alpha-fetoprotein (rhAFP)	GTC Biotherapeutics <i>Framingham, MA</i>	treatment of myasthenia gravis	Phase I (508) 620-9700
<b>Remicade®</b> infliximab	Centocor Ortho Biotech <i>Horsham, PA</i>	treatment of chronic sarcoidosis (see also gastrointestinal disorders)	Phase III completed (800) 457-6399
reslizumab	Cephalon <i>Frazer, PA</i>	treatment of children with eosinophilic esophagitis	Phase III (610) 344-0200

\* 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 in this report, please use the contact information provided.

## AUTOIMMUNE DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Rituxan®</b> rituximab	Genentech <i>South San Francisco, CA</i>	treatment of patients with anti-neutrophil cytoplasmic antibody-associated vasculitis (Wegener's granulomatosis, microscopic polyangiitis, and Churg-Strauss syndrome)	Phase III (650) 225-1000
		treatment of immune thrombocytopenic purpura	Phase II (650) 225-1000
<b>Simponi™</b> golimumab	Centocor Ortho Biotech <i>Horsham, PA</i>	treatment of chronic sarcoidosis	Phase II (800) 457-6399
SM101 (recombinant human soluble Fc-gamma receptor IIB)	SuppreMol <i>Munich, Germany</i>	treatment of idiopathic thrombocytopenic purpura	Phase I www.suppremol.com
<b>Veldona®</b> interferon-alfa	Amarillo Biosciences <i>Amarillo, TX</i>	treatment of Behcet's disease (see also blood disorders, infectious diseases)	Phase II (806) 376-1741

## BLOOD DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
ARC1779	Archemix <i>Cambridge, MA</i>	treatment of thrombotic thrombocytopenic purpura	Phase II (617) 621-7700
<b>Cinquil™</b> reslizumab	Cephalon <i>Frazer, PA</i>	treatment of hypereosinophilic syndrome	Phase III (610) 344-0200
CYT-387	YM Biosciences USA <i>Lehigh Valley, PA</i>	treatment of myelofibrosis	Phase I/II (610) 560-0600
eptacog alfa (recombinant factor VIIa)	Novo Nordisk <i>Princeton, NJ</i>	treatment of bleeding episodes in Glanzmann's thrombasthenia	Phase III (609) 987-5800
FBS 0701	Ferrokin BioSciences <i>San Francisco, CA</i>	treatment of chronic iron overload in patients with transfusion-dependent anemias	Phase I www.ferrokin.com
<b>Ferriprox™</b> deferiprone	ApoPharma <i>Toronto, Canada</i>	treatment of iron overload in patients with hematologic disorders requiring chronic transfusion therapy (see also genetic disorders)	Phase III completed (416) 749-9300
INCB18424 (JAK tyrosine kinase inhibitor)	Incyte <i>Wilmington, DE</i>	treatment of essential thrombocythemia	Phase III (302) 498-6700
		treatment of myelofibrosis	Phase III (302) 498-6700
		treatment of polycythemia vera	Phase III (302) 498-6700

## BLOOD DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
<b>octaplex</b> <sup>®</sup> human prothrombin complex	Octapharma USA <i>Hoboken, NJ</i>	reversal of anticoagulation therapy in patients needing treatment of serious or life-threatening bleeding and/or needing urgent surgery or invasive procedures (Fast Track)	Phase III (866) 766-4860
<b>Sanvar</b> <sup>®</sup> vapeptide	Debiopharm <i>Lausanne, Switzerland</i>	treatment of esophageal hemorrhage patients with portal hypertension	application submitted www.debiopharm.com
TG-101348	TargeGen <i>San Diego, CA</i>	treatment of secondary and primary myelofibrosis	Phase I/II (858) 678-0760
tissue repair stem cell therapy	Aastrom Biosciences <i>Ann Arbor, MI</i>	treatment of osteonecrosis (see also cardiovascular diseases)	Phase III (734) 930-5555
<b>Veldona</b> <sup>®</sup> interferon-alpha	Amarillo Biosciences <i>Amarillo, TX</i>	treatment of polycythemia vera (see also autoimmune disorders, infectious diseases)	Phase II (806) 376-1741

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
3F8 mAb	United Therapeutics <i>Silver Spring, MD</i>	treatment of neuroblastoma	Phase II (301) 608-9292
abagovomab	Menarini Ricerche <i>Florence, Italy</i>	treatment of epithelial ovarian cancer	Phase II/III www.menarini.com
ABC294650	Apogee Biotechnology <i>Hummelstown, PA</i>	treatment of idiopathic pulmonary fibrosis	Phase I (843) 792-3420
<b>Abraxane</b> <sup>®</sup> paclitaxel protein-bound particles for injectable suspension, albumin-bound	Celgene <i>Summit, NJ</i>	treatment of pancreatic cancer (see also cancer, skin)	Phase III (908) 673-9000
AEZS-108	AEterna Zantaris <i>Warren, NJ</i>	treatment of ovarian cancer	Phase II (908) 626-5428
<b>Afinitor</b> <sup>®</sup> everolimus	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of gastroenteropancreatic neuroendocrine tumors (see also cancer, blood)	Phase III (888) 669-6682
amrubicin	Celgene <i>Summit, NJ</i>	treatment of small-cell lung cancer (Fast Track)	Phase III (908) 673-9000
antineoplaston AS2-1	Burzynski Research Institute <i>Houston, TX</i>	treatment for patients with brain stem glioma	Phase II
<b>Archexin</b> <sup>®</sup> antisense oligonucleotide	Rexahn <i>Rockville, MD</i>	treatment of pancreatic cancer	Phase II (240) 268-5300

# CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
ARO-002	AROG Pharmaceuticals <i>Dallas, TX</i>	enhancement of stem cell engraftment through ex vivo treatment of human allogeneic hematopoietic stem cells (treatment of neutropenia, thrombocytopenia, lymphopenia, and anemia)	Phase II (214) 451-4500
astuprotimut-R	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of MAGE-A3 positive non-small-cell lung cancer (see also cancer, skin)	Phase III (888) 825-5249
atiprimod	Callisto Pharmaceuticals <i>New York, NY</i>	treatment of carcinoid tumors	Phase II (212) 297-0010
<b>Avastin</b> <sup>®</sup> bevacizumab	Genentech <i>South San Francisco, CA</i>	therapeutic treatment of patients with ovarian cancer (see also cancer, skin)	Phase III (650) 225-1000
		in combination with a platinum and 5-FU or capecitabine for the treatment of stomach cancer	Phase III (650) 225-1000
		treatment of fallopian tube carcinoma	Phase III (650) 225-1000
		treatment of primary peritoneal carcinoma	Phase III (650) 225-1000
axitinib	Pfizer, Inc. <i>New York, NY</i>	treatment of follicular, medullary and anaplastic thyroid carcinoma and metastatic or locally advanced papillary thyroid cancer	Phase II (860) 732-5156
<b>Azedra</b> <sup>™</sup> iobenguane I-131	Molecular Insight Pharmaceuticals <i>Cambridge, MA</i>	treatment of neuroendocrine tumors (Fast Track)	Phase II (617) 492-5554
BC-819	BioCancell Therapeutics <i>Jerusalem, Israel</i>	treatment of pancreatic cancer	Phase I/II www.biocancell.com
		treatment of ovarian cancer	Phase I/II www.biocancell.com
cabozantinib	Exelixis <i>South San Francisco, CA</i>	treatment of follicular, medullary and anaplastic thyroid carcinoma and metastatic or locally advanced papillary thyroid cancer	Phase III (650) 837-7000
catumaxomab	Fresenius Biotech North America <i>Waltham, MA</i>	treatment of ovarian cancer	Phase II www.fresenius-biotech.com
		treatment of gastric cancer	Phase II www.fresenius-biotech.com
<b>Cerepro</b> <sup>™</sup> sitimagene ceradenovec	Ark Therapeutics <i>London, England</i>	use with gancyclovir in the treatment of malignant glioma	Phase III www.arktherapeutics.com

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
ch14.18 mAb	United Therapeutics <i>Silver Spring, MD</i>	treatment of neuroblastoma	Phase II (301) 608-9292
cilengitide	EMD Serono <i>Rockland, MA</i>	treatment of malignant glioma	Phase III (800) 283-8088
cintredekin besudotox (IL-13-PE38QQR)	NeoPharm <i>Waukegan, IL</i>	treatment of malignant glioma (Fast Track)	Phase I (847) 887-0800
clivatuzumab (90Y-hPAM4)	Immunomedics <i>Morris Plains, NJ</i>	treatment of pancreatic cancer	Phase I/II (973) 605-8200
CO-101	Clavis Pharma <i>Oslo, Norway</i> Clovis Oncology <i>Boulder, CO</i>	treatment of pancreatic cancer	Phase II (303) 625-5000
<b>Cotara</b> <sup>TM</sup> iodine I-131 radiolabeled chimeric MAb tumor necrosis treatment (TNT-1B)	Peregrine Pharmaceuticals <i>Tustin, CA</i>	treatment of glioblastoma multiforme (Fast Track)	Phase II (714) 508-6000
CPI-613	Cornerstone Pharmaceuticals <i>Cranbury, NJ</i>	treatment of pancreatic cancer	Phase I/II (609) 409-7050
CPP-1X (eflornithine)	Cancer Prevention Pharmaceuticals <i>Tucson, AZ</i>	treatment of neuroblastoma	Phase I (520) 908-7774
crizotinib	Pfizer <i>New York, NY</i>	treatment of ALK-positive non-small-cell lung cancer (Fast Track)	application submitted (860) 732-5156
<b>CVac</b> <sup>TM</sup> cancer vaccine MUC-1	Prima Biomed <i>Armdale, Australia</i>	treatment of ovarian cancer	Phase II <a href="http://www.primabiomed.com">www.primabiomed.com</a>
<b>DCVax-Brain</b> <sup>®</sup>	Northwest Biotherapeutics <i>Bothell, WA</i>	treatment of primary brain malignant cancer	Phase II (425) 608-3000 <a href="http://www.nwbio.com">www.nwbio.com</a>
denosumab	Amgen <i>Thousand Oaks, CA</i>	treatment of patients with giant cell tumor of bone	Phase II (805) 447-1000
EC20 (technetium Tc 99m pterotetramide)	Endocyte <i>West Lafayette, IN</i>	for the identification of ovarian carcinomas	Phase II (765) 463-7175
EGEN-001 (DNA plasmid vector-expressing human IL-12 gene)	EGEN <i>Huntsville, AL</i>	treatment of ovarian cancer	Phase I (256) 512-0077
ENMD-2076	EntreMed <i>Rockville, MD</i>	treatment of ovarian carcinoma (see also cancer, blood)	Phase II (240) 864-2600
ensituximab (NPC-1C)	Neogenix Oncology <i>Great Neck, NY</i>	treatment of pancreatic cancer	Phase I (516) 482-1200

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Product Name	Sponsor	FDA Official Designation	Development Status
farletuzumab (MORAb-003)	Morphotek <i>Exton, PA</i>	treatment of ovarian cancer	Phase III (877) 873-4724
fosbretabulin (combretastatin A4 phosphate)	OXiGENE <i>Watertown, MA</i>	treatment of anaplastic thyroid cancer, medullary thyroid cancer, and stage IV papillary or follicular thyroid cancer (Fast Track)	Phase II/III (781) 547-5900
		treatment of ovarian cancer	Phase II (781) 547-5900
<b>Fusilev</b> <sup>®</sup> levoleucovorin	Spectrum Pharmaceuticals <i>Irvine, CA</i>	for use in combination chemotherapy with the approved agent 5-fluorouracil in the palliative treatment of metastatic adenocarcinoma of the colon and rectum	application submitted (949) 788-6700
G17DT immunogen	Cancer Advances <i>Durham, NC</i>	treatment of gastric cancer	Phase III (919) 361-2162
		treatment of adenocarcinoma of the pancreas	Phase III (919) 361-2162
gimatecan	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of malignant glioma	Phase I/II (888) 669-6682
girentuximab	Wilex Biotechnology <i>Munich, Germany</i>	treatment of renal cell carcinoma	Phase III www.wilex.com
GL-0810 (HPV-16 peptide vaccine)	Gliknik <i>Baltimore, MD</i>	treatment of HPV-16 expressing head and neck squamous cell carcinoma	Phase I www.gliknik.com
GL-0817 (mage-A3 peptide vaccine)	Gliknik <i>Baltimore, MD</i>	treatment of MAGE-A3 expressing head and neck squamous cell carcinoma	Phase I www.gliknik.com
<b>GliAtak</b> <sup>™</sup> cancer gene therapy	Advantagene <i>Waban, MA</i>	treatment of malignant brain tumors	Phase I/II (617) 916-5445
<b>Glionix</b> <sup>®</sup> belagen-pumatucl-L (cancer vaccine)	NovaRx <i>San Diego, CA</i>	treatment of astrocytic tumors	Phase I (858) 552-8600
glufosfamide	Threshold Pharmaceuticals <i>Redwood City, CA</i>	treatment of pancreatic cancer (Fast Track)	Phase III completed (650) 474-8200
<b>GVAX</b> <sup>®</sup> Pancreatic	BioSante Pharmaceuticals <i>Lincolnshire, IL</i>	treatment of pancreatic cancer	Phase II (847) 478-0500
<b>HyperAcute</b> <sup>®</sup> <b>Pancreas</b> algenpantucel-L	NewLink Genetics <i>Ames, IA</i>	treatment of pancreatic cancer	Phase III (515) 296-3520
ICT-107 (dendritic cell-based vaccine)	ImmunoCellular Therapeutics <i>Woodland Hills, CA</i>	treatment of glioblastoma or brain stem glioma	Phase I (818) 992-2907

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Product Name	Sponsor	FDA Official Designation	Development Status
indium-111 pentetreotide	Radio Isotope Therapy of America <i>Houston, TX</i>	treatment of neuroendocrine tumors	Phase II/III (713) 781-6200
irofulven (E7850)	Eisai <i>Woodcliff Lake, NJ</i>	treatment of ovarian cancer	Phase II (888) 422-4743
IRX-2 (human leukocyte-derived cytokine mixture)	IRX Therapeutics <i>New York, NY</i>	neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck (Fast Track)	Phase II (212) 582-1199
<b>Junovan™</b> mifamuride	Millennium: The Takeda Oncology Company <i>Cambridge, MD</i>	treatment of osteosarcoma	Phase III (800) 390-5663
lansoprazole	Apricus Biosciences <i>San Diego, CA</i> FasTrack Pharmaceuticals <i>San Diego, CA</i>	treatment of hepatocellular carcinoma	Phase II (858) 222-8041 (858) 348-4630
linifanib	Abbott Laboratories <i>Abbott Park, IL</i>	treatment of hepatocellular carcinoma	Phase III (847) 937-6100
LOR-2040	Lorus Therapeutics <i>Toronto, Ontario</i>	treatment of renal cell carcinoma (see also cancer, blood)	Phase II (416) 798-1200
lorvotuzumab mertansine (IMGN901)	ImmunoGen <i>Waltham, MA</i>	treatment of Merkel cell carcinoma	Phase II (781) 895-0600
		treatment of multiple myeloma	Phase II (781) 895-0600
melphalan (percutaneous hepatic perfusion delivery system)	Delcath Systems <i>New York, NY</i>	treatment of neuroendocrine tumors (see also cancer, skin)	Phase II completed (212) 489-2100
MLN-8237	Millennium, The Takeda Oncology Company <i>Cambridge, MA</i>	treatment of ovarian cancer	Phase II (800) 390-5663
MSC-1936369B (MEK inhibitor)	EMD Serono <i>Rockland, MA</i>	treatment of patients with pancreatic cancer	Phase I/II (800) 283-8088
<b>Multikine®</b> leukocyte interleukin	CEL-SCI <i>Vienna, VA</i>	neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck	Phase II (703) 506-9460
NGR-hTNF	MolMed <i>Milan, Italy</i>	treatment of malignant pleural mesothelioma	Phase III www.molmed.com
nimotuzumab	YM Biosciences USA <i>Lehigh Valley, PA</i>	treatment of glioma	Phase II (610) 560-0600
NTX-010	Neotropix <i>Malvern, PA</i>	treatment of neuroendocrine tumors	Phase I (617) 460-1200
<b>Onalta™</b> Yttrium90-labeled edotreotide	Molecular Insight Pharmaceuticals <i>Cambridge, MA</i>	treatment of somatostatin receptor-positive neuroendocrine gastroenteropancreatic tumors	Phase II (617) 492-5554

# CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Oncolar</b> <sup>®</sup> octreotide	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of neuroendocrine tumors	Phase III (888) 669-6682
<b>Oncophage</b> <sup>®</sup> vitespen	Antigenics <i>New York, NY</i>	treatment of renal cell carcinoma (Fast Track) (see also cancer, skin)	Phase II (212) 994-8200
		treatment of glioma	Phase II (212) 994-8200
oregovomab	Quest PharmaTech <i>Edmonton, Canada</i>	treatment of epithelial ovarian cancer	Phase I/II (780) 448-1400
<b>OVax</b> <sup>®</sup>	AVAX Technologies <i>Philadelphia, PA</i>	adjuvant therapy for the treatment of ovarian cancer	Phase I/II (215) 241-9760
paclitaxel aqueous gel	Protherics (BTG) <i>Brentwood, TN</i>	treatment of esophageal cancer	Phase II (615) 327-1027
palifosfamide	ZIOPHARM Oncology <i>New York, NY</i>	treatment of soft tissue sarcomas	Phase III (646) 214-0700
pazopanib	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of soft tissue sarcomas	Phase III (888) 825-5249
PEG-arginine deiminase	Polaris Pharmaceuticals <i>San Diego, CA</i>	treatment of hepatocellular carcinoma (see also cancer, skin)	Phase II (858) 452-6688
<b>Photofrin</b> <sup>®</sup> porfimer sodium	Axcan Pharma <i>Birmingham, AL</i>	for the photodynamic therapy of patients with transitional cell carcinoma in situ of the urinary bladder	Phase I/II (800) 950-8085
Poly-ICLC	Oncovir <i>Washington, DC</i>	treatment of primary brain tumors	Phase II www.oncovir.com
regorafenib	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i>	treatment of gastrointestinal stromal tumors	Phase III (888) 842-2937
<b>Rexin-G</b> <sup>™</sup> targeted nanomedicine	Epeius Biotechnologies <i>San Marino, CA</i>	treatment of osteosarcoma	Phase II (626) 441-6695
		treatment of pancreatic cancer (Fast Track)	Phase I/II (626) 441-6695
		treatment of soft tissue sarcoma	Phase I/II (626) 441-6695
ridaforolimus	Merck <i>Whitehouse Station, NJ</i>	treatment of bone sarcoma (Fast Track)	Phase III (800) 672-6372
		treatment of soft tissue sarcoma (Fast Track)	Phase III (800) 672-6372
rindapepimut	Celldex Therapeutics <i>Needham, MA</i>	treatment of EGFRvIII-expressing glioblastoma multiforme (Fast Track)	Phase II (781) 433-0771

# CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
S1 (tegafur/gimeracil/ oteracil)	Taiho Pharma USA <i>Princeton, NJ</i>	treatment of gastric cancer	Phase III (609) 750-5300
salirasib	Concordia Pharmaceuticals <i>Ft. Lauderdale, FL</i>	treatment of pancreatic cancer	Phase I/II (954) 357-1823
SP1049C	Supratek Pharma <i>Montreal, Canada</i>	treatment of esophageal carcinoma	Phase II (514) 849-6094
		treatment of gastric cancer	Phase II (514) 849-6094
squalamine	Ohr Pharmaceuticals <i>New York, NY</i>	treatment of ovarian cancer refractory or resistant to standard chemotherapy	Phase II completed (212) 682-8452
talactoferrin alfa	Agennix <i>Houston, TX</i>	treatment of non-small-cell lung cancer (Fast Track)	Phase III (713) 552-1091
		treatment of renal cell carcinoma (Fast Track)	Phase II (713) 552-1091
<b>Tarceva</b> <sup>®</sup> erlotinib	Genentech <i>South San Francisco, CA</i>	treatment of malignant gliomas	Phase II (650) 225-1000
<b>Tasigna</b> <sup>®</sup> nilotinib	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of gastrointestinal stromal tumors	Phase III (888) 669-6682
telatinib	ACT Biotech <i>San Francisco, CA</i>	treatment of gastric cancer	Phase II (415) 230-3900
tesetaxel	Genta <i>Berkeley Heights, NJ</i>	treatment of gastric cancer (Fast Track) (see also cancer, skin)	Phase II (908) 286-9800
<b>ThermoDox</b> <sup>®</sup> heat-activated liposomal encapsulated doxorubicin	Celsion <i>Columbia, MD</i>	treatment of hepatocellular carcinoma	Phase III www.celsion.com
TLC-388	TLC Biopharmaceuticals <i>South San Francisco, CA</i>	treatment of hepatocellular carcinoma (HCC)	Phase I (650) 872-8816
TM601 (unlabeled)	TransMolecular <i>King of Prussia, PA</i>	treatment of malignant glioma	Phase I (610) 768-8041
TM601-I-131 (intracranial)	TransMolecular <i>King of Prussia, PA</i>	treatment of malignant glioma	Phase II (610) 768-8041
TM601-I-131 (intravenous)	TransMolecular <i>King of Prussia, PA</i>	treatment of malignant glioma (see also cancer, skin)	Phase I/II (610) 768-8041
Toca-511 (cytosine deaminase gene therapy)	Tocagen <i>San Diego, CA</i>	treatment of recurrent glioblastoma multiforme	Phase I/II (858) 412-8400
<b>Trisenox</b> <sup>®</sup> arsenic trioxide	Cephalon <i>Frazer, PA</i>	treatment of malignant glioma (see also cancer, blood)	Phase I (610) 344-0200

# CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
TVI-Brain-1	TVAX Biomedical <i>Lenexa, KS</i>	treatment of central nervous system malignancies	Phase I/II (913) 492-2221
<b>Tykerb®</b> lapatinib	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of ErbB2-positive gastric cancer	Phase III (888) 825-5249
		treatment of ErbB2-positive esophageal cancer	Phase III (888) 825-5249
vandetanib	AstraZeneca <i>Wilmington, DE</i>	treatment of patients with follicular thyroid carcinoma, medullary thyroid carcinoma, anaplastic thyroid carcinoma, and locally advanced and metastatic papillary thyroid carcinoma (Fast Track)	application submitted (800) 236-9933
VB4-845	Viventia Biotechnologies <i>Mississauga, Canada</i>	treatment of Ep-CAM-positive squamous cell carcinoma of the head and neck (Fast Track)	Phase II (905) 362-2973
veliparib	Abbott Laboratories <i>Abbott Park, IL</i>	treatment of epithelial ovarian cancer in combination with DNA-damaging agents (see also cancer, skin)	Phase II (847) 937-6100
		treatment of hepatocellular carcinoma in combination with DNA-damaging agents	Phase I (847) 937-6100
verubulin (MPC-6827)	Myrexis <i>Salt Lake City, UT</i>	treatment of glioblastoma multiforme	Phase II (801) 214-7800
virulizin	Zor Pharmaceuticals <i>Philadelphia, PA</i>	treatment of pancreatic cancer	Phase III (609) 718-2305
<b>Xcytrin®</b> motexafin gadolinium	Pharmacyclics <i>Sunnyvale, CA</i>	for use in conjunction with whole brain radiation for the treatment of brain metastases arising from solid tumors	Phase III (408) 774-0330
<b>Yondelis®</b> trabectedin	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	treatment of patients with epithelial ovarian cancer	application submitted (800) 817-5286
		treatment of soft tissue sarcoma	Phase III (800) 817-5286
<b>Zadaxin®</b> thymalfasin	SciClone Pharmaceuticals <i>San Mateo, CA</i>	treatment of hepatocellular carcinoma (see also cancer, skin; infectious diseases)	Phase II/III (650) 358-3456
<b>Zolinza®</b> vorinostat	Merck <i>Whitehouse Station, NJ</i>	treatment of mesothelioma (see also cancer, blood)	Phase III (800) 672-6372

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Zybrestat™</b> fosbretabulin	OXiGENE <i>South San Francisco, CA</i>	treatment of anaplastic thyroid cancer, medullary thyroid cancer, and stage IV papillary or follicular thyroid cancer (Fast Track)	Phase III (650) 635-7000
		treatment of ovarian cancer	Phase II (650) 635-7000

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
AC220 (quizartinib)	Ambit Biosciences <i>San Diego, CA</i>	treatment of acute myeloid leukemia	Phase II (858) 334-2100
<b>Afinitor®</b> everolimus	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of Waldenstrom macroglobulinemia (also known as lymphoplasmacytic lymphoma) (see also cancer)	Phase I/II (888) 669-6682
AFM-13 (recombinant antibody against CD30 and CD16a)	Affimed Therapeutics <i>Heidelberg, Germany</i>	treatment of Hodgkin lymphoma	Phase I www.affimed.com
alvocidib	sanofi-aventis <i>Bridgewater, NJ</i>	treatment of B-cell chronic lymphocytic leukemia (B-CLL) or prolymphocytic leukemia arising from CLL	Phase II (800) 633-1610
<b>Amplimexon®</b> imexon	AmpliMed <i>Tucson, AZ</i>	treatment of multiple myeloma (see also cancer, skin)	Phase I/II completed (520) 529-1000
AP24534 (ponatinib)	Ariad Pharmaceuticals <i>Cambridge, MA</i>	treatment of chronic myeloid leukemia	Phase I (617) 494-0400
		treatment of Philadelphia chromosome-positive acute lymphoblastic leukemia	Phase I (617) 494-0400
AS-1411	Antisoma <i>Cambridge, MA</i>	treatment of acute myeloid leukemia	Phase II (617) 252-6100
AS-1413	Antisoma <i>Cambridge, MA</i>	treatment of acute myeloid leukemia (Fast Track)	Phase III (617) 252-6100
AT-101	Ascenta Therapeutics <i>Malvern, PA</i>	treatment of chronic lymphocytic leukemia	Phase II (610) 408-0301
AT9283	Astex Therapeutics <i>Cambridge, United Kingdom</i>	treatment of acute myeloid leukemia	Phase I/II www.astex-therapeutics.com
AZD1152	AstraZeneca Pharmaceuticals <i>Wilmington, DE</i>	treatment of acute myeloid leukemia	Phase II (800) 236-9933

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
bafetinib	CytRx <i>Los Angeles, CA</i>	treatment of Philadelphia chromosome-positive chronic myelogenous leukemia	Phase I (310) 826-5648
belinostat	Spectrum Pharmaceuticals <i>Irvine, CA</i>	treatment of peripheral T-cell lymphoma (Fast Track)	Phase III (949) 788-6700
BI-505	BioInvent International <i>Lund, Sweden</i>	treatment of multiple myeloma	Phase I www.bioinvent.com
<b>BiovaxID®</b>	Biovest International <i>Tampa, FL</i>	treatment of follicular lymphoma (Fast Track)	Phase III (813) 864-2558
		treatment of mantle cell lymphoma	Phase II (813) 864-2558
blinatumomab (MT-103)	Micromet <i>Bethesda, MD</i>	treatment of acute lymphocytic leukemia	Phase II (240) 752-1420
BP-100-1-01	Bio-Path <i>Houston, TX</i>	treatment of chronic myelogenous leukemia	Phase I (832) 971-6616
brentuximab vedotin (SGN-35)	Seattle Genetics <i>Bothell, WA</i>	treatment of Hodgkin's lymphoma	Phase III (425) 527-4000
		treatment of anaplastic large-cell lymphoma	Phase II (425) 527-4000
BT-062	Biotest AG <i>Dreieich, Germany</i>	treatment of multiple myeloma	Phase I/II www.biotest.com
cancer vaccine PR1	The Vaccine Company <i>South San Francisco, CA</i>	treatment of acute myelogenous leukemia	Phase III (650) 244-4100
		treatment of myelodysplastic syndromes requiring therapy	Phase II (650) 244-4100
		treatment of chronic myelogenous leukemia	Phase II (650) 244-4100
carfilzomib	Onyx Therapeutics <i>Emeryville, CA</i>	treatment of multiple myeloma	Phase III (510) 597-6500
CAT-8015	MedImmune <i>Gaithersburg, MD</i>	treatment of CD22-positive chronic lymphocytic leukemia	Phase I/II (301) 398-0000
		treatment of hairy cell leukemia	Phase I (301) 398-0000
cenersen	Eleos <i>Omaha, NE</i>	treatment of acute myeloid leukemia	Phase II (402) 393-3034
		treatment of chronic lymphocytic leukemia	Phase II (402) 393-3034
<b>Ceplene™</b> histamine	EpiCept <i>Tarrytown, NY</i>	adjunct to cytokine therapy in the treatment of acute myeloid leukemia	application submitted (914) 606-3500

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Clolar</b> <sup>®</sup> clofarabine	Genzyme <i>Cambridge, MA</i>	treatment of acute myelogenous leukemia	application submitted (617) 252-7500
CPX-351 (cytarabine/ daunorubicin)	Celator Pharmaceuticals <i>Princeton, NJ</i>	treatment of acute myeloid leukemia	Phase II (609) 243-0123
dacetuzumab (SGN-40)	Seattle Genetics <i>Bothell, WA</i>	treatment of chronic lymphocytic leukemia	Phase I/II (425) 527-4000
		treatment of multiple myeloma	Phase I (425) 527-4000
<b>Dacogen</b> <sup>®</sup> decitabine	Eisai <i>Woodcliff Lake, NJ</i>	treatment of acute myeloid leukemia	Phase III (888) 422-4743
darinaparsin	ZIOPHARM Oncology <i>New York, NY</i>	treatment of peripheral T-cell lymphoma	Phase II (646) 214-0700
DCC-2036	Deciphera Pharmaceuticals <i>Lawrence, KS</i>	treatment of Philadelphia chromosome-positive chronic myeloid leukemia	Phase I/II (785) 830-2100
dovitinib	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of multiple myeloma	Phase II (888) 669-6682
elacytarabine	Clavis Pharma <i>Oslo, Norway</i>	treatment of acute myeloid leukemia	Phase III www.clavispharma.com
ENMD-2076	EntreMed <i>Rockville, MD</i>	treatment of acute myeloid leukemia (see also cancer)	Phase I (240) 864-2600
		treatment of multiple myeloma	Phase I (240) 864-2600
enzastaurin	Eli Lilly <i>Indianapolis, IN</i>	treatment of diffuse large B-cell lymphoma	Phase III (800) 545-5979
epratuzumab	Immunomedics <i>Morris Plains, NJ</i>	treatment of acute lymphoblastic leukemia	Phase II (973) 605-8200
		treatment of non-Hodgkin's lymphoma	Phase II (973) 605-8200
<b>Erwinase</b> <sup>®</sup> crisantaspase	EUSA Pharma <i>Langhorne, PA</i>	treatment of acute lymphocytic leukemia	application submitted (800) 833-2533
<b>Estybon</b> <sup>™</sup> (ON 01910.Na)	Onconova Therapeutics <i>Newton, PA</i>	treatment of myelodysplastic syndromes	Phase III (267) 759-3680
<b>Fodosine</b> <sup>™</sup> forodesine	BioCryst Pharmaceuticals <i>Birmingham, AL</i>	treatment of acute lymphoblastic leukemia	Phase I (205) 444-4600
<b>Foloty</b> <sup>™</sup> pralatrexate	Allos Therapeutics <i>Westminster, CO</i>	treatment of advanced or metastatic transitional cell carcinoma of the urinary bladder	Phase II (303) 426-6262

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Genasense®</b> oblimersen	Genta <i>Berkeley Heights, NJ</i>	treatment of chronic lymphocytic leukemia (Fast Track) (see also cancer, skin)	application submitted (908) 286-9800
		treatment of acute myelocytic leukemia	Phase III (908) 286-9800
<b>GVAX® Leukemia</b>	BioSante Pharmaceuticals <i>Lincolnshire, IL</i>	treatment of acute myeloid leukemia	Phase II (847) 478-0500
GX15-070 (obatoclast)	Gemin X Biotechnologies <i>Malvern, PA</i>	treatment of chronic lymphocytic leukemia	Phase I/II (514) 281-8989
ISF35	Memgen <i>Dallas, TX</i>	treatment of chronic lymphocytic leukemia	Phase II (214) 731-3141
KW-0761	Kyowa Hakko Kirin Pharma <i>Princeton, NJ</i>	treatment of patients with cutaneous T-cell lymphoma	Phase II (609) 919-1100
		treatment of peripheral T-cell lymphoma	Phase II (609) 919-1100
L-annamycin	Callisto Pharmaceuticals <i>New York, NY</i>	treatment of acute myeloid leukemia	Phase I/II (212) 297-0010
		treatment of acute lymphoblastic leukemia	Phase I/II (212) 297-0010
L-asparaginase	ERYtech Pharma <i>Philadelphia, PA</i>	treatment of acute lymphoblastic leukemia	Phase II/III (267) 499-2031
lestaurtinib (CEP-701)	Cephalon <i>Frazer, PA</i>	treatment of acute myeloid leukemia	Phase II (610) 344-0200
		treatment of Philadelphia-negative classic myeloproliferative disorders	Phase II (610) 344-0200
LOR-2040	Lorus Therapeutics <i>Toronto, Ontario</i>	treatment of acute myeloid leukemia (see also cancer)	Phase II (416) 798-1200
<b>Marqibo®</b> vincristine sulfate liposome injection	Talon Therapeutics <i>South San Francisco, CA</i>	treatment of acute lymphoblastic leukemia (Fast Track) (see also cancer, skin)	Phase II (650) 588-6404
melphalan intravenous	CyDex Pharmaceuticals <i>Lenexa, KS</i>	high dose conditioning treatment prior to hematopoietic progenitor (stem) cell transplantation	Phase II (888) 436-6910
midostaurin	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of acute myeloid leukemia (see also other)	Phase III (888) 669-6682
milatuzumab	Immunomedics <i>Morris Plains, NJ</i>	treatment of chronic lymphocytic leukemia	Phase I/II (973) 605-8200
		treatment of multiple myeloma	Phase I/II (973) 605-8200

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
naloxone lotion (E-0316)	Elorac <i>Vernon Hills, IL</i>	treatment of pruritus accompanying cutaneous T-cell lymphoma	Phase II completed (847) 362-8200
<b>NeoFuse</b> <sup>®</sup> mesenchymal stem cell therapy	Angioblast Systems <i>New York, NY</i> Mesoblast <i>Melbourne, Australia</i>	treatment of insufficient hematopoietic stem cell production in patients with hematologic malignancies who have failed treatment with conventional chemotherapy	Phase II (212) 880-2060
<b>Nipent</b> <sup>®</sup> pentostatin	Hospira <i>Lake Forest, IL</i>	treatment of patients with chronic lymphocytic leukemia	Phase III (800) 615-0187
<b>Omapro</b> <sup>™</sup> omacetaine mepesuccinate	ChemGenex Pharmaceuticals <i>Menlo Park, CA</i>	treatment of chronic myelogenous leukemia (Fast Track)	application submitted (650) 804-7660
		treatment of myelodysplastic syndromes	Phase II (650) 804-7660
panobinostat	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of Hodgkin's lymphoma	Phase III (888) 669-6682
perifosine	Keryx Biopharmaceuticals <i>New York, NY</i>	treatment of multiple myeloma	Phase III (212) 531-5965
plitidepsin	PharmaMar USA <i>Cambridge, MA</i>	treatment of multiple myeloma	Phase III (617) 621-5300
pomalidomide	Celgene <i>Summit, NJ</i>	treatment of multiple myeloma (see also cancer-related conditions)	Phase II (908) 673-9000
<b>Revlimid</b> <sup>®</sup> lenalidomide	Celgene <i>Summit, NJ</i>	treatment of chronic lymphocytic leukemia	Phase III (908) 673-9000
		treatment of mantle cell lymphoma	Phase III (908) 673-9000
sapacitabine	Cyclacel Pharmaceuticals <i>Berkeley Heights, NJ</i>	treatment of acute myelogenous leukemia	Phase II (908) 517-7330
		treatment of myelodysplastic syndrome	Phase II (908) 517-7330
SB1518	S*BIO <i>Redwood City, CA</i>	treatment of myeloproliferative disorders with the JAK2 V617F mutation	Phase II (650) 730-2860
SF1126	Semafore Pharmaceuticals <i>Westfield, IN</i>	treatment of B-cell chronic lymphocytic leukemia	Phase I (317) 867-1126
		treatment of chronic lymphocytic leukemia (CLL)	Phase I (317) 867-1126
siltuximab	Centocor Ortho Biotech <i>Horsham, PA</i>	treatment of Castleman's disease	Phase II (800) 457-6399
		treatment of multiple myeloma	Phase II (800) 457-6399

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
<b>StemEx</b> <sup>®</sup> carlecortemcel-L	Gamida Cell <i>Jerusalem, Israel</i> Teva Pharmaceuticals USA <i>North Wales, PA</i>	for use as hematopoietic support in patients with chronic myeloid leukemia (Fast Track)	Phase II/III (215) 591-3000
		for use as hematopoietic support in patients with myelodysplastic syndromes (Fast Track)	Phase II/III (215) 591-3000
		for use as hematopoietic support in patients with relapsed or refractory hematologic malignancies who are receiving high-dose therapy (Fast Track)	Phase II/III (215) 591-3000
tamibarotene	CytRx <i>Los Angeles, CA</i>	treatment of acute promyelocytic leukemia (Fast Track)	Phase II (310) 826-5648
<b>Tasigna</b> <sup>®</sup> nilotinib	Novartis Pharmaceutical <i>East Hanover, NJ</i>	treatment of gastrointestinal stromal tumors	Phase II (888) 669-6682
<b>Thymoglobulin</b> <sup>®</sup> anti-thymocyte globulin (rabbit)	Genzyme Transplant <i>Cambridge, MA</i>	treatment of myelodysplastic syndrome	Phase II (888) 764-7828
tosedostat	Chroma Therapeutics <i>Abingdon, United Kingdom</i>	treatment of acute myeloid leukemia	Phase II www.chromatherapeutics.com
<b>Trisenox</b> <sup>®</sup> arsenic trioxide	Cephalon <i>Frazer, PA</i>	treatment of acute myelocytic leukemia subtypes M0, M1, M2, M4, M5, M6, and M7 (see also cancer)	Phase III www.cephalon.com (610) 344-0200
		treatment of multiple myeloma	Phase II www.cephalon.com (610) 344-0200
		treatment of myelodysplastic syndrome	Phase II www.cephalon.com (610) 344-0200
<b>Velcade</b> <sup>®</sup> bortezomib	Millennium, The Takeda Oncology Company <i>Cambridge, MA</i>	treatment of follicular non-Hodgkin lymphoma	Phase III (800) 390-5663
veltuzumab	Immunomedics <i>Morris Plains, NJ</i>	treatment of chronic lymphocytic leukemia	Phase I/II (973) 605-8200
<b>Vidaza</b> <sup>®</sup> azacitidine	Celgene <i>Summit, NJ</i>	treatment of acute myeloid leukemia	Phase III (908) 673-9000
vosaroxin	Sunesis Pharmaceuticals <i>South San Francisco, CA</i>	treatment of acute myeloid leukemia	Phase II (650) 266-3500
<b>XmAb</b> <sup>®</sup> 2513	Xencor <i>Monrovia, CA</i>	treatment of Hodgkin lymphoma	Phase I (626) 305-5900

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
zanolimumab	TenX Biopharma <i>Philadelphia, PA</i>	treatment of mycosis fungoides (cutaneous T-cell lymphoma) (Fast Track)	Phase III (215) 774-1176
<b>Zolinza</b> <sup>®</sup> vorinostat	Merck <i>Whitehouse Station, NJ</i>	treatment of multiple myeloma (see also cancer)	Phase III (800) 672-6372
zosuquidar trihydrochloride	Kanisa Pharmaceuticals <i>San Diego, CA</i>	treatment of acute myeloid leukemia	Phase III completed (858) 436-1800

## CANCER, SKIN

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Abraxane</b> <sup>®</sup> paclitaxel protein-bound particles for injectable suspension, albumin-bound	Celgene <i>Summit, NJ</i>	treatment of stage IIb to IV melanoma (see also cancer)	Phase III (908) 673-9000
<b>Allovetin-7</b> <sup>®</sup> velimogene aliplasmid	Vical <i>San Diego, CA</i>	treatment of invasive and metastatic melanoma (stages II, III, and IV)	Phase III (858) 646-1100
ALS-357	Advanced Life Sciences <i>Woodridge, IL</i>	topical treatment of metastatic melanoma	Phase I (630) 739-6744
<b>Amplimexon</b> <sup>®</sup> imexon	AmpliMed <i>Tucson, AZ</i>	treatment of metastatic malignant melanoma (see also cancer, blood)	Phase I/II completed (520) 529-1000
astuprotimut-R	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of MAGE-A3-positive stages IIb to IV malignant melanoma (see also cancer)	Phase III (888) 825-5249
<b>Avastin</b> <sup>®</sup> bevacizumab	Genentech <i>South San Francisco, CA</i>	treatment of melanoma stages IIb through IV as part of a combination chemotherapy regimen (see also cancer)	Phase II (650) 225-1000
BCL-005 (fused-hybrid melanoma-dendritic cells)	BruCells <i>Brussels, Belgium</i>	treatment of stage IIb to IV melanoma in HLA-A2 positive patients	Phase I www.brucells.com
<b>Clearazide</b> <sup>™</sup> clorethamine	Yaupon Therapeutics <i>Radnor, PA</i>	treatment of mycosis fungoides	Phase II/III (610) 975-9290
denenicokin (rIL-21)	ZymoGenetics <i>Seattle, WA</i>	treatment of stage II (T4), III or IV malignant melanoma	Phase II (206) 442-6600
<b>Genasense</b> <sup>®</sup> oblimersen	Genta <i>Berkeley Heights, NJ</i>	treatment of advanced malignant melanoma (stages II, III, IV) (Fast Track) (see also cancer, blood)	Phase III (908) 286-9800

# CANCER, SKIN

Product Name	Sponsor	FDA Official Designation	Development Status
GSK1120212	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of BRAF V600 mutation positive stage IIB through IV melanoma	Phase III (888) 825-5249
GSK2118436	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of stage IIb through stage IV melanoma	Phase III (888) 825-5249
intetumumab	Centocor Ortho Biotech <i>Horsham, PA</i>	treatment of patients with high-risk stage II, stage III, and stage IV malignant melanoma	Phase I/II (610) 651-6000
ipilimumab	Bristol-Myers Squibb <i>Princeton, NJ</i> Medarex <i>Princeton, NJ</i>	treatment of high-risk stage II, stage III, and stage IV melanoma	application submitted (212) 546-4000
<b>Marqibo®</b> vincristine sulfate liposome injection	Talon Therapeutics <i>South San Francisco, CA</i>	treatment of metastatic uveal melanoma (see also cancer, blood)	Phase II (650) 588-6404
melanoma peptide vaccine (MDX-010+ MDX1379) (ipilimumab+ disomotide/ ovemotide)	Bristol-Myers Squibb <i>Princeton, NJ</i> Medarex <i>Princeton, NJ</i>	treatment of HLA-A2+m patients with stage IIB, IIC, III and IV malignant melanoma	Phase III (212) 546-4000
melphalan (percutaneous hepatic perfusion delivery system)	Delcath Systems <i>New York, NY</i>	treatment of patients with cutaneous melanoma	application submitted (212) 489-2100
		treatment of patients with ocular (uveal) melanoma	application submitted (212) 489-2100
muparfostat (PI-88)	Medigen Biotechnology <i>Taipei, Taiwan</i>	treatment of high-risk stage II, stage III, and stage IV melanoma	Phase II (650) 610-7800
<b>Oncophage®</b> vitespen	Antigenics <i>New York, NY</i>	treatment of metastatic melanoma (Fast Track) (see also cancer)	Phase III (212) 994-8200
PEG-arginine deiminase	Polaris Pharmaceuticals <i>San Diego, CA</i>	treatment of invasive malignant melanoma (see also cancer)	Phase II (858) 452-6688
<b>PEG-Intron A®</b> peginterferon alfa-2b	Merck <i>Whitehouse Station, NJ</i>	treatment of malignant melanoma stages IIb through IV	application submitted (800) 672-6372
POL-103A	Polynoma <i>San Diego, CA</i>	treatment of stage IIb to stage IV melanoma	Phase II (858) 205-2501
PV-10	Provectus Pharmaceuticals <i>Knoxville, TN</i>	treatment of metastatic melanoma	Phase II (866) 594-5999
RG7256	Roche <i>Nutley, NJ</i>	treatment of patients with IIb or stage IV melanoma positive for the BRAF(v600) mutation	Phase I (973) 235-5000

## CANCER, SKIN

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Solazed™</b> ioflubenzamide I-131	Molecular Insight Pharmaceuticals <i>Cambridge, MA</i>	treatment of metastatic melanoma, stages IIB, IIC, III and IV	Phase I (617) 492-5554
tasisulam	Eli Lilly <i>Indianapolis, IN</i>	treatment of melanoma stages IIB through IV	Phase III (800) 545-5979
tesetaxel	Genta <i>Berkeley Heights, NJ</i>	treatment of stages IIB, IIC, III, and stage IV melanoma (see also cancer)	Phase II (908) 286-9800
TM601-I-131 (intravenous)	TransMolecular <i>King of Prussia, PA</i>	treatment of stage IIb, IIc, III & IV melanoma (see also cancer)	Phase I/II (610) 768-8041
tremelimumab	Pfizer <i>New York, NY</i>	treatment of stage IIb to stage IV metastatic melanoma	Phase III (860) 732-5156
veliparib	Abbott Laboratories <i>Abbott Park, IL</i>	treatment of malignant melanoma stages IIb through IV (see also cancer)	Phase II (847) 937-6100
<b>Zadaxin®</b> thymalfasin	SciClone Pharmaceuticals <i>San Mateo, CA</i>	treatment of stage IIb through stage IV malignant melanoma (see also cancer, infectious diseases)	Phase II (650) 358-3456

## CANCER-RELATED CONDITIONS

Product Name	Sponsor	FDA Official Designation	Development Status
benzylamine oral rinse	Angelini Pharmaceuticals <i>Rome, Italy</i> Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	prophylactic treatment of oral mucositis resulting from radiation therapy for head and neck cancer	Phase III (800) 817-5286
<b>Leukotac®</b> inolimomab	EUSA Pharma <i>Langhorne, PA</i>	treatment of graft-versus-host disease	Phase II (215) 867-4900
<b>MultiStem®</b> stem cell therapy	Athersys <i>Cleveland, OH</i>	treatment and prophylaxis of graft-versus-host disease	Phase I (216) 431-9900
pegsitacase	3SBio <i>Shenyang, China</i>	prophylaxis of hyperuricemia in cancer patients prone to develop tumor lysis syndrome during chemotherapy (see also other)	Phase I www.3sbio.com
		treatment of tumor lysis syndrome in cancer patients undergoing chemotherapy	Phase I www.3sbio.com
		treatment of Lesch-Nyhan syndrome	Phase I www.3sbio.com

## CANCER-RELATED CONDITIONS

Product Name	Sponsor	FDA Official Designation	Development Status
pomalidomide	Celgene <i>Summit, NJ</i>	treatment of persons with myeloproliferative neoplasm-associated myelofibrosis and anemia who are red blood cell transfusion dependent (see also cancer, blood)	Phase III (908) 673-9000
<b>Prochymal</b> <sup>®</sup> human mesenchymal stem cell therapy	Osiris Therapeutics <i>Columbia, MD</i>	treatment of acute graft-versus-host disease (Fast Track) (see also autoimmune disorders)	Phase III (443) 545-1800
sodium thiosulfate	Adherex Technologies <i>Durham, NC</i>	prevention of platinum-induced ototoxicity in pediatric patients	Phase III (919) 484-8484
uridine triacetate (PN-401)	Wellstat Therapeutics <i>Gaithersburg, MD</i>	an antidote in the treatment of 5-fluorouracil poisoning	in clinical trials (240) 631-2500
<b>Voraxaze</b> <sup>™</sup> glucarpidase	Protherics (BTG) <i>Brentwood, TN</i>	treatment of patients at risk of methotrexate toxicity (Fast Track)	application submitted (615) 327-1027
<b>Xerecept</b> <sup>®</sup> corticotropin acetate injection	Celtic Pharma <i>Hamilton, Bermuda</i>	treatment of peritumoral brain edema	Phase III completed (212) 616-4000

## CARDIOVASCULAR DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
PRT-201 (recombinant human type 1 pancreatic elastase)	Proteon Therapeutics <i>Waltham, MA</i>	prevention of arteriovenous fistula maturation and arteriovenous graft failure in patients with end-stage renal disease who are receiving hemodialysis or preparing for hemodialysis (Fast Track)	Phase I/II (781) 890-0102
recombinant P-selectin glycoprotein ligand (YSPSL)	Y's Therapeutics <i>San Bruno, CA</i>	prevention of ischemic reperfusion injury in all solid organ transplants (see also transplantation)	Phase II (650) 777-7000
TAL-05-00018 (human plasmin)	Talecris Biotherapeutics <i>Rsch. Triangle Park, NC</i>	treatment of acute peripheral arterial occlusion	Phase I/II completed (919) 316-6300
tissue repair stem cell therapy	Aastrom Biosciences <i>Ann Arbor, MI</i>	treatment of dilated cardiomyopathy (see also blood disorders)	Phase II (734) 930-5555
<b>Trinam</b> <sup>®</sup> taberminogene vadenovec	Ark Therapeutics <i>London, United Kingdom</i>	prevention of complications due to neointimal hyperplasia disease in certain vascular anastomoses (Fast Track)	Phase II/III <a href="http://www.arktherapeutics.com">www.arktherapeutics.com</a>
<b>Vascugel</b> <sup>®</sup> peripheral vascular disorder endothelial cell therapy	Pervasis Therapeutics <i>Cambridge, MA</i>	prevention of arteriovenous fistula or arteriovenous graft failure in patients with end-stage renal disease receiving hemodialysis or preparing for hemodialysis	Phase II (617) 621-3407

## EYE DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
CNTO-2476 (human umbilical tissue-derived cells)	Centocor Ortho Biotech <i>Horsham, PA</i>	treatment of retinitis pigmentosa	Phase I (800) 457-6399
<b>Durezol™</b> difluprednate	Alcon Research <i>Fort Worth, TX</i>	treatment of endogenous and traumatic anterior uveitis and panuveitis	application submitted (817) 293-0450
LIPO-102	Lithera <i>San Diego, CA</i>	treatment of symptomatic exophthalmos associated with thyroid related eye disease	Phase II (858) 750-1008
<b>Luveniq™</b> voclosporin	Lux Biosciences <i>Jersey City, NJ</i>	treatment and chronic control of non-infectious posterior, intermediate and pan-uveitis (Fast Track)	application submitted (201) 946-0551
microplasmin	ThromboGenics <i>New York, NY</i>	adjunct to surgery in cases of pediatric vitrectomy	Phase II (212) 201-0920
<b>Nexagon®</b> antisense oligonucleotide	CoDa Therapeutics <i>San Diego, CA</i>	treatment of persistent corneal epithelial defects	Phase I completed (858) 677-0474
NT-501	Neurotech USA <i>Lincoln, RI</i>	treatment of retinitis pigmentosa (Fast Track)	Phase II/III (401) 333-3880
ocriplasmin (recombinant human microplasmin)	ThromboGenics <i>New York, NY</i>	adjunct to surgery in cases of pediatric vitrectomy	Phase II (212) 201-0920
<b>Ozurdex™</b> dexamethasone intravitreal implant	Allergan <i>Irvine, CA</i>	treatment of non-infectious ocular inflammation of the posterior segment in patients with intermediate, posterior, and panuveitis	Phase II/III (800) 433-8871
secukinumab	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	adjunctive treatment of chronic non-infectious uveitis requiring systemic immunosuppression	Phase III (888) 669-6682
trabomycin	Mobius Therapeutics <i>St. Louis, MO</i>	treatment of refractory glaucoma as an adjunct to ab externo glaucoma surgery	in clinical trials (314) 615-6930

## GASTROINTESTINAL DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
Aes-210	AesRx <i>Newton, MA</i>	topical treatment of children and adults with pouchitis	Phase II (617) 965-2521
ALTH12	Altheus Therapeutics <i>Oklahoma City, OK</i>	treatment of pediatric patients with ulcerative colitis (inclusive through age 16 years)	Phase I (405) 319-8180
AST-120	Ocera Therapeutics <i>San Diego, CA</i>	treatment of chronic pouchitis	Phase II (858) 436-3900

## GASTROINTESTINAL DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Gattex™</b> teduglutide	NPS Pharmaceuticals <i>Bedminster, NJ</i>	treatment of short bowel syndrome	Phase III (908) 450-5300
<b>Humira®</b> adalimumab	Abbott Laboratories <i>Abbott Park, IL</i>	treatment of pediatric Crohn's disease	Phase III (847) 937-6100
<b>Lialda®</b> mesalamine controlled-release	Shire Pharmaceuticals <i>Wayne, NJ</i>	treatment of active, mild to moderate ulcerative colitis in children 5 to 17 years	Phase I (800) 828-2088
mesalazine rectal high-concentration	Axcan Pharma US <i>Birmingham, AL</i>	treatment of pediatric ulcerative colitis	Phase III (800) 950-8085
metronidazole 10% ointment	S.L.A. Pharma <i>Liestal, Switzerland</i>	topical treatment of active perianal Crohn's disease	Phase II www.slapharma.com
phenylephrine	S.L.A. Pharma <i>Liestal, Switzerland</i>	treatment of ileal pouch anal anastomosis-related fecal incontinence	Phase I www.slapharma.com
<b>Remicade®</b> infliximab	Centocor Ortho Biotech <i>Horsham, PA</i>	treatment of pediatric (0 to 16 years of age) ulcerative colitis (see also autoimmune disorders)	Phase III (800) 457-6399

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
A-0001 (alpha-tocopherol quinone)	Endo Pharmaceuticals <i>Chadds Ford, PA</i>	treatment of inherited mitochondrial respiratory chain diseases	Phase II (610) 558-9800
AAV2-RP65	Applied Genetic Technologies <i>Alachua, FL</i>	treatment of type II Leber's congenital amaurosis	Phase I/II (386) 462-2204
ACE-031 (ActRIIB-IgG1)	Acceleron Pharma <i>Cambridge, MA</i>	treatment of Duchenne muscular dystrophy	Phase II (617) 649-9200
acid sphingomyelinase (rhASM)	Genzyme <i>Cambridge, MA</i>	treatment of acid sphingomyelinase deficiency (Niemann-Pick disease)	Phase I (617) 252-7500
AER002	Aerovance <i>Berkeley, CA</i>	treatment of cystic fibrosis	Phase II (510) 549-5500
<b>Aerosurf®</b> sinapultide (aerosolized KL4 surfactant)	Discovery Laboratories <i>Warrington, PA</i>	treatment of cystic fibrosis	Phase II (215) 488-9300
afamelanotide	Clinuvel Pharmaceuticals <i>Jersey City, NJ</i>	treatment of erythropoietic porphyrias	Phase II (201) 633-4773
A-galactosidase A	Alpha Gal <i>Owensboro, KY</i>	treatment of Fabry's disease	Phase III (270) 689-2570
AGTC-0106	Applied Genetic Technologies <i>Alachua, FL</i>	treatment of alpha1-antitrypsin deficiency	Phase II (386) 462-2204

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Amigal™</b> migalast	Amicus Therapeutics <i>Cranbury, NJ</i>	treatment of Fabry disease	Phase III (609) 662-2000
AMT-011	Amsterdam Molecular Therapeutics <i>Amsterdam, The Netherlands</i>	treatment of lipoprotein lipase deficiency	Phase I www.amtbv.com
ARD-3100	Aradigm <i>Hayward, CA</i>	management of cystic fibrosis	Phase II (510) 265-9000
ataluren	PTC Therapeutics <i>South Plainfield, NJ</i>	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 (908) 222-7000
		treatment of muscular dystrophy resulting from premature stop mutations in the dystrophin gene (Fast Track)	Phase II/III completed (908) 222-7000
<b>ATryn®</b> recombinant human antithrombin III	GTC Biotherapeutics <i>Framingham, MA</i>	treatment of antithrombin III dependent heparin resistance requiring anticoagulation	Phase III (508) 620-9700
AVI-4658	AVI BioPharma <i>Bothell, WA</i>	treatment of Duchenne muscular dystrophy	Phase I/II (425) 354-5038
BMN-110	BioMarin Pharmaceutical <i>Novato, CA</i>	use in the treatment of mucopolysaccharidosis type IV A (Morquio A syndrome)	Phase II (415) 506-6700
<b>Bronchitol®</b> mannitol inhalation	Pharmaxis <i>Exton, PA</i>	for use to facilitate clearance of mucus in patients with bronchiectasis and in patients with cystic fibrosis at risk for bronchiectasis (Fast Track)	Phase III (610) 363-5120
conestat alfa	Pharming <i>Leiden, The Netherlands</i> Santarus <i>San Diego, CA</i>	treatment of (acute attacks of) angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency (Fast Track) (see also transplantation)	application submitted www.pharming.com (858) 314-5700
		prophylactic treatment of angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency (Fast Track)	application submitted www.pharming.com (858) 314-5700
<b>Cystaran™</b> cysteamine ophthalmic	Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	treatment of corneal cystine crystal accumulation in cystinosis patients	application submitted (800) 447-0169
cystic fibrosis gene therapy	Copernicus Therapeutics <i>Cleveland, OH</i>	treatment of cystic fibrosis	Phase I/II (216) 231-0227
<b>Demser®</b> metyrosine	Aton Pharma <i>Lawrenceville, NJ</i>	treatment of velocardiofacial syndrome associated psychosis	Phase II (609) 671-9010

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
denufosal tetrasodium	Inspire Pharmaceuticals <i>Durham, NC</i>	treatment of cystic fibrosis (Fast Track)	Phase III (919) 941-9777
ecopipam	Psyadon Pharmaceuticals <i>Germantown, MD</i>	symptomatic treatment of self injurious behaviors in patients with Lesch-Nyhan disease	Phase I/II (301) 919-2020
eliglustat	Genzyme <i>Cambridge, MA</i>	treatment of Gaucher disease	Phase III (617) 252-7500
ENB0040	Enobia Pharma <i>Montreal, Canada</i>	treatment of hypophosphatasia (Fast Track)	Phase II (514) 596-2901
EXR-101	ExSAR <i>Monmouth Junction, NJ</i>	treatment of adult Tay-Sachs disease	Phase I (732) 438-6500
<b>Ferriprox™</b> deferiprone	ApoPharma <i>Toronto, Canada</i>	treatment of Friedreich's ataxia (see also blood disorders)	Phase II (416) 749-9300
<b>Fibrogammin® P</b> factor XIII concentrate (human)	CSL Behring <i>King of Prussia, PA</i>	treatment of congenital factor XIII deficiency	Phase III (610) 878-4000
<b>Fosamax®</b> alendronic acid	Merck <i>Whitehouse Station, NJ</i>	treatment of osteogenesis imperfecta in pediatric patients 4 years of age and older	application submitted (800) 672-6372
GMI-1070	GlycoMimetics <i>Gaithersburg, MD</i>	treatment of vaso-occlusive crisis in patients with sickle cell disease (Fast Track)	Phase II (240) 243-1201
<b>GNE-Lipoplex™</b>	Gradalis <i>Carrollton, TX</i>	treatment of hereditary inclusion body myopathy type 2	Phase I (214) 442-8100
GSK184072	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of MELAS syndrome	Phase I (888) 825-5249
GSK2402968 (PRO051)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i> Prosensa Therapeutics <i>Leiden, The Netherlands</i>	treatment of Duchenne muscular dystrophy	Phase I/II (888) 825-5249
HGT-1410 (sulfamidase)	Shire Human Genetic Therapies <i>Cambridge, MA</i>	for treatment of Sanfilippo syndrome	Phase I/II (800) 828-2088
HGT-2310 (idursulfase intrathecal)	Shire Human Genetic Therapies <i>Cambridge, MA</i>	for treatment of neurocognitive symptoms associated with Hunter syndrome	Phase I/II (800) 828-2088
HQK-1001	HemaQuest Pharmaceuticals <i>Seattle, WA</i>	treatment of beta thalassemia	Phase I/II (206) 682-1233
		----- treatment of sickle cell disease	Phase I/II (206) 682-1233
icatibant	Jerini <i>Berlin, Germany</i>	treatment of angioedema (Fast Track)	application submitted www.jerini.com

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
idebenone	Santhera Pharmaceuticals <i>Charlestown, MA</i>	treatment of Friedreich's ataxia (Fast Track)	Phase III (617) 886-5161
		treatment of Duchenne muscular dystrophy	Phase III (617) 886-5161
		treatment of Leber's hereditary optic neuropathy	Phase II completed (617) 886-5161
		treatment of mitochondrial myopathy, encephalopathy, lactic acidosis with stroke-like episodes syndrome (MELAS)	Phase II (617) 886-5161
<b>Kineret®</b> anakinra	Swedish Orphan Biovitrum <i>Stockholm, Sweden</i>	treatment of cryopyrin-associated periodic syndromes	Phase III www.sobi.com
KRN12 (recombinant human IgG1 mAb)	Kyowa Hakko Kirin Pharma <i>Princeton, NJ</i>	treatment of X-linked hypophosphatemia (formerly known as vitamin D-resistant rickets)	Phase I (609) 919-1100
lancovutide	Lantibio <i>Chapel Hill, NC</i> AOP Orphan Pharmaceuticals <i>Vienna, Austria</i>	treatment of cystic fibrosis	Phase II (919) 960-0217
L-glutamine	Emmaus Medical <i>Torrance, CA</i>	treatment of sickle cell disease	Phase II (877) 420-6493
lomitapide (AEGR-733)	Aegerion Pharmaceuticals <i>Bridgewater, NJ</i>	treatment of homozygous familial hypercholesterolemia	Phase III (908) 707-2100
lyophilised oxalobacter formigenes	OxThera <i>Alachua, FL</i>	treatment of primary hyperoxaluria	Phase II/III (386) 418-1428
mipomersen	Genzyme <i>Cambridge, MA</i>	treatment of homozygous familial hypercholesterolemia	Phase III (617) 252-7500
OBI-1 (recombinant porcine factor VIII)	Inspiration Biopharmaceuticals <i>Laguna Nigel, CA</i>	treatment and prevention of episodic bleeding in patients with inhibitor antibodies to human coagulation factor VIII	Phase III www.inspirationbio.com
PEG-PAL (phenylalanine ammonia lyase)	Biomarin Pharmaceutical <i>Novato, CA</i>	treatment of hyperphenylalaninemia	Phase II (415) 506-6700
QLT-09110	QLT <i>Vancouver, Canada</i> Retinagenix <i>Seattle, WA</i>	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	Phase I (607) 707-7000

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
recombinant factor VIII Fc fusion protein	Biogen Idec Hemophilia <i>Waltham, MA</i>	treatment of hemophilia A	Phase II/III (781) 547-6000
recombinant human alpha-1 antitrypsin (Raat)	Arriva Pharmaceuticals <i>Alameda, CA</i>	to delay progression of chronic obstructive pulmonary disease resulting from AAT deficiency-mediated emphysema and bronchiectasis	Phase II (510) 337-1250
recombinant human von Willebrand factor	Baxter Healthcare <i>Deerfield, IL</i>	treatment for von Willebrand disease	Phase I (800) 422-9837
<b>Replagal</b> <sup>®</sup> agalsidase alfa	Shire Human Genetic Therapies <i>Cambridge, MA</i>	long-term enzyme replacement therapy for the treatment of Fabry disease	Phase III completed (800) 828-2088
rFIXFc	Biogen Idec Hemophilia <i>Waltham, MA</i>	for the control and prevention of hemorrhagic episodes in patients with hemophilia B (congenital factor IX deficiency or Christmas disease)	Phase II/III (781) 547-6000
rFXIII (recombinant factor XIII)	Novo Nordisk <i>Princeton, NJ</i>	treatment of congenital FXIII deficiency	Phase III (609) 987-5800
		for the prophylaxis of bleeding associated with congenital FXIII deficiency	Phase III (609) 987-5800
<b>Spiriva</b> <sup>®</sup> <b>Respimat</b> <sup>®</sup> Inhaler tiotropium bromide	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	to improve pulmonary function in conjunction with standard therapy in the management of patients with cystic fibrosis	Phase III (203) 798-9988
STX207	Seaside Therapeutics <i>Cambridge, MA</i>	treatment of the behavioral abnormalities associated with fragile X syndrome	Phase I completed (617) 374-9009
STX209	Seaside Therapeutics <i>Cambridge, MA</i>	treatment of the behavioral abnormalities associated with fragile X syndrome	Phase II (617) 374-9009
tafamidis	FoldRx Pharmaceuticals <i>Cambridge, MA</i>	treatment of familial amyloid polyneuropathy	Phase III (617) 252-5500
taliglucerase alfa	Protalix Biotherapeutics <i>Carmiel, Israel</i>	treatment of Gaucher disease (Fast Track)	application submitted <a href="http://www.protalix.com">www.protalix.com</a>
TD101	TransDerm <i>Santa Cruz, CA</i>	treatment of pachyonychia congenita	Phase I (831) 420-1684
tgAAG76	Targeted Genetics <i>Seattle, WA</i>	treatment of Leber's congenital amaurosis	Phase I/II (206) 623-7612
thymosin beta 4 (RGN137)	RegeneRx Biopharmaceuticals <i>Bethesda, MD</i>	treatment of epidermolysis bullosa	Phase II (301) 280-1992

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
varespladib (A-001)	Anthera Pharmaceuticals <i>Hayward, CA</i>	prevention of acute chest syndrome in at-risk patients with sickle cell disease	Phase II (510) 856-5600
VX-770 (ivacaftor)	Vertex Pharmaceuticals <i>Cambridge, MA</i>	treatment of patients with cystic fibrosis (Fast Track)	Phase III (617) 444-6100
VX-809	Vertex Pharmaceuticals <i>Cambridge, MA</i>	treatment of cystic fibrosis	Phase II (617) 444-6100
<b>Xenbilox</b> <sup>®</sup> chenodeoxycholic acid	Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	treatment of cerebrotendinous xanthomatosis	application submitted (800) 447-0169
<b>Zavesca</b> <sup>®</sup> miglustat	Actelion Pharmaceuticals US <i>South San Francisco, CA</i>	treatment of the neurological manifestations of Niemann-Pick disease, type C	application submitted (650) 624-6900

## GROWTH DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
macimorelin	AEterna Zentaris <i>Warren, NJ</i>	diagnosis of growth hormone deficiency	Phase III (908) 626-5428
MOD-423 high analogue (long-acting somatropin)	PROLOR Biotech <i>Nes Ziona, Israel</i>	treatment of growth hormone deficiency	Phase II www.prolor-biotech.com
octreotide acetate subcutaneous implant	Endo Pharmaceuticals <i>Chadds Ford, PA</i>	treatment of acromegaly	Phase III (800) 462-3636
oral octreotide	Chiasma Pharma <i>Jerusalem, Israel</i>	treatment of acromegaly	Phase I completed www.chiasmapharma.com
pasireotide	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of acromegaly (see also other)	Phase III (888) 669-6682

## INFECTIOUS DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
<b>ABthrax</b> <sup>™</sup> raxibacumab	Human Genome Sciences <i>Rockville, MD</i>	treatment of anthrax (Fast Track)	application submitted (301) 309-8504
<b>Ampligen</b> <sup>®</sup> rintatolimod	Hemispherx Biopharma <i>Philadelphia, PA</i>	treatment of AIDS (see also autoimmune)	Phase II completed (215) 988-0080
anthrax immune globulin	Emergent Biosolutions <i>Rockville, MD</i>	treatment of inhalation anthrax disease (Fast Track)	Phase I/II (301) 795-1800
anthrax immune globulin	Cangene <i>Winnipeg, Canada</i>	treatment of toxemia associated with inhalational anthrax	Phase II completed (204) 275-4200

## INFECTIOUS DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Arikace™</b> inhaled liposomal amikacin	Transave <i>Monmouth Junction, NJ</i>	treatment of bronchiectasis in patients with <i>Pseudomonas aeruginosa</i> or other susceptible microbial pathogens	Phase II completed (732) 438-9434
		treatment of bronchopulmonary <i>Pseudomonas aeruginosa</i> infections in cystic fibrosis patients	Phase I/II completed (732) 438-9434
AVP 21D9	Emergent Product Development Gaithersburg <i>Gaithersburg, MD</i>	for post-exposure prophylaxis and treatment of inhalation anthrax (Fast Track)	Phase I (301) 590-0129
<b>Cayston™</b> aztreonam lysine for inhalation	Gilead Sciences <i>Foster City, CA</i>	inhalation therapy for control of gram-negative bacteria in the respiratory tract of patients with cystic fibrosis	Phase III (650) 574-3000
		improvement of respiratory symptoms in patients with bronchiectasis and gram-negative bacteria in the airways	Phase II (650) 574-3000
ciprofloxacin inhalation	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i>	management of pulmonary infection due to <i>Pseudomonas aeruginosa</i> in cystic fibrosis patients	Phase II (888) 842-2937
cytomegalovirus DNA vaccine	Vical <i>San Diego, CA</i>	prevention of clinically significant cytomegalovirus (CMV) viremia, CMV disease and associated complications in at-risk hematopoietic cell transplant and solid transplant populations	Phase II (858) 646-1100
ETI-204	Elusys Therapeutics <i>Pine Brook, NJ</i>	treatment of exposure to <i>anthracis</i> spores (Fast Track)	Phase I (973) 808-0222
GS9310/11 (fosfomycin/) tobramycin	Gilead Sciences <i>Foster City, CA</i>	treatment of pulmonary infections associated with cystic fibrosis	Phase II (800) 445-3235
hepatitis C virus immune globulin (human)	Biotest Pharmaceuticals <i>Boca Raton, FL</i>	prophylaxis of hepatitis C infection in liver transplant recipients	Phase II (561) 989-5800
itraconazole topical suspension	Fera Pharmaceuticals <i>New York, NY</i>	topical treatment of fungal otitis externa (otomycosis)	Phase II www.ferapharma.com
MDX-1303	Medarex <i>Princeton, NJ</i> PharmAthene <i>Annapolis, MD</i>	treatment of anthrax infection (Fast Track)	Phase I (609) 430-2880 (410) 571-8920
miltefosine	Paladin Labs <i>St-Laurent, Canada</i>	treatment of leishmaniasis	Phase II (888) 376-7830
moxidectin	Pfizer <i>New York, NY</i>	treatment of onchocerciasis volvulus in children and adults	Phase III (860) 732-5156

## INFECTIOUS DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
MP-376 (levofloxacin inhalation)	Mpex Pharmaceuticals San Diego, CA	treatment of pulmonary infections due to <i>Pseudomonas aeruginosa</i> and other bacteria in patients with cystic fibrosis	Phase II (858) 875-2840
nifurtimox	Bayer HealthCare Pharmaceuticals Wayne, NJ	treatment of Chagas disease (American typanosomiasis) caused by <i>T. cruzi</i>	Phase II (888) 842-2937
<b>Nuarte</b> <sup>TM</sup> artesunate intravenous	Sigma-Tau Pharmaceuticals Gaithersburg, MD US Army Medical Materiel Development Activity Fort Detrick, MD	immediate treatment of severe and complicated <i>P. falciparum</i> malaria	Phase III completed (800) 447-0169
PA-824	Global Alliance for TB Drug Development New York, NY Novartis Pharmaceuticals East Hanover, NJ	treatment of tuberculosis	Phase II (212) 227-7540 (888) 669-6682
pagibaximab	Biosynexus Gaithersburg, MD	prophylaxis of <i>Staphylococcus epidermidis</i> sepsis in low birth weight (1500 grams or less) infants	Phase II/III (301) 330-5800
<b>ShigamAbs</b> <sup>TM</sup> anti-shiga toxin mAb	Thallion Pharmaceuticals Montreal, Canada	treatment of shiga-toxin producing bacterial infections	Phase I (514) 940-3600
spherusol (coccidioidin-SD)	AllerMed Laboratories San Diego, CA	for the diagnosis of coccidioidomycosis	Phase II (800) 221-2748
SQ-109	Sequella Rockville, MD	treatment of tuberculosis (Fast Track)	Phase I (301) 762-7776
tecovirimat (ST-246)	SIGA Technologies New York, NY	treatment of orthopoxvirus infections	Phase II completed (212) 672-9100
		treatment of smallpox (Fast Track)	Phase II (212) 672-9100
		post-exposure prophylaxis against smallpox	Phase II (212) 672-9100
<b>Valtorim</b> <sup>®</sup>	PharmAthene Annapolis, MD	treatment of anthrax infection (Fast Track)	Phase I (410) 269-2600
varicella zoster immune globulin (human)	Cangene Winnipeg, Canada	passive immunization for the treatment of exposed, susceptible individuals who are at risk for complications from varicella	Phase III (204) 275-4178
<b>Veldona</b> <sup>®</sup> interferon-alpha	Amarillo Biosciences Amarillo, TX	treatment of papillomavirus warts in the oral cavity of HIV-positive patients (see also autoimmune disorders, blood disorders)	Phase II (806) 376-1741
VQD-001 (stibogluconate)	VioQuest Pharmaceuticals Basking Ridge, NJ	treatment of cutaneous leishmaniasis	Phase II (908) 766-4400

## INFECTIOUS DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
West Nile immune globulin	OMRIX Biopharmaceuticals <i>New York, NY</i>	treatment of the West Nile virus infection	Phase II (212) 887-6500
<b>Zadaxin</b> <sup>®</sup> thymalfasin	SciClone Pharmaceuticals <i>San Mateo, CA</i>	treatment of chronic active hepatitis B (see also cancer; cancer, skin)	Phase III (650) 358-3456

## NEUROLOGICAL DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
ACR-16 (pridopidine)	NeuroSearch AB <i>Goteborg, Sweden</i>	treatment of Huntington's disease	Phase II www.neurosearch.com
AEOL 10150	Aeolus Pharmaceuticals <i>Laguna Niguel, CA</i>	treatment of amyotrophic lateral sclerosis	Phase I (949) 481-9825
arimoclomol	CytRx <i>Los Angeles, CA</i>	treatment of amyotrophic lateral sclerosis (Fast Track)	Phase II/III (301) 826-5648
ATI355 (anti-Nogo-A mAb)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of acute spinal cord injury	Phase I (888) 669-6682
autologous incubated macrophage therapy	Proneuron Biotechnologies <i>New York, NY</i>	therapy to improve the motor and sensory neurological outcome in acute cases of spinal cord injury	Phase II (212) 758-9763
BA-210	BioAxone Therapeutics <i>Montreal, Quebec</i>	treatment of acute spinal cord injury	Phase II (514) 282-9990
BHR-100	BHR Pharma <i>Herndon, VA</i>	treatment of traumatic brain injury	Phase III (703) 964-3033
<b>Botox</b> <sup>®</sup> onabotulinum toxin A	Allergan <i>Irvine, CA</i>	treatment of dynamic muscle contracture in pediatric cerebral palsy patients	Phase III (714) 246-4500
CK-2017357	Cytokinetics <i>South San Francisco, CA</i>	treatment of amyotrophic lateral sclerosis	Phase II (650) 624-3000
clazosentan	Actelion Pharmaceuticals US <i>South San Francisco, CA</i>	treatment of cerebral vasospasm following subarachnoid hemorrhage	Phase III (650) 624-6900
clobazam	Lundbeck <i>Deerfield, IL</i>	treatment of Lennox-Gastaut syndrome	Phase III (866) 337-6996
davunetide intranasal	Allon Therapeutics <i>Vancouver, Canada</i>	treatment of progressive supranuclear palsy (Fast Track)	Phase II completed (604) 736-0634
dexpramipexole	Biogen Idec <i>Weston, MA</i> Knopp Neurosciences <i>Pittsburgh, PA</i>	treatment of amyotrophic lateral sclerosis (Fast Track)	Phase II (781) 464-2000 (412) 488-1776
dichlorphenamide (DCP)	Taro Pharmaceuticals USA <i>Hawthorne, NY</i> University of Rochester <i>Rochester, NY</i>	treatment of primary periodic paralyses	Phase III (800) 544-1449

## NEUROLOGICAL DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Dimebon™</b> atrepirdine	Medivation <i>San Francisco, CA</i>	treatment of Huntington's disease	Phase III (415) 543-3470
<b>Duodopa®</b> levodopa/carbidopa intraduodenal	Abbott Laboratories <i>Abbott Park, IL</i>	treatment of late-stage Parkinson's disease (Fast Track)	Phase III (847) 937-6100
<b>Dysport®</b> abobotulinum- toxinA	Tericia (Ipsen Group) <i>Brisbane, CA</i>	treatment of dynamic muscle contractures in pediatric cerebral palsy patients	Phase III (650) 624-4900
<b>Eladur™</b> transdur- bupivacaine	DURECT <i>Cupertino, CA</i> King Pharmaceuticals <i>Bristol, TN</i>	relief of persistent pain associated with postherpetic neuralgia	Phase II (408) 777-1417 (800) 776-3637
<b>Flebogamma®</b> human immune globulin	Grifols <i>Barcelona, Spain</i> Grifols USA <i>Los Angeles, CA</i>	treatment of post-polio syndrome	Phase III (888) 474-3657
gabapentin controlled-release	Abbott Laboratories <i>Abbott Park, IL</i>	management of postherpetic neuralgia	application submitted (847) 937-6100
<b>Gammagard Liquid™</b> immune globulin intravenous	Baxter Healthcare <i>Deerfield, IL</i>	treatment of multifocal motor neuropathy	Phase III (800) 422-9837
ganaxolone	Marinus Pharmaceuticals <i>Brandford, CT</i>	treatment of infantile spasms	Phase II (203) 315-0566
GRNOPC1	Geron <i>Menlo Park, CA</i> California Stem Cell <i>Irvine, CA</i>	treatment of spinal muscular atrophy	Phase I (650) 473-7700
GSK1223249 (anti-Nogo-A mAb)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	treatment of amyotrophic lateral sclerosis	Phase I (888) 825-5249
ISIS-SOD1Rx	Isis Pharmaceuticals <i>Carlsbad, CA</i>	treatment of amyotrophic lateral sclerosis	Phase I (760) 931-9200
melatonin controlled-release	Neurim Pharmaceuticals <i>Tel Aviv, Israel</i>	treatment of non-24-hour sleep-wake disorder in blind individuals without light perception	Phase III www.neurim.com
midazolam intranasal	Ikano Therapeutics <i>Saddle Brook, NJ</i>	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 (201) 843-3308
NH-001 (apomorphine subcutaneous)	NeuroHealing Pharmaceuticals <i>Newton, MA</i>	for the treatment of patients in a vegetative state or minimally conscious state for up to 12 months following a severe traumatic brain injury (traumatic or spontaneous) (Fast Track)	Phase II (617) 331-4111

## NEUROLOGICAL DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
NP-1 (amitriptyline/ ketamine)	EpiCept <i>Tarrytown, NY</i>	treatment of postherpetic neuralgia	Phase II completed (914) 606-3500
olesoxime	Trophos <i>Cedex, France</i>	treatment of amyotrophic lateral sclerosis	Phase I www.trophos.com
<b>Qutenza®</b> capsaicin 8%	NeurogesX <i>San Mateo, CA</i>	treatment of painful HIV-associated neuropathy (Fast Track)	Phase III (650) 358-3300
RP103 (mercaptamine delayed-release)	Raptor Pharmaceutical <i>Novato, CA</i>	treatment of neuronal ceroid lipofuscinoses (Batten disease) (see also other)	Phase I/II (877) 727-8680
		treatment of Huntington's disease	Phase I (877) 727-8680
RTL-1000	Artielle ImmunoTherapeutics <i>Tigard, OR</i>	treatment of multiple sclerosis patients who are both HLA-DR2 positive and autoreactive to myelin oligodendrocyte glycoprotein residues 35-55	Phase I (503) 626-1144
talampanel	Teva Neuroscience <i>Kansas City, MO</i>	treatment of amyotrophic lateral sclerosis	Phase II (816) 508-5000
tasimelteon (VEC-162)	Vanda Pharmaceuticals <i>Rockville, MD</i>	treatment of non-24 hour sleep/wake disorder in blind individuals without light perception	Phase III (240) 599-4500
		treatment of sleep-wake disorder in Smith-Magenis syndrome associated with diurnal melatonin secretion	Phase III (240) 599-4500
tideglusib	Noscira <i>Madrid, Spain</i>	treatment of progressive supranuclear palsy (Fast Track)	Phase II www.noscira.com
zucapsaicin	Winston Laboratories <i>Vernon Hills, IL</i>	treatment of postherpetic neuralgia	Phase III (847) 362-8200
		treatment of postherpetic neuralgia of the trigeminal nerve	Phase II (847) 937-6100

## RESPIRATORY DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
ARD-3150	Aradigm <i>Hayward, CA</i>	management of bronchiectasis	Phase II (510) 265-9000
calfactant	Pneuma Pharmaceuticals <i>Amherst, NY</i>	acute respiratory distress syndrome (ARDS)	Phase III
carlumab	Centocor Ortho Biotech <i>Horsham, PA</i>	treatment of idiopathic pulmonary fibrosis	Phase II (800) 457-6399

## RESPIRATORY DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
cicletanine	Gilead Sciences <i>Foster City, CA</i>	treatment for pulmonary arterial hypertension	Phase II (800) 445-3235
<b>Esbriet</b> <sup>®</sup> pirfenidone	InterMune <i>Brisbane, CA</i>	treatment of idiopathic pulmonary fibrosis (Fast Track)	application submitted (415) 466-2200
<b>Gleevec</b> <sup>®</sup> imatinib	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of pulmonary arterial hypertension	Phase III (888) 669-6682
<b>INOmax</b> <sup>®</sup> nitric oxide for inhalation	Ikaria <i>Clinton, NJ</i>	treatment of acute respiratory distress syndrome in adults	Phase III (908) 238-6600
		to reduce the risk of chronic lung disease in premature neonates	Phase III (908) 238-6600
IW001 (purified bovine type collagen)	ImmuneWorks <i>Indianapolis, IN</i> Lung Rx <i>Silver Spring, MD</i>	treatment of idiopathic pulmonary fibrosis	Phase I (317) 278-0205
<b>Letairis</b> <sup>™</sup> ambrisentan	Gilead Sciences <i>Foster City, CA</i>	treatment of idiopathic pulmonary fibrosis	Phase III (800) 445-3235
macitentan	Actelion Pharmaceuticals US <i>South San Francisco, CA</i>	treatment of pulmonary arterial hypertension	Phase III (650) 624-6900
		treatment of idiopathic pulmonary fibrosis	Phase II (650) 624-6900
nitrate inhalation	Aires Pharmaceuticals <i>San Diego, CA</i>	treatment of pulmonary arterial hypertension	Phase I (858) 366-9670
PRX-8066	Clinical Data <i>Newton, MA</i>	treatment for pulmonary arterial hypertension	Phase II (617) 527-9933
selexipag	Actelion Pharmaceuticals US <i>South San Francisco, CA</i>	treatment of pulmonary arterial hypertension	Phase III (650) 624-6900
<b>Surfaxin</b> <sup>®</sup> lucinactant	Discovery Laboratories <i>Warrington, PA</i>	treatment of respiratory distress syndrome in premature infants	Phase II (215) 488-9300
		treatment of acute respiratory distress syndrome in adults (Fast Track)	Phase II (215) 488-9300
		treatment of bronchopulmonary dysplasia in premature infants (Fast Track)	Phase II (215) 488-9300

# TRANSPLANTATION

Product Name	Sponsor	FDA Official Designation	Development Status
Amevive® alefacept	Astellas Pharma US <i>Deerfield, IL</i>	for use as prophylaxis of rejection in patients receiving allogeneic solid organ transplants	Phase II (800) 888-7704
ATG-Fresenius S® (antithymocyte globulin)	Fresenius Biotech North America <i>Waltham, MA</i>	prophylaxis of acute allograft rejection in adult recipients in solid organ transplantation	Phase III www.fresenius-biotech.com
		prevention of graft-versus-host disease (GVHD)	Phase III www.fresenius-biotech.com
ATIR™	Kiadis Pharma <i>Amsterdam, The Netherlands</i>	for reduction of transplant related mortality caused by graft-versus-host disease and/or infections following allogeneic bone marrow transplantation	Phase II/III www.kiadis.com
BB3	Angion Biomedica <i>Garden City, NY</i>	to improve renal function and prevent delayed graft function following renal transplantation (Fast Track)	Phase II www.angion.com
belatacept	Bristol-Myers Squibb <i>Princeton, NJ</i>	prophylaxis of organ rejection in renal allograft recipients	application submitted (212) 546-4000
carbon monoxide inhalation	Ikaria <i>Clinton, NJ</i>	for use in the reduction of the incidence and severity of delayed graft function in patients undergoing solid organ transplantation	Phase II (908) 238-6600
constat alfa	Pharming <i>Leiden, The Netherlands</i> Santarus <i>San Diego, CA</i>	prevention and/or treatment of delayed graft function after solid organ transplantation (see also genetic disorders)	Phase I www.pharming.com (858) 314-5700
cyclosporine inhalation solution	APT Pharmaceuticals <i>Burlingame, CA</i>	treatment of acute rejection in patients requiring allogeneic lung transplants	Phase III (650) 931-1666
		prophylaxis of organ rejection in patients receiving allogeneic lung transplants	Phase III (650) 931-1666
diannexin	Alavita Pharmaceuticals <i>Mountain View, CA</i>	prevention of ischemia-reperfusion injury in solid organ transplants	Phase II (650) 969-2405
emricasan	Conatus Pharmaceuticals <i>San Diego, CA</i>	prophylaxis of organ rejection in patients receiving allogeneic liver transplants	Phase II completed (858) 457-7221
FT-1050	Fate Therapeutics <i>San Diego, CA</i>	treatment of active cytomegalovirus infections	Phase I (858) 875-1800
NX-001	NephRx <i>Kalamazoo, MI</i>	prevention of delayed graft function in renal transplant recipients	Phase I (269) 372-8703
		treatment of delayed graft function in renal transplant recipients	Phase I (269) 372-8703

## TRANSPLANTATION

Product Name	Sponsor	FDA Official Designation	Development Status
<b>orBec</b> <sup>®</sup> beclomethasone dipropionate	Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i> Soligenix <i>Princeton, NJ</i>	for oral administration in the treatment of intestinal graft-versus-host disease (Fast Track)	Phase III (800) 447-0169 (609) 538-8200
		prevention of gastrointestinal graft-versus-host disease	Phase II (800) 447-0169 (609) 538-8200
		treatment of gastrointestinal symptoms with chronic graft-versus- host disease in patients undergoing allogeneic hematopoietic cell transplantation	Phase I (800) 447-0169 (609) 538-8200
QPI-1002	Quark Pharmaceuticals <i>Fremont, CA</i>	prophylaxis of delayed graft function in renal transplant patients	Phase I/II (510) 402-4020
recombinant P-selectin glycoprotein ligand (YSPSL)	Y's Therapeutics <i>San Bruno, CA</i>	prevention of delayed graft function in renal transplant patients (see also cardiovascular diseases)	Phase I/II completed (650) 777-7000
<b>Rhitol</b> <sup>™</sup>	Kiadis Pharma <i>Amsterdam, The Netherlands</i>	for immune reconstitution and prevention of graft-versus-host disease following allogeneic hema- topoietic stem cell transplantation	Phase I/II completed <a href="http://www.kiadis.com">www.kiadis.com</a>
STX-100	Stromedix <i>Cambridge, MA</i>	treatment of fibrosis-associated chronic allograft nephropathy in kidney transplant patients	Phase I completed (617) 674-8400
TOL101	Tolera Therapeutics <i>Kalamazoo, MI</i>	prophylaxis of acute rejection of solid organ transplantation	Phase I/II (877) 986-5372
TXA127 (angiotensin 1-7)	Tarix Pharmaceuticals <i>Brookline, MA</i> US Biotest <i>San Luis Obispo, CA</i>	to accelerate engraftment of hematopoietic cells (treatment of neutropenia, thrombocytopenia, lymphoma, and anemia) in hematopoietic stem cell transplants	Phase II (617) 827-6824
<b>Uvadex</b> <sup>®</sup> methoxsalen	Therakos <i>Raritan, NJ</i>	for use in conjunction with the UVAR photopheresis system to treat graft-versus-host disease	Phase II <a href="http://www.therakos.com">www.therakos.com</a>

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Acetadote</b> <sup>®</sup> N-Acetylcysteine injection	Cumberland Pharmaceuticals <i>Nashville, TN</i>	treatment of acute liver failure	application submitted (877) 484-2700
alicaforfen	Atlantic Pharmaceuticals <i>Cambridge, United Kingdom</i>	treatment of pouchitis (Fast Track)	Phase III <a href="http://www.atlantichc.com">www.atlantichc.com</a>

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
AM-111	Auris Medical <i>Chicago, IL</i>	treatment of acute sensorineural hearing loss	Phase II (312) 283-5633
BIO-300	Humanetics <i>Eden Prairie, MN</i>	prevention of acute radiation syndrome	Phase II (952) 937-7660
bioartificial liver system	Excorp Medical <i>St. Paul, MN</i>	treatment of patients with acute liver failure presenting with encephalopathy deteriorating beyond Parson's grade 2	Phase I/II (651) 484-0042
CBLB502	Cleveland BioLabs <i>Buffalo, NY</i>	for reducing the risk of death following total body irradiation	Phase I (716) 849-6810
<b>Corlux™</b> mifepristone	Corcept Therapeutics <i>Menlo Park, CA</i>	treatment of the clinical manifestations of endogenous Cushing's syndrome	Phase III (650) 327-3270
<b>Debrase®</b> bromelain topical	MediWound <i>Yavne, Israel</i>	debridement of acute, deep dermal burns in hospitalized patients	Phase II www.mediwound.com
defibrotide	Gentium <i>Como, Italy</i> Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	for the treatment of hepatic veno-occlusive disease (Fast Track)	Phase III (800) 447-0169
		for the prevention of hepatic veno-occlusive disease (Fast Track)	Phase III (800) 447-0169
droxidopa	Chelsea Therapeutics <i>Charlotte, NC</i>	treatment of neurogenic symptomatic orthostatic hypotension in patients with primary autonomic failure, dopamine-beta-hydroxylase deficiency, and nondiabetic autonomic neuropathy	Phase III (704) 341-1516
<b>ELAD®</b> immortalized human liver cells found in the extracorporeal liver assist device	Vital Therapies <i>San Diego, CA</i>	treatment of fulminant hepatic failure (acute liver failure)	Phase II/III (858) 673-6840
fresolimumab	Genzyme <i>Cambridge, MA</i>	treatment of primary focal segmental glomerulosclerosis	Phase I (617) 252-7500
<b>Gestiva™</b> hydroxy-progesterone caproate	Cytc Prenatal Products (Hologic) <i>Bedford, MA</i>	prevention of preterm birth in singleton pregnancies	application submitted (781) 999-7300
<b>Gymiso™</b> misoprostol	Gynuity Health Projects <i>New York, NY</i>	treatment of intrauterine fetal death not accompanied by complete expulsion of the products of conception in the second and third trimesters of pregnancy	Phase III (212) 448-1230

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
HPN-100	Hyperion Therapeutics <i>South San Francisco, CA</i>	maintenance treatment of patients with deficiencies in enzymes of the urea cycle (Fast Track)	Phase III (888) 897-4276
		for intermittent or chronic treatment of patients with cirrhosis and any grade hepatic encephalopathy	Phase II (888) 897-4276
		treatment of grade III and IV hepatic encephalopathy	Phase II (888) 897-4276
<b>Kiacta™</b> eprodysate	Bellus Health <i>Laval, Quebec</i>	treatment of secondary amyloidosis	Phase II/III completed (450) 680-4500
<b>Levulan® PDT</b> aminolevulinic acid	DUSA Pharmaceuticals <i>Wilmington, MA</i>	treatment of esophageal dysplasia	Phase II (978) 657-7500
<b>Lucassin™</b> terlipressin	Orphan Therapeutics <i>Lebanon, NJ</i>	treatment of hepatorenal syndrome (Fast Track)	application submitted (908) 849-4805
masitinib	AB Science <i>Paris, France</i>	treatment of mastocytosis	Phase III www.ab-science.com
metreleptin	Amylin Pharmaceuticals <i>San Diego, CA</i>	treatment of metabolic disorders secondary to lipodystrophy	Phase II (858) 552-2200
		treatment of leptin deficiency secondary to generalized lipodystrophy and partial familial lipodystrophy	Phase II (858) 552-220
midostaurin	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of mastocytosis (see also cancer, blood)	Phase II (888) 669-6682
NPSP-558	NPS Pharmaceuticals <i>Bedminster, NJ</i>	treatment of hypoparathyroidism	Phase III (908) 450-5300
obeticholic (INT-747)	Intercept Pharmaceuticals <i>New York, NY</i>	treatment of primary biliary cirrhosis	Phase II (646) 747-1000
OCR-002	Ocera Therapeutics <i>San Diego, CA</i>	treatment of hyperammonemia and resultant hepatic encephalopathy (HE) in patients with acute liver failure (Fast Track)	Phase I (858) 436-3900
oral viscous budesonide	Meritage Pharma <i>San Diego, CA</i>	treatment for pediatric eosinophilic esophagitis	Phase II (858) 436-1660
pasireotide	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of Cushing's disease (see also growth disorders)	Phase III (888) 669-6682
pegsitacase	3S Bio <i>Shenyang, China</i>	treatment of hyperuricemia in patients with gout refractory to conventional therapy or in whom conventional therapy is contraindicated (see also cancer-related conditions)	Phase I www.3sbio.com

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Plaquase™</b> collagenase (lyophilized) for injection	Auxilium Pharmaceuticals <i>Malvern, PA</i>	treatment of Peyronie's disease	Phase III (484) 321-5900
RG1068 (synthetic human secretin)	Repligen <i>Waltham, MA</i>	for use in conjunction with diagnostic procedures (including ERCP) for pancreatic disorders to increase pancreatic fluid secretion (Fast Track)	Phase III (800) 622-2259
<b>RiVax™</b> ricin vaccine	Soligenix <i>Princeton, NJ</i>	prevention of ricin intoxication	Phase I (609) 538-8200
RP103 (mercaptamine delayed-release)	Raptor Pharmaceutical <i>Novato, CA</i>	treatment of cystinosis (see also neurological disorders)	Phase III (877) 727-8680
S-DFO (deferoxamine- starch conjugate)	Biomedical Frontiers <i>Minneapolis, MN</i>	treatment of acute iron poisoning	Phase II (612) 378-0228
<b>Soliris®</b> eculizumab	Alexion Pharmaceuticals <i>Cheshire, CT</i>	treatment of atypical hemolytic uremic syndrome	Phase II (203) 272-2596
<b>Syprine®</b> trientine	Valeant Pharmaceuticals <i>Mississauga, Canada</i>	treatment of manganism	Phase II (905) 286-3000
tenecteplase	Genentech <i>South San Francisco, CA</i>	restore function to hemodialysis catheters	Phase III (800) 626-3553
<b>Voraxaze®</b> glucarpidase	Protherics (BTG) <i>Brentwood, TN</i>	treatment of patients at risk of methotrexate toxicity (Fast Track)	application submitted (615) 327-1027
<b>Xiaflex™</b> collagenase (lyophilized) for injection	Auxilium Pharmaceuticals <i>Malvern, PA</i>	treatment of Peyronie's disease	Phase III (484) 321-5900

The content of this report has been obtained through industry sources and the Adis "R&D Insights" database based on the latest information. Orphan designations from FDA's Office of Orphan Products Development through January 24, 2011. **Report current as of January 24, 2011.** The information may not be comprehensive. For more specific information about a particular product, contact the individual company directly or go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov). The entire series of *Medicines in Development* is available on PhRMA's web site.

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**acquired immune deficiency syndrome (AIDS)**—A manifestation of infection with the **human immunodeficiency virus (HIV)** characterized by the presence of one or more opportunistic diseases which occur primarily, or in some cases only, when an individual's immune system function is defective.

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

**adipose redistribution syndrome**—A group of rare metabolic disorders which can be either inherited or acquired. They are characterized by abnormalities in fatty (adipose) tissue associated with total or partial loss of body fat, abnormalities of carbohydrate and lipid metabolism, severe resistance to naturally occurring and synthetic insulin, and immune system dysfunction. These disorders are differentiated by degrees of severity, and by areas or systems of the body affected. They can also be associated with other disorders and various developmental abnormalities.

**adjunct**—An auxiliary treatment that is secondary to the main treatment.

**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.

**bacteremia**—Presence of bacteria in the blood stream. The bacteria (anaerobic) that cause **gram-negative bacteremia** release poison after they die. This results in fever, and causes fluid to leak from more permeable walls of blood capillaries into surrounding tissues. **Endotoxic shock**, a serious drop in blood pressure, can result.

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

**candidiasis**—A fungal (*Candida*) infection, usually of the moist cutaneous areas of the body, including the skin, mouth, esophagus and respiratory tract.

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

**cerebral palsy**—A general term for disorders of movement and posture resulting from damage to the brain in pregnancy, during birth, in the newborn period, or in early childhood.

**cervical dystonia**—Disorder or lack of muscle tone in the muscles of the neck.

**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.

**cystinosis**—Cystine, an amino acid, accumulated in internal organs, resulting in damage and cystinuria (presence of cystine in urine).

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

**dysplasia, bronchopulmonary**—Abnormal growth of the cells of the lungs and air passages associated with exposure of immature lungs to high levels of oxygen.

**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.

**esophageal varices, bleeding**—Dilated, weakened veins in walls of the lower part of the esophagus, which can rupture and cause acute bleeding.

**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.

**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.

**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.

**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 vs. 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. **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.

**histoplasmosis**—A disease caused by a fungal infection that can affect all organs of the body.

**HIV**—Human immunodeficiency virus, the virus that causes AIDS.

**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.

**immune 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.

**Lennox-Gastaut syndrome**—Characterized by seizures and mental retardation in infants and young children.

**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 nonHodgkin'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.

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

**methotrexate**—An anticancer drug.

**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.

**muscular dystrophy**—Inherited muscular disorder of unknown cause in which muscle fibers slowly degenerate. **Duchenne MD** is the most common type.

**myasthenia gravis**—A chronic autoimmune neuromuscular disease characterized by varying degrees of weakness of the skeletal (voluntary) muscles of the body. The hallmark of myasthenia gravis is muscle weakness that increases during periods of activity and improves after periods of rest. Certain muscles such as those that control eye and eyelid movement, facial expression, chewing, talking, and swallowing are often, but not always, involved in the disorder. With current therapies, most cases of myasthenia gravis are not as “grave” as the name implies. For the majority of individuals with the disease, life expectancy is not lessened by the disorder.

**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.

**papillomavirus/papillomatosis**—The papillomavirus is the viral agent of warts, believed to be contagious and mostly harmless, affecting only the skin’s topmost layer.

**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 I**—Safety testing and pharmacological profiling of new drugs in small numbers of humans.

**Phase II**—Effectiveness testing and identification of side effects of new drugs in humans.

**Phase III**—Extensive clinical trials in humans to verify effectiveness and monitor adverse reactions of new drugs.

**Phase IV**—Additional post-marketing testing of drugs sometimes required by the FDA.

**photodynamic therapy (PDT)**—A treatment that uses a drug, called a photosensitizer or photosensitizing agent, and a particular type of light. When photosensitizers are exposed to a specific wavelength of light, they produce a form of oxygen that kills nearby cancer cells.

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

**precocious puberty**—Onset of early puberty. It can be a normal variant or familial trait, or be caused by serious diseases, such as hypothalamic lesions, encephalitis and some

tumors. If not detected early, children may be dwarfed.

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

**pulmonary**—Pertaining to 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.

**systemic**—Affecting the whole body.

**xeroderma pigmentosa**—A rare, inherited skin disease caused by extreme sensitivity to sunlight, which causes the skin to become dry, wrinkled, freckled and prematurely aged by about the age of five. Various types of benign and malignant skin tumors also develop.

# FREQUENTLY ASKED QUESTIONS ABOUT RARE DISEASES

## **How can you find out about clinical research on rare diseases?**

There's a web site that was just set up a few years ago by the federal government. It's called [www.clinicaltrials.gov](http://www.clinicaltrials.gov). It's important to remember ".gov" because there are some commercial sites that have similar names. Every research project receiving any money from the U.S. government must be listed on this site. It's a requirement. You can type in the disease name and find all sorts of information about the studies, where they're being conducted, what is needed to be eligible, and who to contact to learn more about participating. If you don't have a computer, ask your local librarian to help you search on that web site.

## **How can people help a family member with a rare disease get the best possible treatment?**

The Internet is a wonderful tool for finding a doctor and hospital with experience in treating your family member's disease. Ask your doctor or the patient support group for your disease for help. Doctors understand the importance of involving someone who has experience with the disease. You can also search a web site, [www.pubmed.gov](http://www.pubmed.gov), to see what has been published lately about your disease and who has written articles about it in medical journals.

It's usually best, with rare diseases, to be treated at a teaching hospital affiliated with a university, rather than a smaller community hospital. The teaching hospitals would be more likely to have experience with rare diseases.

Another very important thing to do is to find out if there is a patient organization for your disease. These organizations can be tremendously helpful in many everyday ways, and they are also a wonderful way to network with other people who have the same disease. Patients can learn a lot from each other, and they can alert each other to other sources of help. Many of the new treatments for rare diseases that are being developed today were made possible because of the involvement... through fund-raising and other ways... of patient organizations. When you read in the newspaper that a gene has been identified for a certain genetic disorder, or that a new drug has been approved, you can bet that a patient organization played a significant role in that research.

## **Where can you go for information about rare diseases?**

The Internet is a wonderful tool, and even if you don't have a computer you can get access to it through your local

library, through a senior center, or through other social service offices.

If you go online yourself at home, just remember that there is no guarantee that everything you read online is accurate. It's important to use web sites you can trust, and that includes ones from the federal government, from universities, from teaching hospitals, or from non-profit organizations. The National Institutes of Health ([www.nih.gov](http://www.nih.gov)) has some excellent web sites with information about certain rare diseases. One NIH web site, [www.MedlinePlus.com](http://www.MedlinePlus.com), has good, understandable information. The Centers for Disease Control and Prevention ([www.cdc.gov](http://www.cdc.gov)) also has some good information about some rare diseases.

## **Why does it take so long to get a diagnosis? How can we educate our doctors?**

It's difficult getting a diagnosis because there are 6,000 rare diseases, and the first doctor you go to may never have seen the particular rare disease you have. Genetic Alliance, NORD, and other patient organizations are working to raise awareness among physicians, and efforts such as the Human Genome Project have certainly raised awareness, but diagnosis is still a problem. A government study done in 1989 showed that it takes longer for people with rare diseases to get a diagnosis, compared to other diseases. That study was replicated on a smaller scale recently, and the numbers were about the same.

## **Is there anything you can do if you or your loved one, do not have a diagnosis?**

This is a very difficult situation. It's important to see the right kind of doctor for your disease. Also, it's a good idea to keep a notebook, and write things down that seem significant so that you won't forget to mention them when you see the doctor. You know your loved one better than the doctor does, so if something seems unusual or significant to you, chances are it is.

Source: National Organization for Rare Disorders, Inc.

## **Additional Resources**

Genetic Alliance, [www.geneticalliance.org](http://www.geneticalliance.org)  
National Institutes of Health, Office of Rare Diseases Research, [www.rarediseases.info.nih.gov](http://www.rarediseases.info.nih.gov)  
National Organization for Rare Diseases (NORD), [www.rarediseases.org](http://www.rarediseases.org)

## THE DRUG DISCOVERY, DEVELOPMENT AND APPROVAL PROCESS

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

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

## THE DRUG DEVELOPMENT AND APPROVAL PROCESS

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

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

On average, it costs a company \$1.3 billion to get one new medicine from the laboratory to U.S. patients, according to a 2007 study by the Tufts Center for the Study of Drug Development.

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

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

**Investigational New Drug Application (IND).** After completing preclinical testing, a company files an IND with the U.S. Food and Drug Administration (FDA) to begin to test the drug in people. The IND shows results of previous experiments; how, where and by whom the new studies will be conducted; the chemical structure of the compound; how it is thought to work in the body; any toxic effects found in the animal studies; and how the compound is manufactured. All clinical trials must be reviewed and approved by the Institutional Review Board (IRB) where the trials will be conducted. Progress reports on clinical trials must be submitted at least annually to FDA and the IRB.

**Clinical Trials, Phase I.** These tests usually involve

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

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

**Clinical Trials, Phase III.** This phase usually involves 1,000 to 5,000 patients in clinics and hospitals. Physicians monitor patients closely to confirm efficacy and identify adverse events.

**New Drug Application (NDA)/Biologic License Application (BLA).** Following the completion of all three phases of clinical trials, a company analyzes all of the data and files an NDA or BLA with FDA if the data successfully demonstrate both safety and effectiveness. The applications contain all of the scientific information that the company has gathered. Applications typically run 100,000 pages or more. The average review time for the 25 new therapeutics approved by the FDA in 2009 was 13.3 months.

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

Discovering and developing safe and effective new medicines is a long, difficult, and expensive process. Pharmaceutical companies invested an estimated \$65.3 billion in research and development in 2009.

# TRACKING THE BIOPHARMACEUTICAL RESEARCH PIPELINE

Today, more than 2,900 new medicines are in development in the United States. Many of these potential new medicines will fail in clinical trials, but some may represent tomorrow's new treatments. Bringing each new medicine to patients will require, on average, 10 to 15 years of testing and review.

PhRMA publishes several reports that track the biopharmaceutical research pipeline for many diseases, including the leading causes of death among Americans—heart disease, cancer, and stroke. The reports include medicines currently in clinical trials or at the U.S. Food and Drug Administration (FDA) for review. Below is a summary of our most popular reports.

- **Cancer**—There are few things that cause patients more fear and uncertainty as a cancer diagnosis. Yet today—because of a steady stream of new and improved medicines and treatments—cancer can increasingly be managed and even beaten. *The 2009 report found 861 medicines in the pipeline.*
- **Heart Disease and Stroke**—Keeping up the momentum of drug discovery that has helped cut deaths from heart disease and stroke in half in the past three decades, biopharmaceutical companies are working on new medicines for these diseases. *The 2011 report found 299 medicines in the pipeline.*
- **Diabetes**—Approximately 4,110 people are diagnosed with diabetes every day. To help fight this disease, pharmaceutical and biotechnology researchers are working on new medicines to treat it and related conditions. *The 2010 report found 235 medicines in the pipeline.*
- **Mental Illnesses**—Pharmaceutical and biotechnology researchers are testing many new medicines to help the more than 450 million people worldwide who suffer from some form of mental illness. *The 2010 report found 313 medicines in the pipeline.*
- **Biotechnology**—Millions of people have already benefited from medicines and vaccines developed through biotechnology, and a new report offers hope that many more will benefit in the future. *The 2008 report found 633 medicines in the pipeline.*
- **Children**—Biopharmaceutical researchers are testing medicines to meet the special needs of children. These medicines offer hope that the significant improvements achieved in children's health over the past few decades will continue and even accelerate. *The 2010 report found 234 medicines in clinical trials.*
- **Infectious Diseases**—Throughout history, infectious diseases have taken a devastating toll on the lives and well-being of people around the world. Today, vaccines and antibiotics have proven to be effective treatments in many cases, but infectious diseases still pose a very serious threat to patients. Infectious diseases may never be eradicated. But, new knowledge, new technologies, and a huge commitment of resources by America's biopharmaceutical research companies and the government can help meet the continuing—and ever-changing—threat from infectious diseases. *The 2010 report found 395 medicines in clinical trials.*



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