

Children

PRESENTED BY AMERICA'S PHARMACEUTICAL COMPANIES

More Than 200 Medicines Are in Testing to Meet the Needs of Children

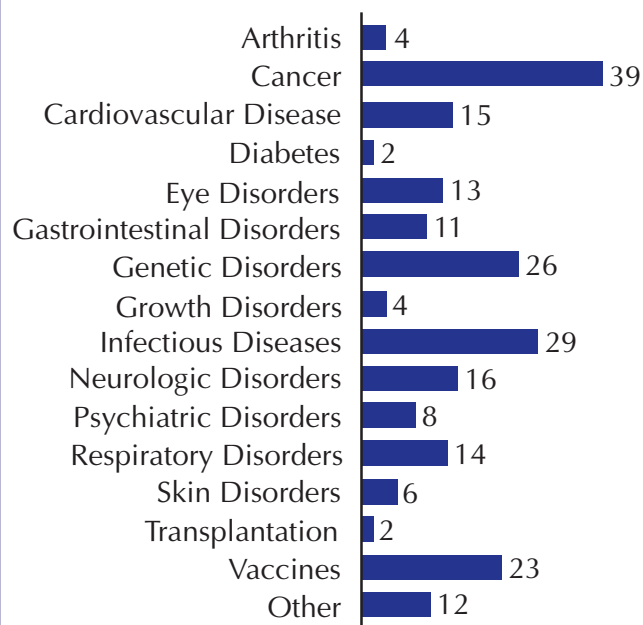
Biopharmaceutical researchers are testing 219 medicines to meet the special health needs of children. These medicines offer hope that the significant improvements achieved in children's health over the past few decades will continue and even accelerate. The potential medicines are either in clinical trials or under review by the Food and Drug Administration (FDA).

A child born today can expect to live 30 years longer than a child born a century ago, according to the National Center for Health Statistics (NCHS). NCHS statistics also show that infant mortality has sunk to record lows. New vaccines protect children against many childhood diseases, including polio, measles, mumps, chicken pox, diphtheria, whooping cough, hepatitis B, and meningitis. Antibiotics prevent deaths from pneumonia, scarlet fever and other diseases that used to claim the lives of children. According to a *New England Journal of Medicine* report, pneumonia deaths among children dropped 97 percent between 1939 and 1996. According to the American Cancer Society, mortality rates from childhood cancer have declined about 49% since 1975. For all childhood cancers combined, five-year relative survival rates have improved markedly over the past 30 years. Biopharmaceutical companies are working to continue this progress and to meet new challenges to children's health, such as heart disease and obesity.

The medicines in the pipeline include:

- 39 for cancer which, despite significant progress, is still the leading cause of death by disease among American children between 5 and 24. These include medicines for leukemia, solid tumors, brain tumors, and lymphomas.
- 29 for such infectious diseases as HIV infection, ear infections, pneumonia, and hepatitis.
- 26 for genetic disorders, including medicines for cystic fibrosis, which affects 30,000 American children and adults.
- 16 for neurologic disorders, including medicines for epilepsy, which affects more than 300,000 school children under age 14 in the United States.
- 15 for cardiovascular disease, including hypertension, high cholesterol, and congenital heart disease.
- 14 for respiratory disorders, including medicines for asthma, the leading serious chronic disease among children, which affects some 6.2 million children.

MEDICINES IN DEVELOPMENT FOR CHILDREN*



*Some medicines are listed in more than one category.

In addition to creating medicines specifically to meet the needs of children, biopharmaceutical companies are testing many existing medicines to determine safe and effective dosage levels for children. On January 4, 2002, President Bush signed the "Best Pharmaceuticals for Children Act (BPCA)," reauthorizing a highly successful program to generate new knowledge about medicines for use in children. As a result of this program, according to the Tufts Center for the Study of Drug Development, more than 120 medicines contain new safety, efficacy, dosing, and risk information for children and teenagers in its labeling. The BPCA must be reauthorized by October 1, 2007.

New medicines, and new knowledge about pediatric use of existing medicines, will mean that children will recover from illnesses more quickly and that more children will enjoy childhood and grow into healthy adults.

Billy Tauzin
President and CEO
PhRMA

Medicines in Development for Children

ARTHRITIS

Product Name	Sponsor	Indication	Development Status ^{†**}
Actemra™ tocilizumab	Chugai <i>Bedminster, NJ</i> Roche <i>Nutley, NJ</i>	systemic juvenile idiopathic arthritis	Phase III completed <i>1 year–19 years</i> (908) 947-2700 (973) 235-5000
Humira® adalimumab	Abbott Laboratories <i>Abbott Park, IL</i>	polyarticular juvenile rheumatoid arthritis (see also gastrointestinal)	Phase III <i>4 years–17 years</i> (847) 937-6100
Orencia™ abatacept	Bristol-Myers Squibb <i>Princeton, NJ</i>	juvenile rheumatoid arthritis	Phase III <i>pediatric patients</i> (212) 546-4000
Remicade® infliximab (Orphan Drug)	Centocor <i>Horsham, PA</i>	juvenile rheumatoid arthritis (see also gastrointestinal)	Phase III completed <i>4 years–18 years</i> (610) 651-6000

CANCER

Product Name	Sponsor	Indication	Development Status
17-AAG	National Cancer Institute <i>Bethesda, MD</i> Kosan Biosciences <i>Hayward, CA</i>	Ewing sarcoma/peripheral primitive neuroectodermal tumor, solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
amolimogene (ZYC101a)	MGI Pharma <i>Bloomington, MN</i>	precancerous cervical dysplasia	Phase II/III <i>13 years–25 years</i> (952) 346-4700
AP23573 (Orphan Drug)	Ariad Pharmaceuticals <i>Cambridge, MA</i>	advanced sarcoma	Phase II <i>15 years and older</i> (617) 494-0400
Avastin® bevacizumab	National Cancer Institute <i>Bethesda, MD</i> Genentech <i>South San Francisco, CA</i>	high-grade astrocytoma	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
AZD2171	National Cancer Institute <i>Bethesda, MD</i> AstraZeneca <i>Wilmington, DE</i>	central nervous system (CNS) tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Azedra™ iobenguane	Molecular Insight Pharmaceuticals <i>Cambridge, MA</i>	neuroblastoma, pheochromocytoma	Phase I <i>18 years and younger</i> (617) 492-5554

† All of the products listed in this report are being tested in children. In most cases, we have identified in the “Development Status” column the ages for which the trials are being conducted.

** For more information about a specific medicine in this report, please call the telephone number listed.

CANCER

Product Name	Sponsor	Indication	Development Status
Camptosar® irinotecan in combination Temodar® temozolomide	Pfizer <i>New York, NY</i>	brain tumors (glioma, medulloblastoma)	Phase II <i>6 months–18 years</i> (860) 732-5156
Dacogen® decitabine	National Cancer Institute <i>Bethesda, MD</i> MGI Pharma <i>Bloomington, MN</i>	leukemia	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Eloxatin® oxaliplatin	National Cancer Institute <i>Bethesda, MD</i> sanofi-aventis <i>Bridgewater, NJ</i>	medulloblastoma, Ewing sarcoma/peripheral primitive neuroectodermal tumor, medulloblastoma	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
		solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
EMD 121974 (cilengitide)	National Cancer Institute <i>Bethesda, MD</i> EMD Pharmaceuticals <i>Durham, NC</i>	CNS primary tumor	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Erbix™ cetuximab	Bristol-Myers Squibb <i>Princeton, NJ</i> Imclone Systems <i>New York, NY</i>	solid tumors	Phase I <i>pediatric patients</i> (212) 546-4000 (908) 218-9588
Gleevec® imatinib	National Cancer Institute <i>Bethesda, MD</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	acute lymphoblastic leukemia (ALL)	in clinical trials NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Herceptin® trastuzumab	National Cancer Institute <i>Bethesda, MD</i> Genentech <i>South San Francisco, CA</i>	osteosarcoma	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
homoharringtonine	ChemGenex Therapeutics <i>Menlo Park, CA</i>	refractory acute promyelocytic leukemia	Phase I/II <i>12 years and older</i> (650) 474-9800
Hycamtin® topotecan	National Cancer Institute <i>Bethesda, MD</i> GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	leptomeningeal metastases	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
IL13-PE38QQR (cintredekin besudotox)	Neopharm <i>Waukegan, IL</i>	recurrent malignant glioma	Phase I/II <i>3 years–21 years</i> (847) 887-0800
interleukin-12	National Cancer Institute <i>Bethesda, MD</i> Wyeth <i>Collegeville, PA</i>	neuroblastoma	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER

CANCER

Product Name	Sponsor	Indication	Development Status
iratutumab (MDX-060) (Orphan Drug)	Medarex <i>Princeton, NJ</i>	anaplastic large-cell lymphoma	Phase II <i>12 years and older</i> (609) 430-2880
ispinesib	National Cancer Institute <i>Bethesda, MD</i> Cytokinetics <i>South San Francisco, CA</i>	solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
karenitecin (BNP1350)	BioNumerik Pharmaceuticals <i>San Antonio, TX</i>	solid tumors	Phase I <i>pediatric patients</i> (210) 614-1701
L-annamycin	Callisto Pharmaceuticals <i>New York, NY</i>	ALL, acute myeloid leukemia (AML)	Phase I <i>12 months–21 years</i> (212) 297-0010
Nexavar® sorafenib tosylate	National Cancer Institute <i>Bethesda, MD</i> Bayer Corporation <i>West Haven, CT</i>	solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
O-6-benzylguanine	National Cancer Institute <i>Bethesda, MD</i> Keryx Biopharmaceuticals <i>Memphis, TN</i>	anaplastic astrocytoma	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
		----- CNS primary tumor	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
perifosine	Keryx Biopharmaceuticals <i>New York, NY</i>	chemotherapy-insensitive sarcomas	Phase II <i>13 years and older</i> (212) 531-5965
Revlimid™ lenalidomide	National Cancer Institute <i>Bethesda, MD</i> Celgene <i>Summit, NJ</i>	CNS primary tumor, solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Rituxan® rituximab	National Cancer Institute <i>Bethesda, MD</i> Biogen Idec <i>Cambridge, MA</i>	non-Hodgkin lymphoma	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
romidepsin	National Cancer Institute <i>Bethesda, MD</i> Gloucester Pharmaceuticals <i>Cambridge, MA</i>	solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
SGN-30	National Cancer Institute <i>Bethesda, MD</i> Seattle Genetics <i>Bothell, WA</i>	anaplastic large-cell lymphoma, primary systemic type	Phase I/II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER

CANCER

Product Name	Sponsor	Indication	Development Status
SLIT™ Cisplatin (inhalation)	Transave <i>Monmouth Junction, NJ</i>	osteosarcoma metastatic to the lung	Phase I/II 13 years and older (732) 438-9434
Sprycel™ dasatinib (Orphan Drug)	Bristol-Myers Squibb <i>Princeton, NJ</i>	refractory chronic myeloid leukemia (CML)	Phase II <i>pediatric patients</i> (212) 546-4000
Sutent® sunitinib malate	National Cancer Institute <i>Bethesda, MD</i> Pfizer <i>New York, NY</i>	solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Taxoprexin® DHA-paclitaxel	Luitpold Pharmaceuticals <i>Shirley, NY</i>	advanced eye melanoma	Phase II 13 years and older (631) 924-1731
Taxotere® docetaxel	sanofi-aventis <i>Bridgewater, NJ</i>	solid tumors	Phase III <i>pediatric patients</i> (800) 633-1610
Tykerb® lapatinib	National Cancer Institute <i>Bethesda, MD</i> GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, IL</i>	CNS primary tumor	Phase I/II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Velcade® bortezomib	National Cancer Institute <i>Bethesda, MD</i> Millennium Pharmaceuticals <i>Cambridge, MA</i>	Hodgkin lymphoma	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
		ALL	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Voraxaze™ glucarpidase (Orphan Drug)	National Cancer Institute <i>Bethesda, MD</i> Protherics <i>Brentwood, TN</i>	solid tumors	in clinical trials NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Xcytrin® motexafin gadolinium	National Cancer Institute <i>Bethesda, MD</i> Pharmacyclics <i>Sunnyvale, CA</i>	diffuse brainstem glioma	Phase II NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Zevalin™ ibritumomab tiuxetan	National Cancer Institute <i>Bethesda, MD</i> Biogen IDEC <i>Cambridge, MA</i>	non-Hodgkin lymphoma	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER
Zolinza™ vorinostat	National Cancer Institute <i>Bethesda, MD</i> Merck <i>Whitehouse Station, NJ</i>	solid tumors	Phase I NCI TRIAL <i>pediatric patients</i> (800) 4-CANCER

CARDIOVASCULAR DISEASE

Product Name	Sponsor	Indication	Development Status
Atacand® candesartan cilexetil	AstraZeneca <i>Wilmington, DE</i>	hypertension	Phase III completed 6 years–17 years (800) 236-9933
		hypertension	Phase III 1 year–6 years (800) 236-9933
Benicar® olmesartan medoxomil	Daiichi Sankyo <i>Parsippany, NJ</i>	hypertension	Phase II/III 1 year–16 years (973) 359-2600
Diovan® valsartan	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	high blood pressure	Phase III 1 year–5 years (888) NOW-NOVA
Inspra® eplerenone	Pfizer <i>New York, NY</i>	hypertension	Phase III 6 years–16 years (860) 732-5156
Plavix® clopidogrel	Bristol-Myers Squibb <i>Princeton, NJ</i> sanofi-aventis <i>Bridgewater, NJ</i>	atherosclerosis, thrombosis	Phase III <i>pediatric patients</i> (212) 546-4000 (800) 633-1610
ramipril	King Pharmaceuticals <i>Bristol, TN</i>	hypertension	Phase III 6 years–16 years (800) 776-3637
Remodulin® treprostinil (sustained-release tablets)	United Therapeutics <i>Silver Spring, MD</i>	pulmonary arterial hypertension	Phase III 12 years and older (301) 608-9292
Revatio® sildenafil citrate	Pfizer <i>New York, NY</i>	pulmonary arterial hypertension	Phase II/III 1 year–17 years (860) 732-5156
tadalafil	Eli Lilly <i>Indianapolis, IN</i>	pulmonary arterial hypertension	Phase III 12 years and older (317) 276-2000
TAK-475	Takeda Pharmaceuticals <i>Deerfield, IL</i>	homozygous familial hypercholesterolemia	Phase III 12 years and older (224) 554-6500
Thelin™ sitaxsentan sodium	Encysive Pharmaceuticals <i>Houston, TX</i>	pulmonary arterial hypertension	Phase III 12 years and older (713) 796-8802
Tracleer® bosentan (Orphan Drug)	Actelion Pharmaceuticals USA <i>South San Francisco, CA</i>	pulmonary arterial hypertension	Phase III 12 years and older (650) 624-6900
Tracleer® bosentan (pediatric formulation) (Orphan Drug)	Actelion Pharmaceuticals USA <i>South San Francisco, CA</i>	pulmonary arterial hypertension	Phase III 2 years–12 years (650) 624-6900

CARDIOVASCULAR DISEASE

Product Name	Sponsor	Indication	Development Status
Ventavis® iloprost inhalation solution (Orphan Drug)	Actelion Pharmaceuticals USA South San Francisco, CA	pulmonary arterial hypertension	Phase III 12 years and older (650) 624-6900
WelChol® colesevelam HCl	Daiichi Sankyo US Parsippany, NJ	hypercholesterolemia (heterozygous familial)	Phase IV 10 years–17 years (973) 359-2600

DIABETES

Product Name	Sponsor	Indication	Development Status
Apidra® insulin glulisine	sanofi-aventis Bridgewater, NJ	type 1 diabetes mellitus	Phase III 4 years–17 years (800) 633-1610
teplizumab (MGA031) (Orphan Drug)	MacroGenics Rockville, MD	type 1 diabetes mellitus	Phase II/III 8 years and older (301) 251-5172

EYE DISORDERS

Product Name	Sponsor	Indication	Development Status
AL-15469A (quinolone)	Alcon Research Fort Worth, TX	bacterial conjunctivitis	Phase III 1 month and older (800) 862-5266
alcaftadine (ophthalmic solution) (R89674)	Vistakon Pharmaceuticals Raritan, NJ	allergic conjunctivitis	Phase III 10 years and older
bepotastine besilate	ISTA Pharmaceuticals Irvine, CA	allergic conjunctivitis	Phase II 10 years and older (949) 788-6000
Betoptic S betaxolol HCl ophthalmic suspension, 0.25%	Alcon Research Fort Worth, TX	intraocular pressure (IOP)- lowering in glaucoma and ocular hypertension	Phase III birth–5 years (800) 862-5266
BOL-303224-A	Bausch & Lomb Rochester, NY	bacterial conjunctivitis	Phase III 1 year and older (585) 338-6000
Cystoran™ cysteamine hydrochloride (Orphan Drug)	Sigma-Tau Pharmaceuticals Gaithersburg, MD	corneal cystine crystal accumulation in cystinosis patients	Phase III newborn–18 years (800) 447-0169
difluprednate ophthalmic (ST-601)	Sirion Therapeutics Tampa, FL	inflammation following ocular surgery	Phase III 2 years and older (813) 496-7325

EYE DISORDERS

Product Name	Sponsor	Indication	Development Status
LX211 (voclosporin) (Orphan Drug)	Lux Biosciences <i>Jersey City, NJ</i>	uveitis	Phase III <i>13 years and older</i> (201) 946-0551
Patanol® Plus olopatadine 0.2%	Alcon Research <i>Fort Worth, TX</i>	allergic conjunctivitis	Phase III <i>10 years and older</i> (800) 862-5266
Timolol Gel Forming Solution timolol maleate 0.25% ophthalmic solution	Alcon Research <i>Fort Worth, TX</i>	IOP-lowering in open-angle glaucoma or ocular hypertension	Phase III <i>birth–5 years</i> (800) 862-5266
Timolol Gel Forming Solution timolol maleate 0.5% ophthalmic solution	Alcon Research <i>Fort Worth, TX</i>	IOP-lowering in open-angle glaucoma or ocular hypertension	Phase III <i>birth–5 years</i> (800) 862-5266
Xibrom™ bromfenac ophthalmic solution	ISTA Pharmaceuticals <i>Irvine, CA</i>	allergic conjunctivitis	Phase III <i>10 years and older</i> (949) 788-6000
Zylet™ loteprednol etabonate 0.5%/ tobramycin 0.3% ophthalmic solution	Bausch & Lomb <i>Rochester, NY</i>	chalazion hordeolum (eye lid inflammation)	Phase IV <i>up to 6 years</i> (585) 338-6000

GASTROINTESTINAL DISORDERS

Product Name	Sponsor	Indication	Development Status
Aciphex® rabeprazole sodium	Eisai <i>Ridgefield Park, NJ</i>	gastroesophageal reflux disease (GERD)	Phase II <i>12 years–16 years</i> (888) 274-2378
Axid® nizatidine	Braintree Laboratories <i>Braintree, MA</i>	GERD	Phase III <i>up to 1 year</i> (800) 874-6756
Cimizia™ certolizumab pegol	UCB <i>Smyrna, GA</i>	Crohn's disease	Phase III <i>up to 18 years</i> (770) 970-7500
Humira® adalimumab	Abbott Laboratories <i>Abbott Park, IL</i>	moderate to severe Crohn's disease (see also arthritis)	Phase III <i>6 years–17 years</i> (847) 937-6100
mepolizumab	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	eosinophilic esophagitis	Phase I/II <i>2 years–17 years</i> (888) 825-5249
Nexium® esomeprazole	AstraZeneca <i>Wilmington, DE</i>	GERD	Phase III <i>1 year–11 years</i> (800) 236-9933

GASTROINTESTINAL DISORDERS

Product Name	Sponsor	Indication	Development Status
Nuvion [®] visilizumab	PDL BioPharma <i>Fremont, CA</i>	ulcerative colitis	Phase I/II <i>16 years and older</i> (510) 574-1400
Oralgam [™] human immunoglobulin	PediaMed Pharmaceuticals <i>Florence, KY</i>	intestinal problems associated with autism	Phase II <i>2 years–18 years</i> (866) KID-MEDS
Prevacid [®] lansoprazole (microgranules, oral suspension)	TAP Pharmaceutical Products <i>Lake Forest, IL</i>	GERD	Phase III <i>1 month–11 months</i> (847) 582-2000
Protonix [®] pantoprazole	Wyeth <i>Collegeville, PA</i>	GERD	Phase III <i>up to 11 months</i> (610) 902-1200
		GERD	Phase III <i>1 year–16 years</i> (610) 902-1200
Remicade [®] infliximab	Centocor <i>Horsham, PA</i>	ulcerative colitis (see also arthritis)	Phase III <i>6 years–17 years</i> (610) 651-6000

GENETIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Actonel [®] risedronate sodium	Procter & Gamble Pharmaceuticals <i>Cincinnati, OH</i> sanofi-aventis <i>Bridgewater, NJ</i>	osteogenesis imperfecta	Phase III <i>4 years–15 years</i> (513) 983-1100 (800) 633-1610
ALTU-135	Altus Pharmaceuticals <i>Cambridge, MA</i>	cystic fibrosis patients with exocrine pancreatic insufficiency	Phase III <i>7 years and older</i> (800) 258-2532
aztreonam lysine for inhalation	Gilead Sciences <i>Foster City, CA</i> <i>Seattle, WA</i>	treatment of <i>Pseudomonas</i> <i>aeruginosa</i> in cystic fibrosis patients	Phase III <i>6 years and older</i> (800) GILEAD-5
C1 esterase inhibitor	CSL Behring <i>King of Prussia, PA</i>	hereditary angioedema	Phase II/III <i>6 years and older</i> (610) 878-4000
C1 esterase inhibitor (human)	Lev Pharmaceuticals <i>New York, NY</i>	hereditary angioedema	Phase III <i>1 year and older</i> (212) 682-3994
denufosal tetrasodium inhalation solution (INS 37217)	Inspire Pharmaceuticals <i>Durham, NC</i>	cystic fibrosis	Phase III <i>5 years and older</i> (919) 941-9777
DNA nanoparticle gene therapy	Copernicus Therapeutics <i>Cleveland, OH</i>	cystic fibrosis	Phase I (216) 231-0227

GENETIC DISORDERS

Product Name	Sponsor	Indication	Development Status
EUR 1008M (PEP capsules)	Eurand <i>Vandalia, OH</i>	exocrine pancreatic insufficiency in cystic fibrosis	Phase III <i>7 years and older</i> (973) 898-9669
Faslodex [®] fulvestrant	AstraZeneca <i>Wilmington, DE</i>	McCune-Albright syndrome (girls)	Phase II <i>up to 10 years</i> (800) 236-9933
IPLEX [™] mecaserum rinfubate (rhIGF-I/rhIGFBP-3)	Insmed <i>Richmond, VA</i>	Noonan syndrome	Phase II <i>2 years–16 years</i> (804) 565-3000
Lomucin [™] talniflumate	Genaera <i>Plymouth Meeting, PA</i>	cystic fibrosis	Phase II <i>12 years and older</i> (610) 941-4020
Lym-X-Sorb [™]	BioMolecular Products <i>Byfield, MA</i>	cystic fibrosis patients with pancreatic insufficiency	Phase II <i>6 years–17 years</i> (978) 462-2224
MOLI 1901	Lantibio <i>Chapel Hill, NC</i>	cystic fibrosis	Phase II <i>12 years and older</i> (919) 960-0217
OBI-1 (recombinant factor VIII)	Ipsen <i>Milford, MA</i> Octagen <i>Blue Bell, PA</i>	hemophilia A	Phase II <i>12 years and older</i> (215) 840-0505
Pancrecarb [®] MS-16 pancrelipase	Digestive Care <i>Bethlehem, PA</i>	cystic fibrosis patients with pancreatic insufficiency	Phase III <i>7 years and older</i> (610) 882-5950
Phenoptin [™] sapropterin dihydrochloride	BioMarin Pharmaceutical <i>Novato, CA</i>	phenylketonuria (PKU)	Phase III <i>4 years and older</i> (415) 506-6700
plasma-derived AT-III concentrate	Grifols <i>Los Angeles, CA</i>	antithrombin III deficiency	Phase II/III <i>12 years and older</i> (888) 474-3657
recombinant human C1 inhibitor	Pharming Technologies <i>Leiden, The Netherlands</i>	hereditary angioedema	Phase II <i>12 years and older</i> www.pharming.com
senicapoc (ICA-17043)	Icagen <i>Durham, NC</i>	sickle cell disease	Phase III <i>16 years and older</i> (919) 941-5206
SPI-8811	Sucampo Pharmaceuticals <i>Bethesda, MD</i>	cystic fibrosis	Phase II (301) 961-3400
thymosin beta 4 (TB4) (Orphan Drug)	RegeneRx Biopharmaceuticals <i>Bethesda, MD</i>	epidermolysis bullosa	Phase II <i>2 years and older</i> (301) 280-1992
TOBI [®] tobramycin inhalation powder	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	cystic fibrosis	Phase III <i>6 years and older</i> (888) NOW-NOVA

GENETIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Ubiquinol coenzyme 10 ubiquinol (Orphan Drug)	Tishcon Westbury, NY	mitochondrial cytopathies	Phase III 12 months–17 years (516) 333-3050
ULTRASE MT 20	Axcan Pharma Birmingham, AL	exocrine pancreatic insufficiency in cystic fibrosis patients	Phase III 7 years and older (800) 472-2634
Zithromax® azithromycin	Pfizer New York, NY Cystic Fibrosis Foundation Therapeutics Bethesda, MD	cystic fibrosis	Phase III 6 years–18 years (860) 732-5156 (800) 344-4823
Zometa® zoledronic acid	Novartis Pharmaceuticals East Hanover, NJ	osteogenesis imperfecta	Phase III 1 year–17 years (888) NOW-NOVA

GROWTH DISORDERS

Product Name	Sponsor	Indication	Development Status
Caslodex® bicalutamide Arimidex® anastrozole combination	AstraZeneca Wilmington, DE	testotoxicosis (boys)	Phase II 2 years and older (800) 236-9933
Genotropin® somatotropin	Pfizer New York, NY	treatment of short, very low birth weight children born appropriate for gestational age	Phase II 4 years–10 years (860) 732-5156
PHA-794428 (pegylated recombinant human growth hormone)	Pfizer New York, NY	growth hormone deficiency	Phase II 6 years–12 years (860) 732-5156
somatropin growth hormone (sustained-release)	LG Life Sciences Seoul, South Korea	insufficient secretion of growth hormone	Phase III 3 years–11 years

INFECTIOUS DISEASES

Product Name	Sponsor	Indication	Development Status
Altastaph™ hyperimmune globulin	Nabi Biopharmaceuticals Boca Raton, FL Rockville, MD	prevention and/or treatment of <i>Staphylococcus aureus</i>	Phase II neonates (301) 255-6919
Aptivus® tipranavir	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	treatment of HIV infection in highly treatment-experienced patients	Phase II 2 years–18 years (800) 243-0127
Baraclude® entecavir	Bristol-Myers Squibb Princeton, NJ	chronic hepatitis B	Phase II pediatric patients (212) 546-4000

INFECTIOUS DISEASES

Product Name	Sponsor	Indication	Development Status
Famvir® famciclovir (pediatric formulation)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	herpes simplex infection, varicella zoster infection	Phase III 1 year–18 years (888) NOW-NOVA
faropenem medoxomil	Replidyne <i>Louisville, CO</i>	acute otitis media	Phase II completed 6 months–7 years (303) 996-5500
FluBIØk™ trivalent recombinant hemagglutinin vaccine	Protein Sciences <i>Meriden, CT</i>	prevention of influenza	Phase II 6 months–59 months (203) 686-0800
Hepsera™ adefovir dipivoxil	Gilead Sciences <i>Foster City, CA</i>	chronic hepatitis B	Phase III 2 years–17 years (800) GILEAD-5
Ketek® telithromycin	sanofi-aventis <i>Bridgewater, NJ</i>	acute otitis media, community- acquired pneumonia, tonsillitis/pharyngitis	Phase III 6 months and older (800) 981-2491
Levaquin® levofloxacin	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	community-acquired pneumonia	Phase III <i>pediatric patients</i> (800) 817-5286
Lexiva™ fosamprenavir	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	HIV infection	Phase II 2 years–18 years (888) 825-5249
maraviroc	Pfizer <i>New York, NY</i>	HIV infection	Phase III 16 years and older (860) 732-5156
NatrOVA Creme Rinse	ParaPro Pharmaceuticals SePRO <i>Carmel, IN</i>	head lice	Phase I 6 months–24 months (800) 419-7779
ominganan pentachloride 1% gel	Cadence Pharmaceuticals <i>San Diego, CA</i>	bloodstream infections in patients undergoing central venous catheterization	Phase III 13 years and older (858) 436-1400
Ovide® malathion (novel formulation)	Taro Pharmaceuticals USA <i>Hawthorne, NY</i>	head lice	Phase III 2 years and older (800) 544-1449
		head lice	Phase II 6 months–24 months (800) 544-1449
PAR-101 (difimicin)	Optimer Pharmaceuticals <i>San Diego, CA</i>	<i>Clostridium difficile</i> -associated diarrhea	Phase II/III 16 years and older (858) 909-0736
PEG-Intron® plus Rebetol	Schering-Plough <i>Kenilworth, NJ</i>	chronic hepatitis C	Phase III 3 years–17 years (908) 298-4000

INFECTIOUS DISEASES

Product Name	Sponsor	Indication	Development Status
pleconaril nasal spray	Schering-Plough Kenilworth, NJ	picornavirus respiratory infections	Phase II 6 years and older (908) 298-4000
Prezista™ darunavir	Tibotec Yardley, PA	HIV infection	Phase II 6 years–17 years (609) 730-7500
REP8839	Replidyne Louisville, CO	skin infections, impetigo including MRSA	Phase I (303) 996-5500
Reyataz® atazanavir	Bristol-Myers Squibb Princeton, NJ	HIV infection	Phase II <i>pediatric patients</i> (212) 546-4000
SP01A	Samaritan Pharmaceuticals Las Vegas, NV	HIV infection (see also other)	Phase II/III (702) 735-7001
Summers 5% LA	Summers Laboratories Collegeville, PA	head lice	Phase III 6 months and older (800) 533-SKIN
Sustiva® efavirenz	Bristol-Myers Squibb Princeton, NJ	HIV infection	Phase II <i>pediatric patients</i> (212) 546-4000
Valcyte® valganciclovir	Roche Nutley, NJ	cytomegalovirus (CMV) disease in kidney allograft patients	Phase III 12 years and older (973) 235-5000
Valcyte® valganciclovir (syrup)	Roche Nutley, NJ	CMV disease in solid organ transplant patients	Phase III 3 months–16 years (973) 235-5000
Valtrex® valaciclovir oral suspension	GlaxoSmithKline Philadelphia, PA Rsch. Triangle Park, NC	herpes simplex	Phase I 1 month–6 years (800) 825-5249
Venticute™ lusupultide	Altana Pharma US Florham Park, NJ	pneumonia	Phase III 12 years and older (973) 514-4240
Vfend® voriconazole	Pfizer New York, NY	prevention of fungal infections	Phase III 2 years and older (860) 732-5156
Zmax™ azithromycinSR extended-release for oral suspension	Pfizer New York, NY	acute bronchitis, secondary infection of chronic respiratory diseases, acne vulgaris, laryngopharyngitis, tonsillitis, acute bacterial rhinosinusitis	Phase III 16 years and older (860) 732-5156

NEUROLOGIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Axert® almotriptan	Johnson & Johnson Pharmaceutical Research & Development Raritan, NJ	migraine	Phase III 12 years–17 years (800) 817-5286

NEUROLOGIC DISORDERS

Product Name	Sponsor	Indication	Development Status
brivaracetam (ucb 34714)	UCB <i>Smyrna, GA</i>	Unverricht-Lundborg disease	Phase III <i>16 years and older</i> (770) 970-7500
		partial-onset seizures	Phase III <i>16 years and older</i> (770) 970-7500
carisbamate	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	partial-onset seizures	Phase III <i>16 years and older</i> (800) 817-5286
clobazam	Ovation Pharmaceuticals <i>Deerfield, IL</i>	Lennox-Gastaut syndrome	Phase II <i>2 years and older</i> (847) 282-1000
Flomax [®] tamsulosin hydrochloride	Boehringer Ingelheim <i>Ridgefield, CT</i>	neurogenic bladder secondary to spinal cord injury or defect	Phase II/III <i>2 years–16 years</i> (203) 798-9988
ganaxolone	Marinus Pharmaceuticals <i>Branford, CT</i>	infantile spasms	Phase II <i>4 months–24 months</i> (203) 315-0566
HuCNS-SC	StemCells <i>Palo Alto, CA</i>	infantile or late infantile neuronal ceroid lipofuscinosis	Phase I <i>18 months–12 years</i> (650) 475-3100
IV APAP (intravenous acetaminophen)	Cadence Pharmaceuticals <i>San Diego, CA</i>	treatment of acute pain and fever	Phase III <i>birth–adolescents</i>
Keppra [®] levetiracetam	UCB <i>Smyrna, GA</i>	partial-onset seizures	Phase III <i>1 month–4 years</i> (770) 970-7500
Lyrica [®] pregabalin	Pfizer <i>New York, NY</i>	partial-onset seizures	Phase I/II <i>1 month–16 years</i> (860) 732-5156
MultiHance [®] gadobenate dimeglumine	Bracco Diagnostics <i>Princeton, NJ</i>	MRI contrast agent in central nervous system disorders	Phase III <i>2 years–17 years</i> (800) 631-5245
oral glycopyrrolate liquid	Sciele Pharma <i>Atlanta, GA</i>	drooling associated with cerebral palsy and other neurologic conditions in children	Phase II/III <i>3 years–16 years</i> (800) 461-3696
PTC124	PTC Therapeutics <i>South Plainfield, NJ</i>	Duchenne muscular dystrophy	Phase II <i>5 years and older</i> (908) 222-7000
rufinamide	Eisai <i>Ridgefield Park, NJ</i>	adjunctive treatment for seizures associated with Lennox-Gastaut syndrome (LGS)	application submitted <i>4 years and older</i> (888) 274-2378
		adjunctive treatment for partial-onset seizures	application submitted <i>12 years and older</i> (888) 274-2378

NEUROLOGIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Sabril® vigabatrin (Orphan Drug)	Ovation Pharmaceuticals <i>Deerfield, IL</i>	infantile spasms	application submitted <i>birth–2 years</i> (847) 282-1000
Topamax® topiramate	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	partial-onset seizures	Phase III <i>1 month–24 months</i> (800) 817-5286
		migraine prevention	Phase III <i>12 years–17 years</i> (800) 817-5286
Uroxatral® alfuzosin	sanofi-aventis <i>Bridgewater, NJ</i>	voiding dysfunction	Phase I <i>pediatric patients</i> (800) 633-1610

PSYCHIATRIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Abilify® aripiprazole	Bristol-Myers Squibb <i>Princeton, NJ</i> Otsuka America Pharmaceutical <i>Rockville, MD</i>	autism	Phase III <i>6 years–17 years</i> (212) 546-4000 (301) 990-0030
Equetro™ carbamazepine (extended-release)	Shire Pharmaceutical <i>Wayne, PA</i>	bipolar I disorder	Phase IV <i>10 years–17 years</i> (484) 595-8800
Geodon® ziprasidone oral capsules	Pfizer <i>New York, NY</i>	schizophrenia	Phase III <i>13 years–17 years</i> (860) 732-5156
		bipolar disorder (manic or mixed)	Phase III <i>10 years–17 years</i> (860) 732-5156
Lexapro™ escitalopram	Forest Laboratories <i>New York, NY</i>	major depressive disorder	Phase III <i>12 years–17 years</i> (800) 947-5227
Risperdal® risperidone	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	bipolar disorder	Phase III <i>pediatrics/adolescents</i> (800) 817-5286
		schizophrenia	Phase III <i>adolescents</i> (800) 817-5286
Seroquel® quetiapine fumarate	AstraZeneca <i>Wilmington, DE</i>	bipolar mania 1	Phase III <i>10 years–17 years</i> (800) 236-9933
		schizophrenia	Phase III <i>13 years–17 years</i> (800) 236-9933

PSYCHIATRIC DISORDERS

Product Name	Sponsor	Indication	Development Status
SPD 503 (guanfacine HCl extended-release)	Shire Pharmaceutical Wayne, PA	attention-deficit/hyperactivity disorder (ADHD)	application submitted 6 years–17 years (484) 595-8800
		ADHD with oppositional symptoms	Phase III 6 years–12 years (484) 595-8800
Zoloft® sertraline	Pfizer New York, NY	post-traumatic stress disorder	Phase III 6 years–17 years (860) 732-5156

RESPIRATORY DISORDERS

Product Name	Sponsor	Indication	Development Status
Aerosurf™ sinapultide (aerosolized)	Discovery Laboratories Warrington, PA	respiratory distress syndrome	Phase II (215) 488-9300
Allermist™ fluticasone furoate nasal spray	GlaxoSmithKline Philadelphia, PA Rsch. Triangle Park, NC	perennial and seasonal allergic rhinitis	application submitted 2 years and older (888) 825-5249
Alvesco® ciclesonide (inhaled)	sanofi-aventis Bridgewater, NJ	asthma	application submitted 4 years and older (800) 633-1610
Aridol™ mannitol inhalation diagnostic	Pharmaxis Frenchs Forest, Australia	detection of bronchial hyperresponsiveness in patients with asthma	Phase III 6 years and older www.pharmaxis.com.au
epinastine nasal spray	Inspire Pharmaceuticals Durham, NC	allergic rhinitis	Phase II 12 years and older (919) 941-9777
HAE1 (PRO98498)	Genentech South San Francisco, CA	allergic asthma	Phase II 12 years and older (650) 225-1000
INOmax nitric oxide for inhalation	INO Therapeutics Clinton, NJ	hypoxic respiratory failure associated with pulmonary hypertension	Phase III premature infants (908) 238-6600
Nasonex® mometasone furoate nasal spray	Schering-Plough Kenilworth, NJ	nasal polyps	Phase III 6 years–17 years (908) 298-4000
Numax™ motavizumab	MedImmune Gaithersburg, MD	respiratory syncytial virus (RSV)	Phase II/III up to 12 months (301) 398-0000
Surfaxin® lucinactant (Orphan Drug)	Discovery Laboratories Warrington, PA	neonatal respiratory distress syndrome	application submitted premature infants (215) 488-9300

RESPIRATORY DISORDERS

Product Name	Sponsor	Indication	Development Status
Symbicort® budesonide/ formoterol	AstraZeneca <i>Wilmington, DE</i>	asthma	application submitted 6 years–11 years (800) 236-9933
uteroglobin (recombinant human Clara cell 10kDa protein) (Orphan Drug)	Claragen <i>College Park, MD</i> CC10 Sweden <i>Stockholm, Sweden</i>	prevention of neonatal bronchopulmonary dysplasia in premature neonates with respiratory distress syndrome	Phase I <i>neonates</i> (301) 452-2899
Xolair® omalizumab	Genentech <i>South San Francisco, CA</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	moderate to severe allergic asthma (see also other)	Phase III 6 years–12 years (650) 225-1000 (888) NOW-NOVA
Zyflo® CR zileuton controlled-release	Critical Therapeutics <i>Lexington, MA</i>	prevention and chronic treatment of asthma	application submitted 12 years and older (781) 402-5700

SKIN DISORDERS

Product Name	Sponsor	Indication	Development Status
calcitrol	Galderma Laboratories <i>Fort Worth, TX</i>	plaque psoriasis	Phase II 12 years–17 years (817) 961-5000
Elidel® pimecrolimus cream	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	atopic dermatitis	Phase III 3 months–12 months (888) NOW-NOVA
Enbrel® etanercept	Amgen <i>Thousand Oaks, CA</i>	plaque psoriasis	Phase III 4 years–17 years (805) 447-1000
Helioblock® SX Cream	L'Oreal USA <i>New York, NY</i>	sunburn	Phase III 6 months–12 years (212) 818-1500
Lamisil® terbinafine HCl	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	tinea capitis	Phase III completed 4 years–12 years (888) NOW-NOVA
rhThrombin (spray formulation)	ZymoGenetics <i>Seattle, WA</i>	control bleeding during skin graft surgery	Phase II 2 years and older (206) 442-6600

TRANSPLANTATION

Product Name	Sponsor	Indication	Development Status
Certican® everolimus	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	de novo renal transplantation	Phase III 16 years and younger (888) NOW-NOVA
Prochymal™ adult mesenchymal stem cells (Orphan Drug)	Osiris Therapeutics <i>Baltimore, MD</i>	graft versus host disease	Phase III 6 months and older (410) 522-5005

VACCINES

Product Name	Sponsor	Indication	Development Status
13-valent pneumococcal conjugate vaccine	Wyeth <i>Collegeville, PA</i>	pneumococcal infections	Phase III 2 months–2 years (610) 902-1200
Cervarix® HPV 16/18 VLP AS04 vaccine	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	human papillomavirus (HPV) (prevention of cervical cancer)	Phase III completed 15 years–25 years (888) 825-5249
Daptacel®	sanofi pasteur <i>Swiftwater, PA</i>	diphtheria, tetanus, pertussis (DTP) (5th dose)	Phase III 4 years–6 years (570) 839-7187
Flu Infants	sanofi pasteur <i>Swiftwater, PA</i>	influenza	Phase III 6 weeks–6 months (570) 839-7187
FluMist® (refrigerated formulation, CAIV-T)	MedImmune <i>Gaithersburg, MD</i>	influenza prevention	application submitted 12 months–59 months (301) 398-0000
Globorix (conjugated vaccine)	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	DTP, hepatitis B, <i>Haemophilus influenzae</i> type B disease, <i>Neisseria meningitis</i> groups A and C disease prophylaxis	Phase III <i>pediatric patients</i> (800) 825-5249
Hib-MenCY-TT (conjugated vaccine)	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	<i>Neisseria meningitis</i> C and Y disease and <i>Haemophilus influenzae</i> type B disease prophylaxis	Phase III <i>pediatric patients</i> (800) 825-5249
Infanrix®-IPV subunit inactivated vaccine	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	DTP, poliomyelitis prophylaxis (booster 5th dose)	Phase III <i>pediatric patients</i> (888) 825-5249
influenza vaccine	Novartis Vaccines <i>East Hanover, NJ</i>	prevention of influenza	Phase II 3 years–10 years (888) NOW-NOVA
Menactra™	sanofi pasteur <i>Swiftwater, PA</i>	meningitis	application submitted 2 years–10 years (570) 839-7187
Menactra™ Toddler	sanofi pasteur <i>Swiftwater, PA</i>	meningitis	application submitted 1 year–2 years (570) 839-7187
MenACWY-TT (conjugated vaccine)	GlaxoSmithKline <i>Philadelphia, PA</i> <i>Rsch. Triangle Park, NC</i>	<i>Neisseria meningitis</i> groups A, C, W and Y disease prophylaxis	Phase II <i>pediatric patients</i> (800) 825-5249
Meninge B	sanofi pasteur <i>Swiftwater, PA</i>	meningitis B	Phase I <i>infants</i> (570) 839-7187
Meninge A,C,Y,W Infant	sanofi pasteur <i>Swiftwater, PA</i>	meningitis	Phase I <i>infants</i> (570) 839-7187
meningococcal conjugate ACWY vaccine	Novartis Vaccines <i>East Hanover, NJ</i>	prevention of meningococcal disease	Phase III 2 years–10 years (888) NOW-NOVA

VACCINES

Product Name	Sponsor	Indication	Development Status
Pentacel™	sanofi pasteur Swiftwater, PA	DTP, polio, <i>H. influenzae</i> B	application submitted (570) 839-7187
Pneumo	sanofi pasteur Swiftwater, PA	meningitis and pneumonia	Phase I <i>infants</i> (570) 839-7187
Priorix-Tetra live attenuated vaccine	GlaxoSmithKline Philadelphia, PA Rsch. Triangle Park, NC	measles, mumps, rubella and varicella prophylaxis	in clinical trials <i>pediatric patients</i> (888) 825-5249
Rotarix® live attenuated oral vaccine	AVANT Immunotherapeutics Needham, MA GlaxoSmithKline Philadelphia, PA Rsch. Triangle Park, NC	rotavirus infections	Phase II <i>pediatric patients</i> (888) 825-5249
RSV/PIV-3 vaccine	MedImmune Gaithersburg, MD	respiratory syncytial virus (RSV) and parainfluenza virus type 3 (PIV3)	Phase I <i>1 year–9 years</i> (301) 398-0000
serogroup B meningococcal vaccine	Novartis Vaccines East Hanover, NJ	prevention of meningococcal disease	Phase II <i>11 years–18 years</i> (888) NOW-NOVA
Simplirix™ herpes simplex vaccine	GlaxoSmithKline Philadelphia, PA Rsch. Triangle Park, NC	herpes simplex virus	Phase III <i>10 years–17 years</i> (888) 825-5249
Synflorix (conjugated vaccine)	GlaxoSmithKline Philadelphia, PA Rsch. Triangle Park, NC	<i>S. pneumoniae</i> disease and non-typeable <i>Haemophilus</i> <i>influenzae</i> disease prophylaxis	Phase III <i>pediatric patients</i> (800) 825-5249

OTHER

Product Name	Sponsor	Indication	Development Status
Amelior® (injectable)	Cumberland Pharmaceuticals Nashville, TN	fever	Phase III <i>6 months–17 years</i> (615) 225-0068
Arimidex® anastrozole	AstraZeneca Wilmington, DE	gynecomastia (boys)	Phase II <i>11 years–18 years</i> (800) 236-9933
CellCept® mycophenolate mofetil	Aspreva Pharmaceuticals Basking Ridge, NJ Roche Nutley, NJ	lupus nephritis	Phase III <i>12 years and older</i> (908) 212-1020 (973) 235-5000
OMS103HP	Omeros Seattle, WA	anterior cruciate ligament (ACL) reconstruction (knee)	Phase III <i>15 years and older</i> (206) 676-5000
Riquent® abetimus sodium (Orphan Drug)	La Jolla Pharmaceutical San Diego, CA	systemic lupus erythematosus	application submitted <i>12 years and older</i> (858) 452-6600

OTHER

Product Name	Sponsor	Indication	Development Status
Rituxan® rituximab	Genentech <i>South San Francisco, CA</i>	lupus nephritis, systemic lupus erythematosus	Phase III <i>16 years and older</i> (650) 225-1000
Seasonique™ levonorelrel ethinyl estradiol	Duramed Research <i>Bala Cynwyd, PA</i>	dysmenorrhea	Phase III <i>up to 17 years</i> (610) 747-2600
SP01A	Samaritan Pharmaceuticals <i>Las Vegas, NV</i>	iatrogenic (exogenous) Cushing syndrome (hypercortisolism) (see also infectious diseases)	Phase I (702) 735-7001
Stanate® stansoporfin	Infacare <i>Plymouth Meeting, PA</i>	severe hyperbilirubinemia	Phase II <i>neonates</i> (610) 260-1485
Venofer® iron sucrose injection	Luitpold Pharmaceuticals <i>Shirley, NY</i>	chronic kidney disease	Phase IV <i>2 years and older</i> (631) 924-4000
		anemia in non-dialysis chronic kidney disease	Phase III <i>12 years and older</i> (631) 924-4000
widow spider antivenom	Instituto Bioclon <i>Toriello Guerra, Mexico</i>	systemic latrodectism (spider bite pain)	Phase II completed <i>10 years and older</i> www.bioclon.com
Xolair® omalizumab	Genentech <i>South San Francisco, CA</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i> Tanox <i>Houston, TX</i>	peanut allergy (see also respiratory)	Phase II completed <i>6 years and older</i> (650) 225-1000 (888) NOW-NOVA

The content of this report has been obtained through public government and industry sources, and the Adis “R&D Insights” database based on the latest information. **Report current as of March 16, 2007.** The information in this report may not reflect all medicines that are in development for use in children. For more specific information about a particular product, contact the individual company directly or go to www.clinicaltrials.gov. The entire series of *Medicines in Development* is available on PhRMA’s web site.

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anaplastic astrocytoma—A fast-growing primary brain tumor that originates in nerve tissue. This kind of tumor can produce signs of abnormal brain function, such as weakness, loss of sensation, and an unsteady gait.

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

asthma—Recurrent attacks of breathlessness and wheezing caused by a narrowing of the airways (bronchioles) leading to the lungs.

atopic dermatitis—A chronic form of eczema characterized by an intensely itchy skin rash occurring in people who have an inherited tendency toward allergies, such as asthma or allergic rhinitis. It is common in babies, often appearing between the ages of 2 – 18 months.

attention deficit/hyperactivity disorder—ADHD is a complex neurological impairment that results in a constantly overactive behavior pattern and a difficulty concentrating. While it primarily affects children, a growing number of adults are being diagnosed with the disorder. Boys are afflicted some three times as often as girls. Children with ADHD are full of energy, fidgety, impulsive, reckless, irritable, emotionally immature and aggressive. Because their attention span is short, they do not conform to orderly routine. ADHD often leads to anti-social acts and difficulty learning, although IQ is normal. No definite cause has been established, but some researchers now believe heredity plays a role.

autism—One of five disorders that falls under the umbrella of pervasive developmental disorders (PDD), a category of neurological disorders characterized by “severe and pervasive impairment in several areas of development.” The five

disorders under PDD are: autistic disorder, Asperger’s disorder, childhood disintegrative disorder (CDD), Rett’s disorder, and PDD-not otherwise specified (PDD-NOS). Autism is a complex developmental disability that typically appears during the first three years of life and is the result of a neurological disorder that affects the normal functioning of the brain, impacting development in the areas of social interaction and communication skills. Both children and adults with autism typically show difficulties in verbal and non-verbal communication, social interactions, and leisure or play activities. As a spectrum disorder, it affects each individual differently and at varying degrees.

bacterial conjunctivitis—A microbial infection involving the mucous membrane of the surface of the eye.

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

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

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

cystic fibrosis—A lethal genetic disorder of exocrine epithelial glands of multiple organs, with a wide range of symptoms including pancreatic insufficiency, dehydrated airways mucus, chronic bacterial infections of the lungs and intestinal obstruction.

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.

depression—A feeling of intense sadness, which may follow a recent loss or other sad event but is out of proportion to that event and persists beyond an appropriate length of time.

Major depressive disorder (or major depression syndrome) includes an episode of depression defined as a persistent (for at least 2 weeks) mood disturbance, plus at least 4 of the following: sleep disturbance, changes in psychomotor activity, loss of ability to experience pleasure and interest, fatigue, feelings of worthlessness or guilt, difficulty in concentrating, and preoccupation with death or a wish to die. Major depression is associated with impairment in social functioning. If criteria for major depression have been met but in addition an episode of mania has ever occurred, then the diagnosis becomes **bipolar disorder** (or manic-depressive illness). The essential feature of mania is a distinct period when the predominant mood is either elevated, expansive, or irritable, with associated symptoms including hyperactivity, pressure of speech, flight of ideas, inflated self-esteem, decreased need for sleep, distractibility and excessive involvement in activities that often are flamboyant, bizarre or disorganized.

diabetes—A chronic disease due to abnormal insulin secretion from the pancreas, thereby causing problems in metabolizing sugar. **Type 2**, in most cases, can be controlled by a combination of dietary measures, weight loss, and oral medication. **Type 1** is the more severe form, requiring insulin treatment.

eosinophilic esophagitis—An inflammatory condition in which the wall of the esophagus becomes filled with large numbers of eosinophils, which are white blood cells (leukocytes) manufactured in the bone marrow

and are particularly active in the type of inflammation caused by allergic reactions. The allergen(s) that causes eosinophilic esophagitis is not known. It is not even known whether the allergen is inhaled or ingested. The condition is more common among individuals with other allergic conditions such as **asthma**, hay fever, **allergic rhinitis**, and **atopic dermatitis**.

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.

gastroesophageal reflux disease (GERD)—A backflow of stomach contents upward into the esophagus caused by poorly functioning muscles in the lower esophagus. Because the esophagus lacks a protective lining, stomach acid that refluxes into it causes pain, inflammation, and damage.

glaucoma—An eye disease associated with increased pressure within the eyeball. If untreated, it may lead to permanent and complete blindness. Its onset is insidious in older age groups. There are no symptoms in early stages. Gradual loss of peripheral vision over a period of years eventually results in tunnel vision.

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.

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 versus host disease, a complication of such trans-

plants, immune system cells attack the transplant recipient's tissues.

gynecomastia—Breast enlargement in males. It may occur during puberty or in old age. The disorder also may be caused by certain diseases, drug therapies, or hormonal imbalance.

Haemophilus influenzae b—The bacteria causing the most serious bacterial infections in early childhood, including meningitis and pneumonia.

hemophilia—Hemophilia A, the "classic" hemophilia, is a genetic bleeding disorder due to deficiency of the coagulation factor VIII.

hepatitis—Inflammation of the liver with accompanying liver cell damage or death, caused most often by viral infection, e.g., **hepatitis A, B, and C**, but also by certain drugs, chemicals or poisons. Hepatitis may be either acute (of limited duration) or chronic (continuing). **Hepatitis A** spreads primarily from the stool of one person to the mouth of another, usually the result of poor hygiene. Both **hepatitis B** and **C** are transmitted through blood, unprotected sex, shared or re-used needles, or from an infected mother to her newborn baby during delivery.

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

herpes simplex virus—There are three strains of the herpes virus: **Herpes simplex virus I (HSV I)**, which causes **cold sores** or fever blisters on the mouth or around the eyes and can be transmitted to the genital region. The latent virus can be reactivated by stress, trauma, other infections or

suppression of the immune system to produce infection. **Herpes simplex II (HSV II)** causes painful sores of the anus or genitals. The virus may lie dormant in nerve tissue and can be reactivated to produce the sores. **Herpes varicella zoster virus (HVZ)**, also called shingles, consists of very painful blisters on the skin and affects areas innervated by specific nerves. It may appear in adulthood as a result of having had chicken pox (caused by the varicella virus) as a child.

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

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.

hypertension (high blood pressure)—Persistent elevation of blood pressure above the normal range while the heart is in systolic (contracting) or diastolic (relaxed) mode. Uncontrolled, chronic hypertension strains the heart, damages arteries and creates a greater risk of heart attack, stroke and kidney problems.

hypoxic—Refers to the lack of oxygen.

infantile spasm (IS)—A specific type of seizure seen in an epilepsy syndrome of infancy and early childhood known as West syndrome. The onset is predominantly in the first year of life, typically between 3-6 months. Spasms tend to begin soon after arousal from sleep. Individual spasms typically last for one to five seconds and occur in clusters, ranging from two to 100 spasms at a time. Infants may have dozens of clusters and several hundred spasms per day. Infantile spasms usually stop by age 5, but are often replaced by other seizure types. West syndrome is characterized by infantile spasms,

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abnormal, chaotic brain wave patterns, and mental retardation. Other neurological disorders, such as **cerebral palsy**, may be seen in 30 percent to 50 percent of those with IS.

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 disease, characterized by a particular kind of abnormal cell. All others are called non-Hodgkin lymphomas, which vary in their malignancy according to the nature and activity of the abnormal cells.

McCune-Albright syndrome—A rare multisystem disorder characterized by the displacement of normal bone tissue with areas of abnormal fibrous growth. These fibrous bony areas may develop in many bones of the body, causing impaired mobility, pain, and in some cases, hearing and visual impairment. This syndrome is due to a genetic mutation that occurs randomly and for no apparent reason after fertilization; it is not inherited from the parents.

medulloblastoma—The most common primary central nervous system tumor which arises in childhood. Medulloblastomas arise in the fourth ventricle, between the brain stem and the cerebellum. Common symptoms are unsteady-

ness, headaches, and vomiting due to hydrocephalus (from blockage of cerebrospinal fluid flow).

meningitis—An inflammation of the membranes that cover the brain and spinal cord (meninges).

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

migraine headache—Severe headache resulting from an abnormal dilation of blood vessels deep within the brain. It can last from two hours to several days and is often accompanied by nausea, vomiting, sensitivity to noise, light or both.

mitochondrial cytopathies—A group of systemic diseases caused by inherited or acquired damage to the mitochondria, which are small, energy-producing structures found in every cell in the body that serve as the cells' "power plants." When the mitochondria are not working properly (as in the case of mitochondrial cytopathy), there is an energy shortage within those areas of the body that consume large amounts of energy such as the muscles, brain, and heart. The result is often muscle weakness, fatigue, and problems with the heart, eyes, and various other systems. Mitochondrial cytopathies are inherited or acquired disorders, although rarely they can be the result of a spontaneous mutation in early development of the embryo.

MRSA—Methicillin-resistant *Staphylococcus aureus* is a type of bacteria that is resistant to certain antibiotics, including methicillin and other more common antibiotics such as oxacillin, penicillin and amoxicillin. Staph infections, including MRSA, occur most frequently among people in hospitals and healthcare settings.

muscular dystrophy—Inherited muscular disorder of unknown cause in which muscle fibers slowly

degenerate. **Duchenne MD** is the most common type.

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

neurogenic bladder—The loss of normal bladder function caused by damage to part of the nervous system. The damage can cause the bladder to be underactive, in which it is unable to contract and unable to empty completely, or it can be overactive, in which it contracts too quickly or frequently. Risk factors for neurogenic bladder include various birth defects, which adversely affect the spinal cord and function of the bladder, tumors within the spinal cord or pelvis, or traumatic spinal cord injury.

neuronal ceroid lipofuscinosis—A rare childhood genetic degenerative nerve system disease. Some symptoms include personality changes, slow learning, clumsiness, and stumbling.

Noonan syndrome—A genetic disorder that causes abnormal development of multiple parts of the body. Frequently seen abnormalities include webbing of the neck, changes in the sternum (usually a sunken chest), facial abnormalities, and congenital heart disease (especially pulmonary stenosis). Because these abnormalities resemble those in Turner syndrome—which only affects females—Noonan syndrome used to be called "male Turner syndrome." That term is no longer used because Noonan syndrome can affect females also.

osteogenesis imperfecta (OI)—An imperfect creation of the bones. It is a genetic disorder caused by an

abnormality in the gene that instructs the body to make type 1 collagen. OI affects 1 child per every 10,000 that are born. The more severe forms of the disease are less common. With OI, the bones break so easily that infants with the condition are usually born with many broken bones. While most children survive, the multiple fractures often cause deformities and dwarfism. Intelligence is normal if the child's brain isn't injured.

otitis media—Inflammation of the middle ear, often caused by a pneumococcal infection.

partial-onset seizures—Epileptic seizures caused by excessive electrical activity in just one hemisphere of the brain, resulting in a range of symptoms that may include sudden, jerky movements of one part of the body, distorted hearing, sense of smell or vision, numbness and a sudden sense of fear.

Phase I—Human clinical trials, involving healthy volunteers, to determine safety dosage.

Phase II—Human clinical trials, involving a small number of volunteers with the condition the medicine is intended to treat, to evaluate the effectiveness of the medicine and look for side effects.

Phase III—Human clinical trials, involving thousands of people with the disease, to verify effectiveness of the medicine and monitor side effects from widespread, long-term use.

Phase IV—Additional post-marketing studies to evaluate long-term effects.

pheochromocytoma—A tumor of the adrenal glands. These tumors are usually benign (non-cancerous), but can cause dangerously high blood pressure and other symptoms, including pounding headaches, heart palpitations, flushing of the face, nausea, and vomiting. Pheochromocytomas can be found

anywhere in the body, though most occur in the adrenal medulla. Even a tiny benign pheochromocytoma can make a person very sick.

phenylketonuria (PKU)—PKU in its "classic" form is a rare, inherited metabolic disease that results in mental retardation and other neurological problems when treatment is not started within the first few weeks of life. When a very strict diet is begun early and well-maintained, affected children can expect normal development and a normal life span. The disease arises from the absence of a single enzyme (phenylalanine hydroxylase), which normally converts the essential amino acid, phenylalanine, to another amino acid, tyrosine. Failure of the conversion to take place results in a buildup of phenylalanine, which is toxic to the central nervous system and causes the severe problems normally associated with PKU.

picornavirus—Any of a group of the smallest known animal viruses. (Pico refers to their small size; rna to their core of RNA.) This group of spheroidal viruses includes viruses that attack the vertebrate intestinal tract and often invade the central nervous system as well (such as polioviruses), viruses that infect tissues in the vertebrate nose (rhinoviruses), and the virus agent of foot-and-mouth disease.

post-traumatic stress disorder—A specific form of anxiety that comes on after stressful or frightening events, such as natural disasters, violence, rape, torture, and serious physical injury. It may also result from military combat and has been called "battle fatigue" or "shell shock." Symptoms include recurring memories or dreams of the event, a sense of personal isolation, disturbed sleep and concentration, a deadening of feelings, irritability and painful feelings of guilt. Symptoms may begin

immediately after the event or may develop many months later.

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.

Pseudomonas aeruginosa—A Gram-negative bacterium common in soil and water. *Pseudomonas aeruginosa* is an opportunistic pathogen, meaning that it exploits some break in the host defenses to initiate an infection. It causes urinary tract infections, respiratory system infections, dermatitis, soft tissue infections, bacteremia, bone and joint infections, gastrointestinal infections and a variety of systemic infections, particularly in patients with severe burns and in cancer and AIDS patients who are immunosuppressed. *Pseudomonas aeruginosa* infection is a serious problem in patients hospitalized with cancer, cystic fibrosis, and burns. The case fatality rate in these patients is 50 percent.

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

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.

respiratory syncytial virus (RSV)—One of the most important causes of lower respiratory tract disease in children.

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rhabdomyosarcoma—A malignant neoplasm derived from skeletal (striated) muscle.

rheumatoid arthritis—A type of arthritis that particularly attacks the small joints of the hands, wrists and feet. With this autoimmune disorder, the joints become painful, swollen, stiff and, in severe cases, deformed.

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

rhinitis—Inflammation of the nasal mucous membrane.

rotavirus—A virus that is a common cause of stomach upsets and diarrhea in infants.

sarcoma—A malignant tumor that arises from deep body tissues, such as muscle, bone or fibrous tissue.

schizophrenia—The most common form of psychotic illness characterized by disturbances in thinking, emotional reaction and behavior. It is disabling and has a prolonged course that almost always results in chronic ill health and some degree of personality change.

sickle cell disease—An inherited, chronic and severe blood disease where the red blood cells are abnormal (sickle shaped), resulting in reduced oxygen-carrying capacity of the blood. It is caused by mutation of the gene that codes for hemoglobin. The disease affects primarily African Americans.

Staphylococcus aureus—A common bacterium that is a frequent cause of hospital infections, including pneumonia, surgical wounds and systemic blood infections.

testotoxicosis—A condition that causes early puberty in boys, including growth in height and development of muscles and sexual organs.

tinea capitis—An infection of the scalp caused by fungi and characterized by scaly patches penetrated by a few dry brittle hairs.

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

Unverricht-Lundborg disease—The most common of an uncommon

group of genetic epilepsy disorders—the progressive myoclonic epilepsies. Patients with Unverricht-Lundborg have myoclonic movements associated with voluntary body movement (physical exertion) or other stimuli, such as periodic flashes of light (photic sensitivity), noises (auditory induced myoclonus), and stress. Myoclonic movements are quick muscle movements or jerks involving particular muscles. The disease has a slow progressive worsening of symptoms, both in seizure control and neurological functioning, with patients maintaining normal cognitive functioning for a long time and decline in intelligence being very slow. Moderate deterioration may take from 10 to 20 years. Most patients live 50-60 years before dying.

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

varicella zoster—**Chicken pox** and herpes zoster are caused by the varicella zoster virus, chicken pox being the acute invasive phase of the virus and zoster (shingles) being the reactivation of the latent phase.

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Ten Leading Causes of Death in Children:

1-4 years	5-14 years	15-24 years
1. Accidents (unintentional injuries).....(1,606)	1. Accidents (unintentional injuries).....(2,578)	1. Accidents (unintentional injuries).....(15,163)
2. Congenital malformations, deformations, and chromosomal abnormalities(580)	2. Cancer(1,028)	2. Assault (homicide)(4,877)
3. Cancer(388)	3. Congenital malformations, deformations, and chromosomal abnormalities(385)	3. Intentional self-harm (suicide)(4,214)
4. Assault (homicide)(354)	4. Assault (homicides)(318)	4. Cancer(1,680)
5. Diseases of heart.....(191)	5. Intentional self-harm (suicide)(292)	5. Diseases of heart.....(978)
6. Influenza and pneumonia.....(132)	6. Diseases of heart.....(233)	6. Congenital malformations, deformations, and chromosomal abnormalities(482)
7. Septicemia(76)	7. Chronic lower respiratory diseases(113)	7. Cerebrovascular diseases.....(207)
8. Certain conditions originating in the perinatal period(58)	8. In situ neoplasms, benign neoplasms, and neoplasms of uncertain or unknown behavior.....(91)	8. Influenza and pneumonia.....(193)
9. In situ neoplasms, benign neoplasms, and neoplasms of uncertain or unknown behavior.....(53)	9. Influenza and pneumonia(84)	9. Human immunodeficiency virus (HIV) disease(191)
10. Chronic lower respiratory diseases.....(43)	10. Cerebrovascular diseases(77)	10. Chronic lower respiratory diseases(168)

Source: Minino AM, Heron MP, Smith BL. *Deaths: Preliminary Data for 2004*. National Vital Statistics Reports. Vol. 54, No. 19. National Center for Health Statistics. 2006

Arthritis

- Nearly 300,000 children today suffer from some form of **arthritis** or rheumatic disease.¹
- **Juvenile arthritis** is the most common form of arthritis in children, affecting 70,000-100,000 children under age 16.²

Cancer³

- While **childhood cancers** are rare, an estimated 10,400 new cases are expected to occur among children up to age 14 in 2007, and some 1,545 deaths due to cancer are expected among that age group this year. Mortality rates for childhood cancer have declined by 48 percent since 1975.
- **Leukemia** accounts for 30 percent of all childhood cancers and cancer deaths.
- Other childhood cancers include: **brain** and other **nervous system** (22.3 percent); **neuroblastoma** (7.3 percent); **Wilms tumor** (5.6 percent); **non-Hodgkin lymphoma** (4.5 percent) and **Hodgkin lymphoma** (3.5 percent); **rhabdomyosarcoma** (3.1 percent); **retinoblastoma** (2.8 percent); **osteosarcoma** (2.4 percent); and **Ewing sarcoma** (1.4 percent).

Cardiovascular Disease

- An estimated 4.5% of kids have **high blood pressure**. While the condition is far more common among adults, the rate among kids is on the rise, a trend that researchers link to the increase in childhood obesity. High blood pressure in babies is usually caused by prematurity or problems with the kidneys or heart.⁴

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Cardiovascular Disease (continued)

- **Homozygous familial hypercholesterolemia**, a rare inherited disease of metabolism, occurs in fewer than 1 in 1 million people within the United States. Patients with the disease are typically children and young adults who develop heart disease early in life. Children younger than age 5 with this disease have suffered heart attacks and death.⁵
- In 2002, about 229,000 **cardiovascular** procedures were performed on children age 15 or younger.⁶
- Each year, 9.0 **congenital cardiovascular defects** per 1,000 live births (36,000) are expected. Of these, several studies suggest that 9,200, or 2.3 per 1,000 live births, require invasive treatment or result in death in the first year of life.⁶
- Nearly 51 percent of deaths from **congenital cardiovascular defects** in 2002 occurred in children under age 15.⁶

Diabetes⁷

- Two million adolescents (or 1 in 6 overweight adolescents) ages 12-19 have **pre-diabetes**.
- About 176,500 people under age 20 have **diabetes**, representing 0.22 percent of all people in that age group.
- The total economic cost of diabetes in 2002 was an estimated \$132 billion, or \$1 out of every \$10 spent in the United States.

Eye Disorders

- Vision problems affect 1 in 20 preschoolers and 1 in 4 school-age children.⁸
- Childhood **glaucoma** develops in 1 out of 10,000 children.⁹

Gastrointestinal Disorders

- **Eosinophilic esophagitis** affects both children and adults. For unknown reasons, men are more commonly affected than women, and it is most commonly found among young boys and men. Eosinophilic esophagitis is more common among individuals with other allergic conditions such as **asthma**, hay fever, **allergic rhinitis**, and **atopic dermatitis**.¹⁰
- It is estimated that 1 in every 800 children has **Crohn's disease**, which usually appears between the ages of 12 and 16.¹¹ Twenty percent of all cases of Crohn's disease affect children.¹²
- One study has estimated that about 3 percent to 5 percent of children (about 2 million) who are basically healthy have **gastroesophageal reflux disease (GERD)**.¹³

Genetic Disorders

- **Cystic fibrosis (CF)** is the second most common life-shortening, childhood onset inherited disorder in the United States, affecting approximately 30,000 children and adults (and 70,000 worldwide).¹⁴
- About 1,000 new cases of **CF** are diagnosed each year. More than 80 percent of patients are diagnosed by age 3.¹⁴
- The median age of survival for a person with **CF** was nearly 37 years in 2005. Today, nearly 40 percent of the CF population is age 18 and older.¹⁴
- **Noonan syndrome (NS)** is a common childhood genetic disorder which occurs in 1 in 1,000-2,500 live births. NS is the most common single gene cause of congenital heart disease.¹⁵
- While the number of people affected with **osteogenesis imperfecta** in the United States is unknown, the best estimate suggests a minimum of 20,000 and possibly as many as 50,000.¹⁶
- **Pompe disease** is estimated to occur in about 1 in 40,000 births. With the infantile, or early onset, form of the disease, most children die from respiratory or cardiac complications before their first birthday.¹⁷
- **Sickle cell disease** is most common in West and Central Africa where as many as 25 percent of the people have sickle cell trait, and up to 2 percent of all babies are born with a form of the disease. In the United States, about 1,000 babies are born with sickle cell disease each year.¹⁸

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Infectious Diseases/Vaccines

AIDS Cases through December 2005¹⁹

9,089 (<age 13); 6,324 (13-19 years)

AIDS Deaths through December 2005¹⁹

4,865 (<age 13); 1,332 (13-19 years)

- From 2001 through 2005, the estimated number of **HIV/AIDS cases** decreased among children under age 13 and in the age group 13-14 and remained stable among those ages 15-19. Of the estimated 6,792 children living with HIV/AIDS, 90 percent were exposed perinatally.¹⁹
- From 2001 through 2005, the estimated number of **AIDS cases** decreased 51 percent among children under age 13, and it also decreased among those ages 13-14. The estimated number of AIDS cases increased in those ages 15-19. The prevalence rate of AIDS among children was estimated at 2.7 per 100,000 at the end of 2005, when 93 cases in children were reported.¹⁹
- From 2001 through 2005, the estimated number of **AIDS deaths** decreased among children under age 13, remained stable among those ages 15-19, and increased among those ages 13-14.¹⁹
- From 1990 to 1994, before there was a vaccine available, **chickenpox (varicella)** caused some 11,000 hospitalizations as well as 50 deaths in children and 50 deaths in adults every year. Children usually miss five or six days of school or childcare due to chickenpox, and about 1 child in 10 has a complication from it serious enough to visit a health-care provider.²⁰
- In the United States, 5,000-7,000 cases of **pertussis** are reported each year, with incidence increasing steadily since the 1980s. In 2002, more than 8,000 pertussis cases were reported, and in 2003, 13 children died. Most deaths occur among unvaccinated children or children too young to be vaccinated. Epidemics occur here every three to five years, with the most recent having occurred in 1996.²¹
- **Respiratory syncytial virus (RSV)** is the most common cause of bronchiolitis and **pneumonia** among infants and children under age one. RSV infection is also the most common cause of hospitalization for acute respiratory disease among children age one or under. Annually, an estimated 51,000-82,000 young children are hospitalized due to RSV, and approximately 2 percent die each year.²²
- **Rotavirus** infection is the most common cause of severe **diarrhea** among children under age 5, resulting in the hospitalization of approximately 55,000 children each year in the United States and the deaths of more than 600,000 children annually worldwide. Most U.S. children are infected by age 2. About 1 in 40 children with rotavirus gastroenteritis will require hospitalization for intravenous fluids.²¹
- **Streptococcus pneumoniae** infection causes tens of thousands of cases of meningitis and pneumonia and 7 million cases of ear infection each year.²³
- The annual cost of medical care for treating **infectious diseases** in the United States alone is estimated to exceed \$120 billion.²³

Neurologic Disorders

- Of the 300,000 people who have a first **convulsion** each year, 120,000 of them are under age 18, and between 75,000 and 100,000 of them are children under age 5 who have had a fever-caused (febrile) seizure.²⁴
- Each year, 200,000 new cases of **epilepsy** are diagnosed; incidence is highest under age 2 and over age 65. Also annually, 45,000 children under age 15 develop epilepsy. Some 326,000 school children through age 14 have epilepsy.²⁴
- By age 20, 1 percent of the population can be expected to have developed **epilepsy**.²⁴
- **Duchenne muscular dystrophy** is the most common form of muscular dystrophy in children. It mainly affects boys. An estimated 1 in 3,500 males worldwide has the disorder, and each year approximately 400 boys in the United States are born with it. Symptoms usually appear between the ages of two and five.¹⁵
- With **Lennox-Gastaut syndrome**, a severe form of epilepsy, seizures usually begin before age 4. In up to 35 percent of cases, no cause can be found.¹⁷
- **Migraine** affects some 10 percent of school-age children.²⁵ During the high school years, about 20 percent of adolescents get migraines. They are more common in girls than in boys. Boys who get migraines have them more often when they are about 10- to 12-years-old. It is not unusual for them to have two to three migraines a week.²⁶

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Psychiatric Disorders

- Ten years ago **autism** was diagnosed in 1 out of 10,000 children and considered a rare disorder. Today, autism is diagnosed in 1 out of 166 children—a growing epidemic with no cure. There are 1.5 million people with autism living in the United States today—450,000 of whom are children needing individualized special education programs.²⁷
- In the United States today, 1 in 10 (some 4 million) children suffer from a **mental disorder** severe enough to cause some level of impairment. Researchers supported by the National Institute of Mental Health (NIMH) have found that half of all lifetime cases of mental illness begin by age 14 and, despite effective treatments, there are long delays—some times decades—between the first onset of symptoms and when people seek and receive treatment. Thus, mental disorders are chronic diseases of the young: **anxiety disorders** often begin in late childhood, **mood disorders** in late adolescence, and **substance abuse** in the early '20s.²⁸
- It is estimated that between 3 percent to 5 percent of children have **attention-deficit/hyperactivity disorder (ADHD)**, or some 2 million children in this country. In a classroom of 25 to 30 children, it is likely that at least one will have ADHD.²⁸ ADHD is one of the most common reasons children are referred for mental health services. The disorder is three to four times more common in boys than in girls, although both genders are affected.²⁹
- As many as 1 in 8 teens and 1 in 33 children have **clinical depression**. Once a young person has experienced an episode of **depression**, he or she is at an increased risk for having another episode of depression within the next five years. Children who experience a depressive episode are five times more likely to have depression as an adult.²⁹
- **Depression** in children is associated with an increased risk for **suicide**. The U.S. Surgeon General estimates that more than 90 percent of children and adolescents who take their lives have a mental health disorder such as depression. The rate of suicide among young people has nearly tripled since 1960.²⁹
- Up to one-third of the 3.4 million children and adolescents with **depression** may actually be experiencing the early onset of **bipolar disorder**.²⁹
- Psychotic symptoms (such as hallucinations and delusions) usually emerge in men in their late teens and early 20s and in women in their mid-20s to early 30s. They seldom occur after age 45 and only rarely before puberty, although cases of **schizophrenia** in children as young as 5 have been reported. People with schizophrenia attempt **suicide** much more often than people in the general population. About 10 percent (especially young adult males) succeed.²⁸
- In 1998, the direct costs for the treatment of child mental health problems (emotional and behavioral) were approximately \$11.75 billion or \$173 per child, including the cost of services provided by health and mental health professionals to treat mental illness.²⁸

Respiratory Disorders

- An estimated 6.2 million children under age 18 have **asthma**, the leading serious chronic illness among children, and 4 million suffered from an asthma attack or episode in 2004.²²
- **Asthma** is the leading cause of school absenteeism attributed to chronic conditions, accounting for 14 million lost school days in 2004.²²
- The annual total cost of asthma to the U.S. economy is \$16.1 billion—about \$11.5 billion in direct health-care costs and \$4.6 billion in indirect costs (e.g., lost productivity).²²
- **Bronchopulmonary dysplasia (BPD)** is a chronic lung disease that predominately develops in premature infants weighing less than 2.2 pounds at birth. BPD ranks with **cystic fibrosis** and **asthma** among the most common chronic lung diseases in infants in the United States. Approximately 5,000 to 10,000 new cases of BPD occur each year. White, male infants seem to be at greater risk for developing BPD.²²
- **Recurrent respiratory papillomatosis (RRP)** is a rare disease—there are perhaps 20,000 active cases in the United States. RRP occurs in both children and adults. In children, the disease is almost always diagnosed by age 10 and usually before the age of 5, showing no sexual preference.³⁰
- **Respiratory syncytial virus (RSV)** is most common in infants and young children and is the most common cause of bronchiolitis and pneumonia among infants and children under age one. Annually, an estimated 51,000-82,000 young children are hospitalized due to RSV and approximately 2 percent die each year.²²

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Skin Disorders

- **Atopic dermatitis (AD)** is a very common skin disease, affecting around 10 percent of all infants and children. It usually begins during the first year of life and almost always within the first five years. It's seldom present at birth, but it often comes on after six weeks.³¹
 - While all races have **psoriasis**, Caucasians tend to have a slightly higher incidence. It appears most often between the ages of 15 and 35, though it can strike in infancy or old age. Psoriasis in infants may be difficult to diagnose and can sometimes be mistaken for eczema. About a third of all patients will present in childhood with psoriasis. Experts say that about 10 percent of all patients with psoriasis get it before the age of 10; this group seems to be more genetically predisposed to psoriasis.³²
 - Children who get ringworm of the scalp (**tinea capitis**) are usually 2 to 10 years old. In the days before antifungal medications, about 5 percent of children in contact with an infected child at school became infected. However, 25 percent of siblings (close contacts) acquired ringworm.³³
-

Transplantation

- The long-term survival rate after bone marrow **transplantation** varies with disease, from 30 percent to 50 percent for children with **leukemia** to 80 percent to 95 percent for children with **genetic diseases**.³⁴
 - A bone marrow or (umbilical) cord blood **transplantation** could benefit more than 35,000 people with life-threatening diseases each year. About 70 percent of the patients who receive a transplant through the National Marrow Donor Program (NMDP) have some form of **leukemia**.³⁵
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27. Autism Education Network (www.autismeducation.net)
28. National Institute of Mental Health (www.nimh.nih.gov)
29. Mental Health America (www.mentalhealthamerica.net)
30. Recurrent Respiratory Papillomatosis Foundation (www.rrp.org)
31. National Eczema Association (www.nationaleczema.org)
32. National Psoriasis Foundation (www.psoriasis.org)
33. University of Michigan Health Systems (www.med.umich.edu)
34. University of California, San Francisco Medical Center (www.ucsfhealth.org)
35. National Marrow Donor Program (www.marrow.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 \$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 29 new therapeutics approved by the FDA in 2006 was 15.6 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.

Medicines in Development for Children is presented by PhRMA in cooperation with the following organizations:

Allergy & Asthma Network Mothers of Asthmatics	CureSearch National Childhood Cancer Foundation
American Academy of Allergy, Asthma and Immunology	Cystic Fibrosis Foundation
American Academy of Child and Adolescent Psychiatry	Easter Seals
American Academy of Dermatology	Epilepsy Foundation
American Academy of Pediatrics	First Candle/SIDS Alliance
American Academy of Physician Assistants	Interamerican College of Physicians & Surgeons
American Brain Tumor Association	The Leukemia and Lymphoma Society
American College of Allergy, Asthma and Immunology	March of Dimes Foundation
American Epilepsy Society	National Alliance for Hispanic Health
American Lung Association	National Association of Children's Hospitals
American Medical Directors Association, Pediatric Long-Term Care Section	National Association of Community Health Centers
American Nurses Association	National Black Nurses Association
American Psychiatric Association	National Cancer Institute
American Society for Microbiology	National Foundation for Infectious Diseases
Asthma and Allergy Foundation of America	National Institute of Child Health and Human Development
Autism Society of America	National Medical Association
Brain Injury Association of America	Pediatric Infectious Diseases Society
Candlelighter's Childhood Cancer Foundation	Sickle Cell Disease Association of America
CHADD: Children and Adults with Attention-Deficit/ Hyperactivity Disorder	Society for Pediatric Research
Children's AIDS Fund	Tourette Syndrome Association
	United Cerebral Palsy Research and Educational Foundation

Being listed in this report in no way implies that the above-mentioned organizations endorse or recommend the use of any of the products in development contained in this publication. For further information, patients should consult their physicians or health care providers.



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