

# Rare Diseases

PRESENTED BY AMERICA'S BIOPHARMACEUTICAL COMPANIES, GENETIC ALLIANCE, AND NATIONAL ORGANIZATION FOR RARE DISEASES

## More Than 300 Medicines Are in Development to Treat or Prevent Rare Diseases

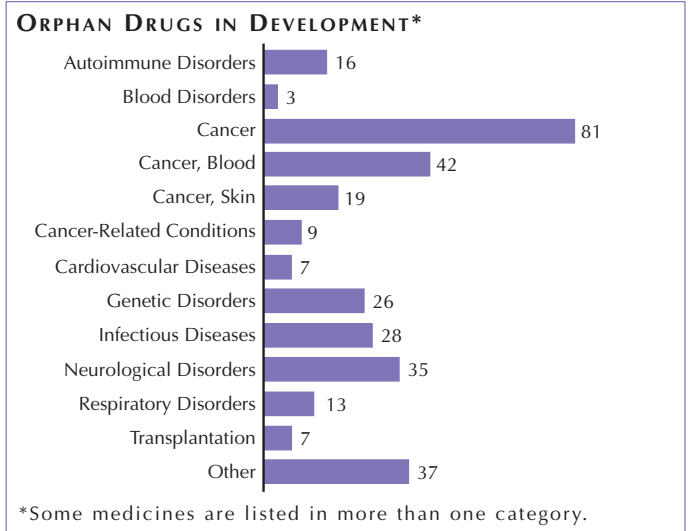
Every patient faces unique health challenges. And, for millions of Americans who have been diagnosed with a rare disease—defined as a condition affecting fewer than 200,000 patients in the United States—treatment options can be limited. But over the last few years, great progress has been made with the approval of several new medicines for rare diseases such as Pompe disease, myelodysplastic syndromes, enzyme deficiencies, and rare cancers. America's biopharmaceutical research companies are continuing that progress with 303 medicines currently in human clinical trials or awaiting approval by the U.S. Food and Drug Administration (FDA) for many rare diseases. This compares to 133 medicines in development in 1989 and 189 in 1992.

Since 1995, more than 160 medicines were approved to treat rare diseases, compared to 108 in the decade before and fewer than 10 in the 1970s. Advances in science, such as a better understanding of molecular and genetic causes of disease, have given researchers new tools to explore rare diseases, which are often more complex than more common diseases. In addition, the Orphan Drug Act of 1983 provided tax relief and some marketing exclusivity for companies that develop an orphan drug. That legislation is credited with the explosion in drug approvals for rare diseases after 1983. Under the Orphan Drug Act, 1,679 medicines have been designated orphan drugs (not all are approved) as of January 10, 2007.

The National Institutes of Health estimates there are 6,000 rare diseases affecting 25 million Americans. A major area of research in rare diseases is cancer. Rare cancers, such as solid tumors of the liver and thyroid, cancer of the blood, and melanoma account for more than one-third of all rare disease research, with 139 medicines in development. Other important areas of research include: neurologic disorders, such as multiple sclerosis and muscular dystrophy, with 35 medicines in development; infectious diseases, such as anthrax and West Nile virus, with 28 medicines in development; and genetic disorders, such as cystic fibrosis, with 26 medicines in development.

Some examples of medicines in development for rare diseases include:

- A monoclonal antibody for chronic sarcoidosis, an immune system disorder.



- A medicine for Lennox-Gastaut syndrome, a severe form of epilepsy.
- Gene therapy for cystic fibrosis.
- A medicine for epidermolysis bullosa, a group of inherited disorders where skin blisters develop in response to minor trauma.
- A medicine for Friedreich's ataxia, a genetic disorder.

Biopharmaceutical research is entering an exciting new era with our growing understanding of the genome and powerful scientific research tools. Experts predict the number of orphan drugs will rise in the coming years as more new medicines are developed that target specific genetic disorders. The 303 medicines in this report represent the enormous commitment by America's research companies to find treatments, and perhaps cures, for many of the 6,000 known rare diseases.

Without question, the Orphan Drug Act of 1983 has worked exceedingly well in achieving the important purpose for which it was enacted—to provide incentives that would encourage orphan drug research and help the 25 million Americans afflicted with a rare disease.

Sincerely,

Billy Tauzin  
President and CEO  
PhRMA

# Orphan Drugs in Development

## AUTOIMMUNE DISORDERS

Product Name	Sponsor	FDA Official Designation*	Development Status**
<b>Ampligen®</b>	Hemispherx Biopharma <i>Philadelphia, PA</i>	treatment of chronic fatigue syndrome (see also infectious diseases)	Phase III (800) 332-6854
<b>Cellcept®</b> mycophenolate mofetil	Aspreva Pharmaceuticals <i>Basking Ridge, NJ</i> Roche <i>Nutley, NJ</i>	treatment of pemphigus vulgaris	Phase III (908) 212-1020 (973) 235-2000
		treatment of myasthenia gravis	Phase III (908) 212-1020 (973) 235-2000
clindamycin hydrochloride	Autoimmunity Research Foundation <i>Thousand Oaks, CA</i>	treatment of sarcoidosis	Phase II (805) 492-3693
desmoglein 3 synthetic peptide (PI-0824)	Peptimmune <i>Cambridge, MA</i>	treatment of pemphigus vulgaris	Phase I (617) 715-8000
humanized anti-human CD16 monoclonal antibody	Genzyme <i>Cambridge, MA</i>	treatment of adult idiopathic thrombocytopenic purpura	Phase I (617) 252-7500
<b>Humira®</b> adalimumab	Abbott Laboratories <i>Abbott Park, IL</i>	treatment of juvenile rheumatoid arthritis	Phase III (847) 937-6100
interferon gamma-1b	InterMune <i>Brisbane, CA</i>	treatment of idiopathic pulmonary fibrosis	Phase III (415) 466-2200
minocycline hydrochloride	Autoimmunity Research Foundation <i>Thousand Oaks, CA</i>	treatment of sarcoidosis	Phase II (805) 492-3693
monarsen	Ester Neurosciences (Medica Venture Partners) <i>Herzlia Pituach, Israel</i>	treatment of myasthenia gravis	Phase I/II <a href="http://www.esterneuro.com">www.esterneuro.com</a>
<b>Oralgam™</b> human gammaglobulin	PediaMed Pharmaceuticals <i>Florence, KY</i> Protein Therapeutics <i>Tucson, AZ</i>	treatment for juvenile rheumatoid arthritis (see also other)	Phase II (520) 844-1187
<b>Prestara™</b> dehydroepian-drosterone	Genelabs Technologies <i>Redwood City, CA</i>	treatment of systemic lupus erythematosus (SLE) and the reduction in the use of steroids in steroid-dependent SLE patients	Phase III (650) 369-9500

\* 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 call the telephone number listed.

## AUTOIMMUNE DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
recombinant human alpha-fetoprotein (rhAFP) (MM093)	Merrimack Pharmaceuticals <i>Cambridge, MA</i>	treatment of myasthenia gravis	Phase I (617) 441-1000
<b>Remicade®</b> infliximab	Centocor <i>Horsham, PA</i>	treatment of juvenile rheumatoid arthritis	Phase III (610) 651-6000
		treatment of chronic sarcoidosis	Phase II (610) 651-6000
riloncept (interleukin-1 trap)	Regeneron Pharmaceuticals <i>Tarrytown, NY</i>	treatment of Still's disease including juvenile rheumatoid arthritis and adult-onset Still's disease (see also genetic)	Phase II (914) 345-7400
<b>Riquent®</b> abetimus	La Jolla Pharmaceutical <i>San Diego, CA</i>	treatment of lupus nephritis	application submitted (858) 452-6600
<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) (see also cancer)	Phase III (650) 225-1000
		treatment of immune thrombocytopenic purpura	Phase II (650) 225-1000

## BLOOD DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
deferitrin	Genzyme <i>Cambridge, MA</i>	treatment of iron overload	Phase II (617) 252-7500
eculizumab	Alexion Pharmaceuticals <i>Cheshire, CT</i>	treatment of paroxysmal nocturnal hemoglobinuria	application submitted (203) 272-2596
<b>Ferriprox™</b> deferiprone	Apotex Research <i>Toronto, Canada</i> Cangene <i>Winnipeg, Canada</i>	treatment of iron overload in patients with hematologic disorders requiring chronic transfusion therapy	Phase III (416) 749-9300

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
A10/AS2-1 antineoplaston	Burzynski Research Institute <i>Houston, TX</i>	treatment for patients with brain stem glioma	Phase II

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
ACA125	Menarini Ricerche <i>Rome, Italy</i> CellControl Biomedical <i>Germany</i>	treatment of epithelial ovarian cancer	Phase I
<b>Advexin®</b> INGN 201	Introgen Therapeutics <i>Houston, TX</i>	treatment of head and neck cancer	Phase III (713) 797-9960
AMG 706	Amgen <i>Thousand Oaks, CA</i>	treatment of gastrointestinal stromal tumors	Phase II (805) 447-1000
atiprimod	Callisto Pharmaceuticals <i>New York, NY</i>	treatment of carcinoid tumors (see also cancer, blood)	Phase II (212) 297-0010
<b>Avastin®</b> bevacizumab	Genentech <i>South San Francisco, CA</i>	therapeutic treatment of patients with ovarian cancer	Phase III (650) 225-1000
		treatment of pancreatic cancer	Phase III (650) 225-1000
		treatment of renal cell carcinoma	Phase III (650) 225-1000
		treatment of malignant glioma	Phase II (650) 225-1000
<b>Azedra™</b> iobenguane I-131	Molecular Insight Pharmaceuticals <i>Cambridge, MA</i>	treatment of neuroendocrine tumors	Phase I (617) 492-5554
CA4P (combretastatin)	OXiGENE <i>Watertown, MA</i>	treatment of ovarian cancer	Phase II (781) 547-5900
		treatment of anaplastic thyroid cancer, medullary thyroid cancer, and stage IV papillary or follicular thyroid cancer	Phase II (781) 547-5900
carmustine (DTI-015)	Direct Therapeutics <i>Redwood City, CA</i>	treatment of intracranial malignancies	Phase II www. directtherapeutics.com
<b>Cerepro™</b>	Ark Therapeutics <i>London, England</i>	use with gancyclovir in the treatment of malignant glioma	in clinical trials www.arktherapeutics.com
cilengitide	EMD Pharmaceuticals <i>Durham, NC</i>	treatment of malignant glioma	Phase II (919) 401-7100
conjugate of human transferrin and a mutant diphtheria toxin (CRM 107)	Xenova <i>Berkshire, United Kingdom</i>	treatment of malignant tumors of the central nervous system	in clinical trials

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Cotara™</b> iodine I-131 radiolabeled chimeric MAb tumor necrosis treatment (TNT-1B)	Peregrine Pharmaceuticals <i>Tustin, CA</i>	treatment of glioblastoma multiforme and anaplastic astrocytoma	Phase II/III (714) 508-6000
<b>DCVax-Brain®</b>	Northwest Biotherapeutics <i>Bothell, WA</i>	treatment of primary brain malignant cancer	Phase II (425) 608-3000
diethylInorspermine (DENSPM)	Genzyme <i>Cambridge, MA</i>	treatment for hepatocellular carcinoma	Phase I (617) 252-7500
DNA plasmid vector-expressing human IL-12 gene	Expression Genetics <i>Huntsville, AL</i>	treatment of ovarian cancer	Phase I (256) 512-0077
<b>Doxorubicin™</b> <b>Transdrug</b> doxorubicin PIHCA nanoparticles	BioAlliance Pharma <i>Paris, France</i>	treatment of hepatocellular carcinoma	Phase I
<b>Efaproxyn™</b> efaproxiral	Allos Therapeutics <i>Westminster, CO</i>	adjunct to whole brain radiation therapy for the treatment of brain metastases in patients with breast cancer	application submitted (303) 426-6262
enzastaurin	Eli Lilly <i>Indianapolis, IN</i>	treatment of glioblastoma multiforme	Phase III (312) 276-2000
<b>Evista®</b> raloxifene	Eli Lilly <i>Indianapolis, IN</i>	reduction of the risk of breast cancer in postmenopausal women	Phase III (317) 276-2000
genetically engineered herpes simplex virus (G207)	MediGene <i>San Diego, CA</i>	treatment of malignant glioma	Phase I (858) 586-2240
gimatecan	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	treatment of malignant glioma	Phase I (888) NOW-NOVA
<b>GliAtak™</b>	Advantagene <i>Waban, MA</i>	treatment of malignant brain tumors	Phase I (617) 916-5445
glufosfamide	Threshold Pharmaceuticals <i>Redwood City, CA</i>	treatment of pancreatic cancer	Phase III (650) 474-8200
GTI-2040	Lorus Therapeutics <i>Toronto, Ontario</i>	treatment of renal cell carcinoma (see also cancer, blood)	Phase II (416) 798-1200
GV-1001 (human telomerase reverse transcriptase peptide vaccine)	Pharmexa <i>Horsholm, Denmark</i>	treatment of pancreatic cancer	Phase III www.pharmexa.com
<b>Herceptin®</b> trastuzumab	Genentech <i>South San Francisco, CA</i>	treatment of patients with pancreatic cancer that overexpress p185HER2	Phase II (650) 225-1000
human leukocyte-derived cytokine mixture	IRX Therapeutics <i>New York, NY</i>	for neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck	Phase II (212) 582-1199

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
I(131)-TM-601 (chlorotoxin)	TransMolecular <i>Birmingham, AL</i>	treatment of malignant glioma	Phase II (617) 995-3050
iboctadekin	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	treatment of renal cell carcinoma	Phase II (888) 825-5249
IL13-PE38QQR	NeoPharm <i>Waukegan, IL</i>	treatment of malignant glioma	Phase I/II (847) 887-0800
imexon	AmpliMed <i>Tucson, AZ</i>	treatment of pancreatic adenocarcinoma (see also cancer, blood; cancer, skin)	Phase I (520) 529-1000
IMMU-107	Immunomedics <i>Morris Plains, NJ</i>	treatment of pancreatic cancer	Phase I (973) 605-8200
<b>IntraDose</b> <sup>®</sup> cisplatin/ epinephrine	Novartis Pharmaceutical <i>East Hanover, NJ</i>	treatment of squamous cell carcinoma of the head and neck (see also cancer, skin)	application submitted (888) NOW-NOVA
<b>Intron A</b> <sup>®</sup> interferon alfa-2b (recombinant)	Schering-Plough <i>Kenilworth, NJ</i>	treatment of carcinoma in situ of the urinary bladder	Phase III (908) 298-4000
irofulven	MGI Pharma <i>Bloomington, MN</i>	treatment of ovarian cancer	Phase II (952) 346-4700
<b>Junovan</b> <sup>™</sup> mifamuritide	IDM Pharma <i>Irvine, CA</i>	treatment of child and adolescent osteosarcoma	Phase III (949) 470-4757
MORAb-003	Morphotek <i>Exton, PA</i>	treatment of ovarian cancer	Phase II (610) 423-6100
MORAb-009	Morphotek <i>Exton, PA</i>	treatment of pancreatic cancer	Phase I (610) 423-6100
MTC-DOX for injection	FeRx <i>San Diego, CA</i>	treatment of hepatocellular carcinoma	Phase I
neuradiab	Bradmer Pharmaceuticals <i>Louisville, KY</i> Duke University Medical Center <i>Durham, NC</i>	treatment of primary malignant brain tumors	Phase II (502) 657-6038
<b>NeuTrexin</b> <sup>®</sup> trimetrexate glucuronate	MedImmune Oncology <i>Gaithersburg, MD</i>	treatment of metastatic colorectal adenocarcinoma	Phase III (301) 398-0000
		treatment of pancreatic adenocarcinoma	Phase II (301) 398-0000
		treatment of patients with advanced non-small-cell carcinoma of the lung	Phase II (301) 398-0000
<b>Nexavar</b> <sup>®</sup> sorafenib	Bayer Pharmaceuticals <i>West Haven, CT</i> Onyx Pharmaceuticals <i>Emeryville, CA</i>	treatment of hepatocellular carcinoma (see also cancer, skin)	Phase III (203) 812-2000 (510) 597-6500

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
nimotuzumab	YM Biosciences <i>Mississauga, Ontario</i>	treatment of glioma	Phase III (905) 629-9761
nucleic acid aptamer binding to tumor cell nucleolin	Antisoma Research <i>London, England</i>	treatment of renal cell carcinoma	Phase I
<b>Oncophage</b> <sup>®</sup> vitespen	Antigenics <i>New York, NY</i>	treatment of renal cell carcinoma (see also cancer, skin)	Phase II (212) 994-8200
<b>Orathecin</b> <sup>™</sup> rubitecan	SuperGen <i>Dublin, CA</i>	treatment of pancreatic cancer	Phase III (925) 560-0100
<b>OvaRex</b> <sup>®</sup> oregovomab	Unither Pharmaceuticals <i>Wellesley Hills, MA</i>	treatment of epithelial ovarian cancer	Phase III
paclitaxel (TOCOSOL)	Sonus Pharmaceuticals <i>Bothell, WA</i>	treatment of urothelial cancer	Phase II (425) 787-9500
<b>Panvec-VF</b> <sup>™</sup>	Therion Biologics <i>Cambridge, MA</i>	treatment of adenocarcinoma of the pancreas	Phase III (617) 252-7500
<b>Panzem</b> <sup>®</sup> NCD (2ME2)	EntreMed <i>Rockville, MD</i>	treatment of multiforme glioblastoma (see also cancer, blood)	Phase II (240) 864-2600
		treatment of ovarian cancer	Phase I (240) 864-2600
patupilone	Novartis Pharmaceutical <i>East Hanover, NJ</i>	treatment of ovarian cancer	Phase III (888) NOW-NOVA
pegylated arginine deiminase	Phoenix Pharmacologics <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 Scandipharm <i>Birmingham, AL</i>	treatment of cholangiocarcinoma	Phase II (205) 991-8085
picoplatin	Poniard Pharmaceuticals <i>South San Francisco, CA</i>	treatment of small-cell-lung cancer	Phase II (650) 583-3774
<b>Proxinium</b> <sup>™</sup>	Viventia Biotech <i>Toronto, Ontario</i>	treatment of Ep-CAM-positive squamous cell carcinoma of the head and neck	Phase I
rapamycin (mTOR) inhibitor	ARIAD Pharmaceuticals <i>Cambridge, MA</i>	treatment of bone sarcoma	Phase II (617) 494-0400
<b>Rencares</b> <sup>®</sup> (WX G250)	Wilex Biotechnology <i>Munich, Germany</i>	treatment of renal cell carcinoma	Phase III www.wilex.com
<b>Rexin-G</b> <sup>™</sup>	Epeius Biotechnologies <i>San Marino, CA</i>	treatment of pancreatic cancer	Phase I (626) 441-6695
RTA 744	Reata Pharmaceuticals <i>Dallas, TX</i>	treatment of malignant gliomas	Phase I (972) 865-2200

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
RX-0201	Rexahn <i>Rockville, MD</i>	treatment of glioblastoma	Phase I (240) 268-5300
		treatment of ovarian cancer	Phase I (240) 268-5300
		treatment of pancreatic cancer	Phase I (240) 268-5300
		treatment of renal cell carcinoma	Phase I (240) 268-5300
		treatment of stomach cancer	Phase I (240) 268-5300
SC-1 monoclonal antibody	Cambridge Antibody Technology <i>Palo Alto, CA</i> Debiovision <i>Montreal, Quebec</i>	treatment of patients with CD55 (sc-1)-positive gastric tumors	Phase I (650) 388-3300
SDF-1 (CTCE 9908)	Chemokine Therapeutics <i>Vancouver, British Columbia</i>	treatment of osteogenic sarcoma	Phase I (604) 822-0301
solasonine and solamargine	Solbec Pharmaceuticals <i>Osborne Park, Australia</i>	treatment of renal cell carcinoma	Phase I www.solbec.com.au
<b>SomatoTher™</b> 111-indium pentetretotide	Louisiana State University Medical Center Foundation <i>New Orleans, LA</i>	treatment of somatostatin receptor-positive neuroendocrine tumors	Phase II
SP 1049C	Supratek Pharma <i>Dorval, Quebec</i>	treatment of esophageal carcinoma	Phase II
SS1(dsFv)-PE38	Cambridge Antibody Technology <i>Palo Alto, CA</i>	treatment of epithelial ovarian cancer	Phase I (650) 388-3300
		treatment of malignant mesothelioma	Phase I (650) 388-3300
talactoferrin alfa	Agennix <i>Houston, TX</i>	treatment of renal cell carcinoma	Phase II (713) 552-1091
<b>Tarceva®</b> erlotinib	Genentech <i>South San Francisco, CA</i>	treatment of malignant gliomas	Phase II (650) 225-1000
<b>Taxoprexin®</b> docosahexanoic DHA-paclitaxel	Luitpold Pharmaceuticals <i>Shirley, NY</i>	treatment of pancreatic cancer (see also cancer, skin)	Phase II (631) 924-4000
		treatment of adenocarcinoma of the stomach or lower esophagus	Phase II (631) 924-4000
		treatment of hormone-refractory prostate cancer	Phase II (631) 924-4000
technetium Tc 99m pterotetramide	Endocyte <i>West Lafayette, IN</i>	for the identification of ovarian carcinomas	Phase II (765) 463-7175

## CANCER

Product Name	Sponsor	FDA Official Designation	Development Status
tegafur/gimeracil/ oteracil	Taiho Pharma USA <i>Princeton, NJ</i>	treatment of gastric cancer	Phase III
temsirolimus	Wyeth <i>Collegeville, PA</i>	treatment of renal cell carcinoma	Phase III (610) 902-1200
tirapazamine	sanofi-aventis <i>Bridgewater, NJ</i>	for the treatment of head and neck cancer	Phase III (800) 981-2491
<b>TransMID™</b>	Xenova <i>Berkshire, United Kingdom</i>	treatment of malignant tumors of the central nervous system	in clinical trials
<b>Trisenox®</b> arsenic trioxide	Cephalon <i>Frazer, PA</i>	treatment of malignant glioma (see also cancer, blood)	Phase II (610) 344-0200
<b>Virulizin®</b>	Lorus Therapeutics <i>Toronto, Ontario</i>	treatment of pancreatic cancer	Phase III (416) 798-1200
<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
XR303	Xenova Biomedix <i>Berkshire, United Kingdom</i>	treatment of pancreatic cancer	Phase I
<b>Yondelis®</b> trabectedin	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	treatment of patients with epithelial ovarian cancer ----- treatment of soft tissue sarcoma	Phase III (800) 817-5286  Phase II (800) 817-5286
<b>Zactima™</b>	AstraZeneca <i>Wilmington, DE</i>	treatment of patients with follicular thyroid carcinoma, medullary vandetanib thyroid carcinoma, anaplastic thyroid carcinoma, and locally advanced and metastatic papillary thyroid carcinoma	Phase II (302) 886-3000

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
17-AGG	Kosan Biosciences <i>Hayward, CA</i>	treatment of multiple myeloma ----- treatment of chronic myelogenous leukemia	Phase I (510) 732-8400  Phase I (510) 732-8400
<b>Actimid™</b> CC 4047	Celgene <i>Warren, NJ</i>	treatment of multiple myeloma	Phase II (908) 673-9000
<b>Amsidyl®</b> amsacrine	Pfizer <i>New York, NY</i>	treatment of acute adult leukemia	application submitted (860) 732-5156
atiprimod	Callisto Pharmaceuticals <i>New York, NY</i>	treatment of multiple myeloma and associated bone resorption (see also cancer)	Phase I (212) 297-0010

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Bethathine</b> <sup>®</sup> beta alethine	LifeTime Pharmaceuticals <i>College Park, MD</i>	treatment of multiple myeloma	Phase I (301) 314-1480
<b>Ceplene</b> <sup>™</sup> histamine	EpiCept <i>Tarrytown, NY</i>	adjunct to cytokine therapy in the treatment of acute myeloid leukemia	Phase III (914) 606-3500
CHIR 12.12 (HCD-122)	Novartis Pharmaceutical <i>East Hanover, NJ</i>	treatment of chronic lymphocytic leukemia	Phase I (888) NOW-NOVA
		treatment of multiple myeloma	Phase I (888) NOW-NOVA
<b>Clofarex</b> <sup>™</sup> clofarabine	Genzyme <i>Cambridge, MA</i>	treatment of acute myelogenous leukemia	application submitted (617) 252-7500
<b>Cloretazine</b> <sup>®</sup>	Vion Pharmaceuticals <i>New Haven, CT</i>	treatment of acute myelogenous leukemia	Phase III (203) 498-4210
<b>Dacogen</b> <sup>™</sup> decitabine	MGI Pharma <i>Bloomington, MN</i>	acute myeloid leukemia (see also genetic)	Phase III (952) 346-4700
		treatment of chronic myelogenous leukemia	Phase II (952) 346-4700
<b>Doxil</b> <sup>®</sup> doxorubicin HCL liposome injection	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	treatment of multiple myeloma	Phase III (800) 817-5286
epratuzumab	Immunomedics <i>Morris Plains, NJ</i>	treatment of non-Hodgkin's lymphoma	Phase II (973) 605-8200
<b>Fodosine</b> <sup>™</sup> forodesine	BioCryst Pharmaceuticals <i>Birmingham, AL</i>	treatment of chronic lymphocytic leukemia and related leukemias to include prolymphocytic leukemia, adult T-cell leukemia, and hairy cell leukemia	Phase II (205) 444-4600
		treatment of T-cell non-Hodgkin's lymphoma	Phase II (205) 444-4600
		treatment of acute lymphoblastic leukemia	Phase I (205) 444-4600
<b>Genasense</b> <sup>®</sup> oblimersen	Genta <i>Berkeley Heights, NJ</i>	treatment of acute myelocytic leukemia	Phase III (908) 286-9800
		treatment of chronic lymphocytic leukemia	Phase III (908) 286-9800
GTI-2040	Lorus Therapeutics <i>Toronto, Ontario</i>	treatment of acute myeloid leukemia (see also cancer)	Phase I (416) 798-1200
homoharringtonine	ChemGenex Pharmaceutical <i>Menlo Park, CA</i>	treatment of chronic myelogenous leukemia	Phase II (650) 474-9800
imexon	AmpliMed <i>Tucson, AZ</i>	treatment of multiple myeloma (see also cancer; cancer, skin)	Phase I (520) 529-1000

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
lestaurtinib (CEP 701)	Cephalon <i>Frazer, PA</i>	treatment of acute myeloid leukemia	Phase II (610) 344-0200
lintuzumab	PDL BioPharma <i>Fremont, CA</i>	treatment of acute myelogenous leukemia	Phase I (510) 574-1400
liposomal annamycin	Callisto Pharmaceuticals <i>New York, NY</i>	treatment of acute myeloid leukemia	Phase I (212) 297-0010
		treatment of acute lymphoblastic leukemia	Phase I (212) 297-0010
lumiliximab	Biogen Idec <i>Cambridge, MA</i>	treatment of chronic lymphocytic leukemia	Phase II (617) 679-2000
MDX-060	Medarex <i>Princeton, NJ</i>	treatment of CD30+ T-cell lymphoma	Phase II (609) 430-2880
		treatment of Hodgkin's disease	Phase II (609) 430-2880
MEDI-538	MedImmune Oncology <i>Gaithersburg, MD</i>	treatment of indolent B-cell lymphoma, excluding CLL and NHL with CNS involvement	Phase I (301) 398-0000
obatoclox mesylate	Gemin X <i>Malvern, PA</i>	treatment of chronic lymphocytic leukemia	Phase I (514) 281-8989
<b>Panzem®</b> NCD (2ME2)	EntreMed <i>Rockville, MD</i>	treatment of multiple myeloma (see also cancer)	Phase II (240) 864-2600
plitidepsin	PharmaMar USA <i>Cambridge, MA</i>	treatment of multiple myeloma	Phase II (617) 621-5300
		treatment of acute lymphoblastic leukemia	Phase II (617) 621-5300
pralatrexate (PDX)	Allos Therapeutics <i>Westminster, CO</i>	treatment of T-cell lymphoma	Phase II (303) 426-6262
prednimustine	Pfizer <i>New York, NY</i>	treatment of malignant non-Hodgkin's lymphoma	Phase I (860) 760-5156
R-(-)-gossypol	Ascenta Therapeutics <i>San Diego, CA</i>	treatment of chronic lymphocytic leukemia	Phase II (858) 436-1200
<b>Rituxan®</b> rituximab	Genentech <i>South San Francisco, CA</i>	treatment of chronic lymphocytic leukemia (see also autoimmune)	Phase III (650) 225-1000
romidepsin	Gloucester Pharmaceuticals <i>Cambridge, MA</i>	treatment of cutaneous T-cell lymphoma	Phase II (617) 583-1300
SGN-30 (anti-CD30 mAb)	Seattle Genetics <i>Bothell, WA</i>	treatment of CD30-positive T-cell lymphomas	Phase II (425) 527-4000
		treatment of Hodgkin's disease	Phase II (425) 527-4000

## CANCER, BLOOD

Product Name	Sponsor	FDA Official Designation	Development Status
SGN-40 (anti-CD40 mAb)	Seattle Genetics <i>Seattle, WA</i>	treatment of chronic lymphocytic leukemia	Phase I (425) 527-4000
		treatment of multiple myeloma	Phase I (425) 527-4000
siplizumab (MEDI-507)	MedImmune Oncology <i>Gaithersburg, MD</i>	treatment of T-cell lymphomas	Phase I (301) 398-0000
<b>Talvesta™</b> talotrexin	Hana Biosciences <i>South San Francisco, CA</i>	treatment of acute lymphoblastic leukemia	Phase I/II (660) 588-6404
<b>Tasigna®</b> nilotinib	Novartis Pharmaceutical <i>East Hanover, NJ</i>	treatment of chronic myelogenous leukemia	application submitted (888) NOW-NOVA
<b>Theralux™</b>	Celmed BioSciences <i>Saint-Laurent, Quebec</i>	treatment of chronic myelogenous leukemia (photodynamic therapy)	Phase I (514) 336-4886
<b>Trisenox®</b> 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 II (610) 344-0200
		treatment of chronic myeloid leukemia	Phase II (610) 344-0200
		treatment of multiple myeloma	Phase II (610) 344-0200
vorinostat	Merck <i>Whitehouse Station, NJ</i>	treatment of multiple myeloma	Phase I (800) 672-6372
zanolimumab (human anti-CD4 monoclonal antibody)	Genmab <i>Princeton, NJ</i>	treatment of mycosis fungoides (cutaneous T-cell lymphoma)	Phase III (609) 430-2481
<b>Zarnestra®</b> tipifarnib	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	treatment of acute myeloid leukemia	application submitted (800) 817-5286
zosuquidar trihydrochloride	Kanisa Pharmaceuticals <i>San Diego, CA</i>	treatment of acute myeloid leukemia	Phase III (858) 436-1800

## CANCER, SKIN

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Allovetin-7®</b>	Vical <i>San Diego, CA</i>	treatment of invasive and metastatic melanoma (stages II, III, and IV)	Phase II (858) 646-1100
<b>Cavatak™</b> coxsackievirus A21	Viralytics <i>North Ryde, Australia</i>	treatment of stage II (T4), stage III, and stage IV melanoma	Phase I www.viralytics.com
CNT095	Centocor <i>Horsham, PA</i>	treatment of patients with high-risk stage II, stage III, and stage IV malignant melanoma	Phase I (610) 651-6000

## CANCER, SKIN

Product Name	Sponsor	FDA Official Designation	Development Status
human immunoglobulin (IgG1k) anti-CTLA-4 MDX-010 (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	Phase III (212) 546-4000
imexon	AmpliMed <i>Tucson, AZ</i>	treatment of metastatic malignant melanoma (see also cancer; cancer, blood)	Phase I (520) 529-1000
<b>IntraDose</b> <sup>®</sup> cisplatin/epinephrine	Novartis Pharmaceutical <i>East Hanover, NJ</i>	treatment of metastatic malignant melanoma (see also cancer)	Phase III (888) NOW-NOVA
mannopentaose phosphate sulfate	Progen Industries <i>Richlands, Australia</i>	treatment of high-risk stage II, stage III, and stage IV melanoma	Phase II
MEDI-522	MedImmune Oncology <i>Gaithersburg, MD</i>	treatment of metastatic melanoma	Phase II (301) 398-0000
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
<b>M-Vax</b> <sup>®</sup>	Avax Technologies <i>Philadelphia, PA</i>	for adjuvant therapy in melanoma patients with surgically resectable lymph node metastases (stage III and limited stage IV disease)	Phase I (215) 241-9760
<b>Nexavar</b> <sup>®</sup> sorafenib	Bayer Pharmaceuticals <i>West Haven, CT</i> Onyx Pharmaceuticals <i>Emeryville, CA</i>	treatment of stage IIB through stage IV melanoma (see also cancer)	Phase III (203) 812-2000 (510) 597-6500
<b>Oncophage</b> <sup>®</sup> vitespen	Antigenics <i>New York, NY</i>	treatment of metastatic melanoma (see also cancer)	Phase III (212) 994-8200
pegylated arginine deiminase	Phoenix Pharmacologics <i>San Diego, CA</i>	treatment of invasive malignant melanoma (see also cancer)	Phase I (858) 452-6688
recombinant human interleukin-21 (rIL-21)	ZymoGenetics <i>Seattle, WA</i>	treatment of stage II (T4), III or IV malignant melanoma	Phase I (206) 442-6600
T4N5 liposome lotion	AGI Dermatics <i>Freeport, NY</i>	to prevent cutaneous neoplasms and other skin abnormalities in xeroderma pigmentosum	Phase III (516) 868-9026
<b>Taxoprexin</b> <sup>®</sup> docosahexanoic DHA-paclitaxel	Luitpold Pharmaceuticals <i>Shirley, NY</i>	treatment of metastatic malignant melanoma (see also cancer)	Phase III (631) 924-4000

## CANCER, SKIN

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Temodar®</b> temozolomide	Schering-Plough <i>Kenilworth, NJ</i>	treatment of advanced metastatic melanoma	application submitted (908) 298-4000
ticilimumab (CTLA4 mAb)	Pfizer <i>New York, NY</i>	treatment of stage IIb to stage IV metastatic melanoma	Phase III (860) 732-5156
<b>Zadaxin®</b> thymalfasin	SciClone Pharmaceuticals <i>San Mateo, CA</i> Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	treatment of stage IIb through stage IV malignant melanoma (see also infectious)	Phase II (650) 358-3456

## CANCER-RELATED CONDITIONS

Product Name	Sponsor	FDA Official Designation	Development Status
dexrazoxane	TopoTarget <i>Copenhagen, Denmark</i>	treatment of anthracycline extravasation during chemotherapy	application submitted
<b>Leukotec®</b> inolimomab	Opi <i>Limonest, France</i>	treatment of graft-versus-host-disease	Phase III
polyethylene glycol-modified uricase (PEG-uricase)	Phoenix Pharmacologics <i>San Diego, CA</i>	prophylaxis of hyperuricemia in cancer patients prone to develop tumor lysis syndrome during chemotherapy (see also other)	Phase I (858) 452-6688
<b>Prochymal™</b>	Osiris Therapeutics <i>Baltimore, MD</i>	treatment of acute graft-versus-host-disease	Phase III (410) 522-5005
<b>Prograf®</b> tacrolimus	Astellas Pharma US <i>Deerfield, IL</i>	prophylaxis of graft-versus-host-disease	application submitted (800) 888-7704
recombinant human fibroblast growth factor-20 (velofermin)	CuraGen <i>Branford, CT</i>	treatment of radiation-induced oral mucositis (stomatitis)	Phase II (888) 436-6642
sodium thiosulfate	Adherex Technologies <i>Durham, NC</i>	prevention of platinum-induced ototoxicity in pediatric patients	Phase II (919) 484-8484
<b>StemEx®</b>	Gamida Cell <i>Jerusalem, Israel</i> Teva Pharmaceuticals USA <i>North Wales, PA</i>	for use as hematopoietic support in patients with relapsed or refractory hematologic malignancies who are receiving high-dose therapy	Phase I (215) 591-3000
<b>Xerecept®</b> corticoreslin acetate injection	Celtic Pharma <i>Hamilton, Bermuda</i> Neurobiological Technologies <i>Richmond, CA</i>	treatment of peritumoral brain edema	Phase III (212) 616-4000 (510) 595-6000

## CARDIOVASCULAR DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
implitapide	Medical Research Laboratories International <i>Highland Heights, KY</i>	treatment of homozygous familial hypercholesterolemia	Phase II
ISIS 301012	Isis Pharmaceuticals <i>Carlsbad, CA</i>	treatment of homozygous familial hypercholesterolemia	Phase II (760) 931-9200
novel acting thrombolytic (NAT)	Nuvelo <i>San Carlos, CA</i>	treatment of peripheral arterial occlusion (PAO)	Phase III (650) 517-8000
recombinant human microplasmin	ThromboGenics <i>Dublin, Ireland</i>	treatment of peripheral arterial occlusion	Phase I
tilarginine acetate	ArgiNOx Pharmaceuticals <i>Redwood City, CA</i>	treatment of cardiogenic shock	Phase III (888) 274-6070
<b>Trinam™</b> ethanolamine	Ark Therapeutics <i>London, England</i>	prevention of complications due to neointimal hyperplasia disease in certain vascular anastomoses	Phase II
vapreotide	Debiovision <i>Montreal, Quebec</i>	treatment of esophageal variceal hemorrhage patients with portal hypertension	application submitted

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
40SD02	Biomedical Frontiers <i>Minneapolis, MN</i>	treatment of chronic iron overload resulting from conventional transfusional treatment of beta-thalassemia major and sickle cell anemia	Phase II
AER002	Aerovance <i>Berkeley, CA</i>	treatment of cystic fibrosis	Phase II (510) 549-5500
AT 1001 (migalast)	Amicus Therapeutics <i>Cranbury, NJ</i>	treatment of Fabry disease	Phase II (609) 662-2000
cystic fibrosis gene therapy	Copernicus Therapeutics <i>Cleveland, OH</i>	treatment of cystic fibrosis	Phase I (216) 231-0227
<b>Cystoran™</b> cysteamine HCl	Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	treatment of corneal cystine crystal accumulation in cystinosis patients	Phase III (800) 447-0169
<b>Dacogen™</b> decitabine	MGI Pharma <i>Bloomington, MN</i>	treatment of sickle cell anemia (see also cancer)	Phase II (952) 346-4700
DCF 987 (dextran 1)	BCY LifeSciences <i>Toronto, Ontario</i>	treatment of cystic fibrosis	Phase II (416) 484-4666
denufosal tetrasodium	Inspire Pharmaceuticals <i>Durham, NC</i>	treatment of cystic fibrosis	Phase III (919) 941-9777
duramycin	Lantibio <i>Chapel Hill, NC</i>	treatment of cystic fibrosis	Phase II (919) 960-0217

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
factor XIII [A2] homodimer, recombinant DNA origin	Novo Nordisk <i>Princeton, NJ</i>	treatment of congenital FXIII deficiency	Phase I (609) 987-5800
		prophylaxis of bleeding associated with congenital FXIII deficiency	Phase I (609) 987-5800
<b>Fosamax</b> <sup>®</sup> alendronate	Merck <i>Whitehouse Station, NJ</i>	treatment of osteogenesis imperfecta in pediatric patients 4 years of age and older	application submitted (800) 672-6372
<b>Hemoxin</b> <sup>™</sup>	Xechem International <i>New Brunswick, NJ</i>	treatment of sickle cell disease	Phase II (732) 247-3300
ICA 17043 bis (4-fluorophenyl) phenylacetamide	ICAgen <i>Durham, NC</i>	treatment of sickle cell disease	Phase III (919) 941-5206
idebenone (INN)	Santhera Pharmaceuticals <i>Liestal, Switzerland</i>	treatment of cardiomyopathy associated with Friedreich's ataxia	Phase II www.santhera.com
		treatment of Leber's hereditary optic neuropathy	Phase II www.santhera.com
INS 316	Inspire Pharmaceuticals <i>Durham, NC</i>	treatment of cystic fibrosis (see also respiratory)	Phase II (919) 941-9777
isofagomine tartrate (AT-2101)	Amicus Therapeutics <i>Cranbury, NJ</i>	treatment of Gaucher disease	Phase I (609) 662-2000
<b>Liazal</b> <sup>®</sup> liarozole	Barrier Therapeutics <i>Princeton, NJ</i>	treatment of congenital ichthyosis	Phase II (609) 945-1200
mannitol	Pharmaxis <i>Frenchs Forest, Australia</i>	to facilitate clearance of mucus in patients with bronchiectasis and in patients with cystic fibrosis at risk for bronchiectasis	Phase III www.pharmaxis.com
methylbicyclone (SPI-8811)	Sucampo Pharmaceuticals <i>Bethesda, MD</i>	treatment of cystic fibrosis	Phase II (301) 961-3400
oxalobacter formigenes (OC3)	OxThera <i>Alachua, FL</i>	treatment of primary hyperoxaluria	Phase I (386) 418-1428
PTC 124	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	Phase II (908) 222-7000
		treatment of muscular dystrophy resulting from premature stop mutations in the dystrophic gene	Phase II (908) 222-7000
recombinant adeno-associated virus alpha 1- antitrypsin vector	Applied Genetic Technologies <i>Alachua, FL</i>	treatment of alpha1-antitrypsin deficiency	Phase I (386) 462-2204

## GENETIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
recombinant human C1-esterase inhibitor	Pharming <i>Leiden, The Netherlands</i>	treatment of (acute attacks of) angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency, prophylactic treatment of angioedema caused by hereditary or acquired C1-esterase inhibitor deficiency	Phase III
recombinant human neutrophil elastase (hNE)	Dyax <i>Cambridge, MA</i>	treatment of cystic fibrosis	Phase II (617) 225-2500
rilonacept (interleukin-1 trap)	Regeneron Pharmaceuticals <i>Tarrytown, NY</i>	treatment of CIAS1-associated periodic syndromes (CAPS) (see also autoimmune)	Phase III (914) 345-7400
thymosin beta 4	RegeneRx Biopharmaceuticals <i>Bethesda, MD</i>	treatment of epidermolysis bullosa	Phase II (301) 280-1992

## INFECTIOUS DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
<b>ABthrax™</b> (PA mAb)	Human Genome Sciences <i>Rockville, MD</i>	treatment of anthrax	Phase I (301) 309-8504
<b>AmBisome®</b> liposomal amphotericin B	Astellas Pharma US <i>Deerfield, IL</i> Gilead Sciences <i>Foster City, CA</i>	treatment of histoplasmosis	Phase II (800) 695-4321 (650) 574-3000
amphotericin B inhalation powder	Nektar Therapeutics <i>San Carlos, CA</i>	prevention of pulmonary fungal infections in patients at risk for aspergillosis due to immunosuppressive therapy, including those receiving organ or stem cell transplants, or treated with chemotherapy or radiation for hematologic malignancies	Phase I (650) 631-3100
<b>Ampligen®</b>	HEM Pharmaceutical <i>Philadelphia, PA</i>	treatment of AIDS (see also autoimmune)	Phase II (800) 332-6854
<b>Anthim™</b> (ETI-204)	Elusys Therapeutics <i>Pine Brook, NJ</i>	treatment of exposure to <i>Bacillus anthracis</i> spores	Phase I (973) 808-0222
<b>Cayston™</b> aztreonam lysine for inhalation	Corus Pharma <i>Seattle, WA</i> 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
chimeric, humanized mAb to staphylococcus (BSYX-A110)	Biosynexus <i>Gaithersburg, MD</i> MedImmune <i>Gaithersburg, MD</i>	prophylaxis of <i>Staphylococcus epidermidis</i> sepsis in low birth weight (1500 grams or less) infants	Phase II (301) 330-5800 (301) 398-0000

## INFECTIOUS DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
chimeric monoclonal antibodies	Caprion Pharmaceuticals <i>Montreal, Ontario</i>	treatment of shiga-toxin producing bacterial infection	Phase I (514) 940-3600
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
doripenem	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	treatment of bronchopulmonary infection in patients with cystic fibrosis who are colonized with <i>Pseudomonas aeruginosa</i> or <i>Burkholderia cepacia</i>	Phase I (800) 817-5286
hepatitis C virus immune globulin (human)	NABI Biopharmaceuticals <i>Boca Raton, FL</i>	prophylaxis of hepatitis C infection in liver transplant recipients	Phase I (561) 989-5809
HepeX-B	Cubist Pharmaceuticals <i>Lexington, MA</i>	prevention of hepatitis B virus (HBV) reinfection in patients who have received a liver transplantation	Phase II (781) 860-8660
Hsp E7	Nventa Biopharmaceuticals <i>San Diego, CA</i>	treatment of recurrent respiratory papillomatosis (RRP)	Phase II (858) 202-4900
immune globulin (human) containing high titers of West Nile virus antibodies	OMRIX Biopharmaceuticals <i>New York, NY</i>	treatment of the West Nile virus infection	Phase I (212) 887-6500
KBPA 101 (human mAb directed against serotype 011 <i>Pseudomonas aeruginosa</i> )	Kenta Biotech <i>Berne, Switzerland</i>	treatment of hospital-acquired pneumonia caused by serotype 011 positive <i>Pseudomonas aeruginosa</i>	Phase II
<b>Nabi-HB</b> <sup>®</sup> hepatitis B immune globulin intravenous (human)	NABI Biopharmaceuticals <i>Boca Raton, FL</i>	prophylaxis against hepatitis B virus reinfection in liver transplant patients	application submitted (561) 989-5800
natural human lymphoblastoid interferon-alpha	Amarillo Biosciences <i>Amarillo, TX</i>	treatment of papillomavirus warts in the oral cavity of HIV-positive patients	Phase II (806) 376-1741
<b>Noxafil</b> <sup>®</sup> posaconazole	Schering-Plough <i>Kenilworth, NJ</i>	treatment of zygomycosis	application submitted (908) 298-4000
<b>Paromomycin</b> aminosidase	The Institute for One World Health <i>San Francisco, CA</i>	treatment of visceral leishmaniasis	Phase III (415) 421-4700

## INFECTIOUS DISEASES

Product Name	Sponsor	FDA Official Designation	Development Status
recombinant human mAb to hsp90	NeuTec Pharma <i>Manchester, England</i>	treatment of invasive candidiasis	Phase II
<b>SLIT™ Amikacin</b>	Transave <i>Monmouth Junction, NJ</i>	treatment of bronchopulmonary <i>Pseudomonas aeruginosa</i> infections in cystic fibrosis patients	Phase II (732) 438-9434
<i>Staphylococcus aureus</i> immune globulin (human)	Nabi Biopharmaceuticals <i>Boca Raton, FL</i>	prophylaxis against <i>Staphylococcus aureus</i> infections in low birth weight neonates	Phase II (561) 989-5000
TNX-355	Tanox <i>Houston, TX</i>	for use in post-exposure prophylaxis for occupational exposure to human immunodeficiency virus (HIV)	Phase III (713) 578-4000
<b>Valortim™</b> MDX-1303	Medarex <i>Princeton, NJ</i> PharmAthene <i>Annapolis, MD</i>	treatment of anthrax infection	Phase I (609) 430-2880 (410) 571-8920
varicella zoster immune globulin (human)	Cangene <i>Winnipeg, Manitoba</i>	passive immunization for the treatment of exposed, susceptible individuals who are at risk for complications from varicella	Phase III (204) 275-4178
<b>Veronate®</b> INH-A00021	Inhibitex <i>Alpharetta, GA</i>	reduction (prevention) of nosocomial bacteremia caused by staphylococci in very low birth weight infants	application submitted (678) 746-1100
XP-28 (xepol)	Pharmalink <i>Stockholm, Sweden</i>	treatment of post-polio syndrome	Phase III
<b>Zadaxin®</b> thymalfasin	SciClone Pharmaceuticals <i>San Mateo, CA</i>	treatment of chronic active hepatitis B (see also cancer)	Phase II (650) 358-3456

## NEUROLOGIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
4975	Anesvia <i>South San Francisco, CA</i>	treatment of intermetatarsal neuroma (Morton's neuroma) that does not respond to conservative treatment and requires either neurectomy or neurolysis	Phase II (650) 624-9600
ACR-16	NeuroSearch AB <i>Goteborg, Sweden</i>	treatment of Huntington's disease	Phase II <a href="http://www.neurosearch.com">www.neurosearch.com</a>
AEOL 10150	Aeolus Pharmaceuticals <i>Laguna Niguel, CA</i>	treatment of amyotrophic lateral sclerosis (ALS)	Phase I (949) 481-9825

## NEUROLOGIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
anatibant (XY 2405)	Xytis Pharmaceuticals <i>Irvine, CA</i>	treatment of patients having experienced a severe traumatic brain injury (Glasgow Coma Scale 3 to 8) in order to decrease early mortality and improve long-term functional and neurological outcome	Phase II (949) 226-1118
arimoclomol (BRX-345)	CytRx <i>Los Angeles, CA</i>	treatment of amyotrophic lateral sclerosis (ALS)	Phase II (301) 826-5648
autologous incubated macrophage	Proneuron Biotechnologies <i>Los Angeles, CA</i>	therapy to improve the motor and sensory neurological outcome in acute cases of spinal cord injury	Phase II www.proneuron.com
BA 210	BioAxone Therapeutics <i>Montreal, Quebec</i>	treatment of acute spinal cord injury	Phase I (514) 282-9990
<b>Botox®</b>	Allergan <i>Irvine, CA</i>	treatment of dynamic muscle contracture in pediatric cerebral palsy patients	Phase III (714) 246-4500
capsaicin	TheraQuest Biosciences <i>Blue Bell, PA</i>	management of postherpetic neuralgia	Phase II (610) 272-2071
ceftriaxone sodium	Massachusetts General Hospital <i>Charlestown, MA</i>	treatment of amyotrophic lateral sclerosis (ALS)	Phase III
civamide	Winston Laboratories <i>Vernon Hills, IL</i>	treatment of postherpetic neuralgia of the trigeminal nerve	Phase II (847) 362-8200
cladribine	EMD Serono <i>Rockland, MA</i>	treatment of the chronic progressive form of multiple sclerosis	Phase III (800) 283-8088
clazosentan	Actelion Pharmaceuticals US <i>South San Francisco, CA</i>	treatment of cerebral vasospasm following subarachnoid hemorrhage	Phase II (650) 624-6900
contulakin-G	Cognetix <i>Salt Lake City, UT</i>	intrathecal treatment of neuropathic pain associated with spinal cord injury	Phase I (801) 581-0400
creatine (ALS-02) (HD-02)	Avicena Group <i>Palo Alto, CA</i>	treatment of amyotrophic lateral sclerosis (ALS)	Phase III (415) 397-2880
		treatment of Huntington's disease	Phase II (415) 397-2880
<b>Duodopa®</b> levodopa and carbidopa	Solvay Pharmaceuticals <i>Marietta, GA</i>	treatment of late-stage Parkinson's disease	Phase III (770) 578-9000
<b>Dysport®</b> botulinum toxin type A	Ipsen <i>Milford, MA</i>	treatment of spasmodic torticollis (cervical dystonia)	Phase III (508) 478-8900
E2080	Eisai <i>Ridgefield Park, NJ</i>	treatment of Lennox-Gastaut syndrome	application submitted (201) 403-2500

## NEUROLOGIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
ethyl eicosapentaenoate	Laxdale <i>Stirling, United Kingdom</i>	treatment of Huntington's disease	Phase III
fampridine-SR	Acorda Therapeutics <i>Hawthorne, NY</i>	treatment of chronic, incomplete spinal cord injury	Phase III (914) 347-4300
		relief of symptoms of multiple sclerosis	Phase III (914) 347-4300
Fx1006A	FoldRx Pharmaceuticals <i>Cambridge, MA</i>	treatment of familial amyloid polyneuropathy	Phase I (617) 252-5500
immune globulin (human)	Talecris Biotherapeutics <i>Rsch. Triangle Park, NC</i>	treatment of chronic inflammatory demyelinating polyneuropathy	Phase II (919) 316-6300
LY 156735 (PD6735)	Phase 2 Discovery <i>Cincinnati, OH</i> Eli Lilly <i>Indianapolis, IN</i>	treatment of circadian rhythm sleep disorders in blind people with no light perception	Phase II (513) 475-6618 (317) 276-2000
methionine/L-methionine	Genopia USA <i>New York, NY</i>	treatment of AIDS myelopathy	Phase II
MYO-029	Wyeth <i>Collegeville, PA</i>	treatment of Duchenne and Becker muscular dystrophies	Phase II (610) 902-1200
<b>Myodur™</b>	Ceptor <i>Hunt Valley, MD</i>	treatment of boys with Duchenne muscular dystrophy	Phase I/II (410) 527-9998
NH-001	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)	Phase II (617) 331-4111
S-adenosyl-methionine	Genopia USA <i>New York, NY</i>	treatment of AIDS myelopathy	Phase II (800) 238-8088
<b>Spheramine®</b>	Schering AG <i>Berlin, Germany</i> Titan Pharmaceuticals <i>South San Francisco, CA</i>	treatment of Hoehn and Yahr stage 3 and 4 Parkinson's disease (advanced Parkinson's disease)	Phase II (650) 244-4990
tramadol hydrochloride (TQ-1017)	TheraQuest Biosciences <i>Blue Bell, PA</i>	treatment of painful HIV-associated neuropathy	Phase I (610) 272-2071
		management of postherpetic neuralgia	Phase I (610) 272-2071
<b>Transacin™</b> (NGX-4010)	NeurogesX <i>San Carlos, CA</i>	treatment of painful HIV-associated neuropathy	Phase II (650) 508-2116
TRO-19622	Trophos SA <i>Cedex, France</i>	treatment of amyotrophic lateral sclerosis	Phase I www.trophos.com
ubiquinol	Gel-Tec, Tishcon <i>Westbury, NY</i>	treatment of Huntington's disease	Phase III (516) 333-3050
		treatment of mitochondrial cytopathies	Phase I (516) 333-3050

## NEUROLOGIC DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
vigabatrin	Ovation Pharmaceuticals <i>Deerfield, IL</i>	treatment of infantile spasms	in clinical trials (847) 282-1000
<b>Xenazine®</b> tetrabenazine	Prestwick Pharmaceuticals <i>Washington, DC</i>	treatment of Huntington's disease	application submitted (202) 296-1400

## RESPIRATORY DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
alpha1-proteinase inhibitor (human)	Kamada <i>Ness Ziona, Israel</i>	chronic inhalation therapy of individuals with congenital deficiency of alpha1-proteinase inhibitor with demonstrable panacinar emphysema	Phase III www.kamada.com
alpha1-proteinase inhibitor (human), inhaled	CSL Behring <i>King of Prussia, PA</i> Nektar Therapeutics <i>San Carlos, CA</i>	for slowing the progression of emphysema in alpha1-antitrypsin deficient patients	Phase II (610) 878-4000
ambrisentan	Gilead Sciences <i>Foster City, CA</i>	treatment of pulmonary arterial hypertension	application submitted (650) 574-3000
Hsp E7	Nventa Pharmaceuticals <i>San Diego, CA</i>	treatment of recurrent respiratory papillomatosis (RRP)	Phase II (858) 202-4900
<b>INOmax</b> nitric oxide	INO Therapeutics <i>Clinton, NJ</i>	treatment of acute respiratory distress syndrome in adults	in clinical trials (908) 238-6600
		to reduce the risk of chronic lung disease in premature neonates	in clinical trials (908) 238-6600
INS 316	Inspire Pharmaceuticals <i>Durham, NC</i>	to facilitate the removal of lung secretions in the treatment of patients with primary ciliary dyskinesia (see also genetic)	Phase II (919) 941-9777
lusupultide (rSP-C lung surfactant)	Altana Pharma <i>Florham Park, NJ</i>	treatment of adult respiratory distress syndrome	Phase III (973) 514-4240
pirfenidone	InterMune <i>Brisbane, CA</i>	treatment of idiopathic pulmonary fibrosis	Phase III (415) 466-2200
<b>PulmoLAR™</b>	PR Pharmaceuticals <i>Fort Collins, CO</i>	treatment of pulmonary arterial hypertension	Phase I (970) 484-5560
recombinant human Clara cell 10kDa protein	Claragen <i>College Park, MD</i> CCIO Sweden <i>Stockholm, Sweden</i>	prevention of neonatal bronchopulmonary dysplasia in premature neonates with respiratory distress syndrome	Phase I (301) 452-2899
sitaxsentan	Encysive <i>Houston, TX</i>	treatment of pulmonary arterial hypertension in the absence of chronic obstructive pulmonary disease or congestive heart failure	application submitted (713) 796-8822

## RESPIRATORY DISORDERS

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Surfaxin</b> <sup>®</sup> lucinactant	Discovery Laboratories <i>Warrington, PA</i>	treatment of respiratory distress syndrome in premature infants	application submitted (215) 488-9300
		treatment of acute respiratory distress syndrome in adults	Phase II (215) 488-9300
vasoactive intestinal polypeptide (aviptadil)	Biogen Idec <i>Cambridge, MA</i> mondoBIOTECH Laboratories <i>Anstalt, Switzerland</i>	treatment of pulmonary arterial hypertension	Phase II (617) 679-2000

## TRANSPLANTATION

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Apomate</b> <sup>™</sup> (technetium Tc99m rh-annexin V)	North American Scientific <i>Chatsworth, CA</i>	diagnosis or assessment of rejection status in heart, heart-lung, single lung, or bilateral lung transplants	Phase II (818) 734-8600
<b>Mozobil</b> <sup>™</sup> plerixafor	Genzyme <i>Cambridge, MA</i>	for combination use with filgrastim to improve the yield of progenitor cells in the apheresis product for subsequent stem cell transplantation following myelosuppressive or myeloablative chemotherapy	Phase III (617) 252-7500
PD03491390	Pfizer <i>New York, NY</i>	treatment of patients undergoing solid organ transplantation	Phase II (860) 723-5156
recombinant Epstein-Barr virus gp350 glycoprotein vaccine	Henogen <i>Charleroi, Belgium</i>	prevention of post-transplantation lymphoproliferative disorders in pediatric recipients of solid-organ transplantation	Phase I
recombinant P-selectin glycoprotein ligand (YSPSL)	Y's Therapeutics <i>Burlingame, CA</i>	prevention of delayed graft function in renal transplant patients	Phase II (650) 777-7000
reparixin	Dompe <i>L'Aquila, Italy</i>	prevention of delayed graft function in solid organ transplant	Phase II
TK gene therapy	MolMed <i>Milan, Italy</i>	immunotherapy for acceleration of T-cell reconstitution in patients undergoing allogeneic hematopoietic stem cell transplantation	Phase I/II www.molmed.com

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
AMG 531	Amgen <i>Thousand Oaks, CA</i>	treatment of immune thrombocytopenic purpura	Phase III (805) 447-1000

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
aminocaproic acid	Eastern Virginia Medical School <i>Norfolk, VA</i> ISTA Pharmaceuticals <i>Irvine, CA</i>	topical treatment of traumatic hyphema of the eye	Phase III (949) 788-6000
<b>Analatro</b> <sup>®</sup> latroectus immune F(ab) <sub>2</sub>	Rare Disease Therapeutics <i>Nashville, TN</i>	treatment of black widow spider envenomations	Phase II (615) 399-0700
<b>Anavip</b> <sup>™</sup>	Rare Disease Therapeutics <i>Nashville, TX</i>	treatment of envenomation by Crotaline snakes	Phase II (615) 399-0700
C1 esterase inhibitor (human)	Lev Pharmaceuticals <i>New York, NY</i>	treatment of angioedema	Phase III (212) 682-3994
C1 esterase inhibitor, human, pasteurized	CSL Behring <i>King of Prussia, PA</i>	prevention and/or treatment of acute attacks of hereditary angioedema	Phase III (610) 878-4000
choline chloride	Feinberg School of Medicine <i>Chicago, IL</i>	prevention and/or treatment of choline deficiency in patients on long-term parenteral nutrition	Phase II (312) 695-4514
<b>Correcta</b> <sup>™</sup> clotrimazole	Effective Pharmaceuticals <i>New York, NY</i>	topical treatment of children and adults with pouchitis	Phase II/III (917) 224-7099
CR-002 (human mAb against platelet-derived growth factor D)	CuraGen <i>Branford, CT</i>	to slow the progression of IgA nephropathy and delay kidney failure in patients affected by the disease	Phase I
debrase gel dressing	MediWound <i>Yavne, Israel</i>	debridement of acute, deep dermal burns in hospitalized patients	Phase II
defibrotide	Gentium <i>Cono, Italy</i> Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	treatment of hepatic veno-occlusive disease	Phase III (800) 447-0169
dehydroepian-drosterone (DHEA)	Paladin Labs <i>Montreal, Quebec</i>	replacement therapy in individuals with adrenal insufficiency	Phase II (514) 340-1112
DX-88	Dyax <i>Cambridge, MA</i>	treatment of angioedema	Phase III (617) 225-2500
<b>ELAD</b> <sup>®</sup> 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 (858) 673-6840
glycopyrrolate	Sciele Pharma <i>Atlanta, GA</i>	treatment of pathologic (chronic moderate to severe) drooling in pediatric patients	Phase II (800) 461-3696
histrelin	Valera Pharmaceuticals <i>Cranbury, NJ</i>	treatment of central precocious puberty	application submitted (888) 262-8855

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
icatibant	Jerini <i>Berlin, Germany</i> KOS Pharmaceutical <i>Cranbury, NJ</i>	treatment of angioedema	Phase III (609) 495-0500
<b>Ipstyl®</b> lanreotide	IPSEN <i>Milford, MA</i>	treatment for acromegaly	Phase I (508) 478-0144
<b>Kiacta™</b> eprosidate	Neurochem <i>Saint-Laurent, Quebec</i>	treatment of secondary amyloidosis	application submitted
mepolizumab	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	first-line treatment in patients with hypereosinophilic syndrome	Phase II (888) 825-5649
MGA-031 (humanized anti-CD3 mAb)	Macrogenics <i>Rockville, MD</i>	treatment of recent-onset type I diabetes	Phase II/III (301) 251-5172
misoprostol	Gynuity <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	in clinical trials (212) 448-1230
<b>NovoSeven®</b>	Novo Nordisk <i>Princeton, NJ</i>	treatment of bleeding in patients experiencing intracranial hemorrhage	Phase III (877) 668-6777
NT 501	Neurotech USA <i>Lincoln, RI</i>	treatment of retinitis pigmentosa	Phase I (401) 333-3880
<b>Oralgam™</b> human gammaglobulin	PediaMed Pharmaceuticals <i>Florence, KY</i> Protein Therapeutics <i>Tucson, AZ</i>	treatment of gastrointestinal disturbances (to include constipation, diarrhea, and abdominal pain) associated with regression-onset autism in pediatric patients (see also autoimmune)	Phase II (520) 844-1187
phenylephrine	S.L.A. Pharma <i>Watford, United Kingdom</i>	treatment of ileal pouch anal anastomosis-related fecal incontinence	Phase II
<b>Plaquase™</b> collagenase (lyophilized) for injection	Auxilium Pharmaceuticals <i>Malvern, PA</i>	treatment of Peyronie's disease	Phase II (484) 321-5900
polyethylene glycol-modified uricase (PEG-uricase)	Phoenix Pharmacologics <i>San Diego, CA</i>	treatment of hyperuricemia in patients with gout refractory to conventional therapy or in whom conventional therapy is contraindicated (see also cancer)	Phase I (858) 452-6688
<b>Puricase®</b> polyethylene glycol (PEG)-uricase	Savient Pharmaceuticals <i>East Brunswick, NJ</i>	to control the clinical consequences of hyperuricemia in patients with severe gout in whom conventional therapy is contraindicated or has been ineffective	Phase III (732) 418-9300

## OTHER

Product Name	Sponsor	FDA Official Designation	Development Status
<b>Serostim™</b> somatropin	EMD Serono <i>Rockland, MA</i>	treatment of patients with HIV-associated adipose redistribution syndrome	application submitted (800) 238-8088
synthetic human secretin (RG1068)	Repligen <i>Waltham, MA</i>	for use in conjunction with diagnostic procedures (including ERCP) for pancreatic disorders to increase pancreatic fluid secretion	Phase II (800) 622-2259
terlipressin (INN)	Orphan Therapeutics <i>Lebanon, NJ</i>	treatment of hepatorenal syndrome	Phase III (908) 849-4805
<b>TheraCLEC-Total</b>	Altus Biologics <i>Cambridge, MA</i>	treatment of pancreatic insufficiency	Phase II (617) 299-2900
TRX 4	TolerRX <i>Cambridge, MA</i>	treatment of new-onset type 1 diabetes mellitus	Phase I (617) 354-8100
<b>Voraxaze®</b> glucarpidase	Protherics <i>Brentwood, TN</i>	treatment of patients at risk of methotrexate toxicity	Phase II (615) 327-1027
XG 102	Auris Medical <i>Chicago, IL</i>	treatment of acute sensorineural hearing loss	Phase I/II (312) 283-5633
<b>Xifaxan™</b> rifaximin	Salix Pharmaceuticals <i>Morrisville, NC</i>	treatment of hepatic encephalopathy	Phase III (919) 862-1000

The content of this report has been obtained through government and 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 November 20, 2006. **Report current as of January 2, 2007.** 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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## GLOSSARY

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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.

**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

## GLOSSARY

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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.

## **Additional Resources.**

Genetic Alliance at [www.geneticalliance.org](http://www.geneticalliance.org) and the National Organization for Rare Diseases (NORD) at [www.rarediseases.org](http://www.rarediseases.org).

Source: National Organization for Rare Disorders, Inc.

## 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		
<b>Years</b>	6.5	1.5	2	3.5	1.5	
<b>Test Population</b>	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
<b>Purpose</b>	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		
<b>Success Rate</b>	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 \$802 million to get one new medicine from the laboratory to U.S. patients, according to a November 2001 report 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 becomes effective if FDA does not disapprove it within 30 days. 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 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.

**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 28 new therapeutics approved by the FDA in 2005 was 12.5 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. PhRMA member companies invested an estimated \$43 billion in research and development in 2006.

The following organizations are partnering with PhRMA to present *“Orphan Drugs in Development for Rare Diseases.”*

**Genetic Alliance [www.geneticalliance.org](http://www.geneticalliance.org)**

Sharon F. Terry, MA, President and CEO, and Jannine D. Cody, Ph.D., Chair

Genetic Alliance, a coalition of more than 600 disease-specific advocacy organizations, serving 25 million Americans, salutes all those who work to bring drugs to market. We applaud all the men and women who enable innovation from early discovery to commercialization. Genetic Alliance leverages the voices of millions of individuals and families living with genetic conditions. We are committed to capacity building in all communities. The technical assistance we provide to advocacy organizations results in measurable growth: increased funding for research, access to services, and support for emerging technologies. Informed consumers, advocacy groups, and community organizations create a demand that is met by genomics technologies, tests, and treatments—thus creating a favorable business climate for the early adoption of new products that result from academia, biotechnology, and pharmaceutical organizations. We support PhRMA’s dedication to rare diseases and all the challenges they represent.

**National Organization for Rare Diseases (NORD) [www.rarediseases.org](http://www.rarediseases.org)**

Abbey S. Myers, President, and Carolyn Asbury, Ph.D., Chair, Board of Directors

NORD’s message of hope—*“out of darkness, into the light”*—rings truer today than ever before because of the successes of biomedical research in recent years. Thanks to the Orphan Drug Act, the dedication of patient organizations and the scientific community, and the development of powerful new research tools, treatments have been brought to market for many rare diseases that previously had none. As we read this report documenting the progress, we salute the researchers, the sponsors, and—most of all—the patients and families who never lose hope, even in the darkest hours.



*New Medicines. New Hope.®*

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