

Children

PRESENTED BY AMERICA'S PHARMACEUTICAL RESEARCH COMPANIES

More Than 200 Medicines Are Now in Testing To Meet Needs of Children and Adolescents

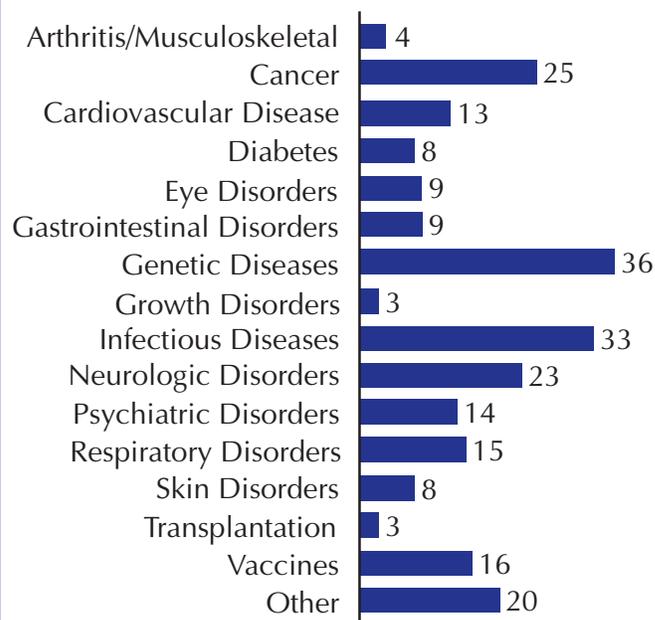
Biopharmaceutical researchers are testing 234 medicines to meet the special health needs of children. These medicines offer hope that the significant improvements achieved in children's health over the past few decades will continue and even accelerate. The potential medicines are either in clinical 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, because of major treatment advances, 80 percent of children diagnosed with cancer last year will survive 5 years or longer, compared to less than 50 percent 30 years ago. Biopharmaceutical research 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:

- 36 for genetic diseases, including medicines for cystic fibrosis, which affects 30,000 American children and adults.
- 33 for such infectious diseases as HIV infection, ear infections, pneumonia, and hepatitis.
- 25 for cancer which, despite significant progress, is still the leading cause of death by disease among American children between ages 5 and 24.
- 23 for neurologic disorders, including medicines for epilepsy, which affects more than 300,000 school children under age 14 in the United States.
- 15 for respiratory disorders, including medicines for asthma, the leading serious chronic disease among children, which affects some 6.7 million children.
- 13 for cardiovascular disease, including hypertension, high cholesterol, and congenital heart disease.

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 September 27, 2007, 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.

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/MUSCULOSKELETAL DISORDERS

Product Name	Sponsor	Indication	Development Status ^{†**}
Actemra™ tocilizumab	Roche <i>Nutley, NJ</i>	juvenile idiopathic arthritis	Phase III 2 years–17 years (973) 235-5000
Humira® adalimumab	Abbott Laboratories <i>Abbott Park, IL</i>	juvenile idiopathic arthritis (see also gastrointestinal)	Phase III 2 years–4 years (847) 937-6100
Ilaris® canakinumab (Orphan Drug)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	systemic juvenile idiopathic arthritis	Phase III 2 years and older (888) 669-6682
Oralgam™ oral human gammaglobulin (Orphan Drug)	Latona Life Sciences <i>Phoenix, AZ</i>	juvenile rheumatoid arthritis	Phase II 2 years–18 years (480) 204-8530

CANCER

Product Name	Sponsor	Indication	Development Status
3F8 mAb	United Therapeutics <i>Silver Spring, MD</i>	neuroblastoma	Phase II 18 months–13 years (301) 608-9292
Alimta® pemetrexed	Eli Lilly <i>Indianapolis, IN</i>	recurrent malignancies	Phase II up to 22 years (800) 545-5979
ARQ-197	ArQule <i>Woburn, MA</i>	microphthalmia transcription (MiT) factor tumors	Phase II 13 years and older (781) 994-0300
Camptosar® irinotecan in combination Temodar® temozolomide	Pfizer <i>New York, NY</i>	brain tumors (glioma, medulloblastoma)	Phase II 6 months–18 years (860) 732-5156
cintredekin besudotox, IL-13	Neopharm <i>Waukegan, IL</i>	recurrent malignant glioma	Phase III 3 years–21 years (847) 887-0800
cisplatin lipid complex (inhalation)	Transave <i>Monmouth Junction, NJ</i>	osteosarcoma metastatic to the lung	Phase I/II 13 years and older (732) 438-9434
cixutumumab (IMC-A12)	Eli Lilly <i>Indianapolis, IN</i> Imclone Systems <i>New York, NY</i>	sarcomas	Phase II 12 years and older (800) 545-5979

† 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
Erbix™ cetuximab	Bristol-Myers Squibb <i>Princeton, NJ</i> Imclone Systems <i>New York, NY</i>	solid tumors	in clinical trials <i>pediatric patients</i> (212) 546-4000 (908) 218-9588
figitumumab (CP-751,871)	Pfizer <i>New York, NY</i>	Ewing's sarcoma	Phase I/II <i>9 years and older</i> (860) 732-5156
HQK-1004	HemaQuest Pharmaceuticals <i>Seattle, WA</i>	Epstein-Barr virus-related malignancies	Phase II <i>3 years and older</i> (206) 682-1233
Kepivance® palifermin	Biovitrum <i>Stockholm, Sweden</i>	stomatitis in patients with leukemia	Phase I <i>1 year–16 years</i> www.biovitrum.com
L-annamycin	Callisto Pharmaceuticals <i>New York, NY</i>	relapsed acute lymphocytic leukemia, relapsed acute myeloid leukemia	Phase I <i>12 months–21 years</i> (212) 297-0010
MDX-1401	Bristol-Myers Squibb <i>Princeton, NJ</i> Medarex <i>Princeton, NJ</i>	Hodgkin lymphoma	Phase I <i>12 years and older</i> (212) 546-4000 (800) 332-2056
nimotuzumab	YM Biosciences USA <i>Wayne, PA</i>	gliomas	Phase II <i>3 years–18 years</i> (610) 560-0600
perifosine (KRX-0401)	Keryx Biopharmaceuticals <i>New York, NY</i>	solid tumors	Phase II <i>up to 18 years</i> (212) 531-5965
PF-03084014	Pfizer <i>New York, NY</i>	cancer, leukemia	Phase I <i>16 years and older</i> (860) 732-5156
Prolia™ denosumab	Amgen <i>Thousand Oaks, CA</i>	bone cancer	Phase II <i>12 years and older</i> (805) 447-1000
Rexin-G® (tumor-targeted gene medicine) (Orphan Drug)	Epeius Biotechnologies <i>San Marino, CA</i>	sarcoma	Phase I/II <i>10 years and older</i> (626) 441-6695
ridaforolimus (Orphan Drug)	Ariad Pharmaceuticals <i>Cambridge, MA</i> Merck <i>Whitehouse Station, NJ</i>	metastatic sarcomas	Phase III <i>13 years and older</i> (617) 494-0400 (800) 672-6372
robatumumab	Merck <i>Whitehouse Station, NJ</i>	Ewing's sarcoma, osteosarcoma	Phase II <i>4 years and older</i> (800) 672-6372
		----- solid tumors	Phase I <i>up to 21 years</i> (800) 672-6372

CANCER

Product Name	Sponsor	Indication	Development Status
SGN-35	Seattle Genetics <i>Bothell, WA</i>	anaplastic large-cell lymphoma, non-Hodgkin lymphoma	Phase II <i>12 years and older</i> (425) 527-4000
Sprycel™ dasatinib (Orphan Drug)	Bristol-Myers Squibb <i>Princeton, NJ</i>	chronic myeloid leukemia	in clinical trials <i>pediatric patients</i> (212) 546-4000
Taxotere® docetaxel	sanofi-aventis <i>Bridgewater, NJ</i>	solid tumors	Phase III <i>pediatric patients</i> (800) 633-1610
Ultratrace™ iobenguane (Orphan Drug)	Molecular Insight Pharmaceuticals <i>Cambridge, MA</i>	neuroblastoma	Phase II <i>12 months and older</i> (617) 492-5554
		paraganglioma, pheochromocytoma	Phase II <i>12 years and older</i> (617) 492-5554
Vectibix® panitumumab	Amgen <i>Thousand Oaks, CA</i>	solid tumors	Phase I <i>1 year–17 years</i> (805) 447-1000

CARDIOVASCULAR DISEASE

Product Name	Sponsor	Indication	Development Status
Benicar® olmesartan medoxomil	Daiichi Sankyo <i>Parsippany, NJ</i>	hypertension	Phase II/III completed <i>1 year–16 years</i> (973) 359-2600
cicletanine (Orphan Drug)	Gilead Sciences <i>Foster City, CA</i>	pulmonary arterial hypertension	Phase II <i>16 years and older</i> (800) 445-3235
Cozaar® losartan	Merck <i>Whitehouse Station, NJ</i>	hypertension	Phase III <i>6 months–6 years</i> (800) 672-6372
Diovan® valsartan	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	hypertension	Phase III <i>6 months–5 years</i> (888) 669-6682
L-citrulline (intravenous)	Asklepion Pharmaceuticals <i>Brentwood, TN</i>	pulmonary hypertension in children undergoing heart bypass surgery	Phase III <i>up to 17 years</i> (615) 377-4617
macitentan	Actelion Pharmaceuticals US <i>South San Francisco, CA</i>	pulmonary arterial hypertension	Phase III <i>12 years and older</i> (650) 624-6900
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

CARDIOVASCULAR DISEASE

Product Name	Sponsor	Indication	Development Status
Remodulin® treprostinil (sustained-release oral formulation)	United Therapeutics <i>Silver Spring, MD</i>	pulmonary arterial hypertension	Phase III <i>12 years and older</i> (301) 608-9292
sitaxsentan	Pfizer <i>New York, NY</i>	pulmonary arterial hypertension	Phase III <i>16 years and older</i> (860) 732-5156
tadalafil	Eli Lilly <i>Indianapolis, IN</i>	pulmonary arterial hypertension	Phase III <i>12 years and older</i> (317) 276-2000
Tektura® aliskiren	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	hypertension	Phase I/II <i>6 years–17 years</i> (888) 669-6682
Tracleer® bosentan (pediatric formulation) (Orphan Drug)	Actelion Pharmaceuticals USA <i>South San Francisco, CA</i>	pulmonary arterial hypertension	Phase III completed <i>2 years–12 years</i> (650) 624-6900
Zetia® ezetimibe	Merck <i>Whitehouse Station, NJ</i>	primary hypercholesterolemia	Phase III <i>6 years–10 years</i> (800) 672-6372

DIABETES

Product Name	Sponsor	Indication	Development Status
alogliptin	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	type 2 diabetes	Phase I <i>10 years and older</i> (877) 582-5332
autoimmune diabetes vaccine (rhGAD65)	Diamyd Therapeutics <i>Stockholm, Sweden</i> <i>Pittsburgh, PA</i>	type 1 diabetes	Phase III <i>10 years and older</i> (412) 770-1310
Byetta® exentide	Amylin Pharmaceuticals <i>San Diego, CA</i> Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase III <i>10 years–16 years</i> (858) 552-2200 (800) 545-5979
Januvia® sitagliptin	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase I <i>10 years–17 years</i> (800) 672-6372
otelixizumab	Tolerx <i>Cambridge, MA</i>	type 1 diabetes	Phase II <i>12 years and older</i> (617) 354-8100
Prochymal® human mesenchymal stem cells	Osiris Therapeutics <i>Columbia, MD</i>	type 1 diabetes (see also transplantation)	Phase II <i>12 years and older</i> (443) 545-1701

DIABETES

Product Name	Sponsor	Indication	Development Status
teplizumab (MGA031) (Orphan Drug)	Eli Lilly <i>Indianapolis, IN</i> MacroGenics <i>Rockville, MD</i>	type 1 diabetes	Phase III <i>8 years and older</i> (800) 545-5979 (301) 251-5172
Victoza® liraglutide	Novo Nordisk <i>Princeton, NJ</i>	type 2 diabetes	Phase I <i>10 years and older</i> (800) 727-6500

EYE DISORDERS

Product Name	Sponsor	Indication	Development Status
AL-46383A (ophthalmic solution)	Alcon Research <i>Fort Worth, TX</i>	adenoviral conjunctivitis	Phase II <i>6 years and older</i> (817) 293-0450
alcaftadine (ophthalmic solution) (R89674)	Vistakon Pharmaceuticals <i>Raritan, NJ</i>	allergic conjunctivitis	Phase III completed <i>10 years and older</i>
Cystoran™ cysteamine hydrochloride (Orphan Drug)	Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	corneal cystine crystal accumulation in cystinosis patients	Phase III completed <i>1 year and older</i> (800) 447-0169
latanoprost/timolol fixed-dose combination	Pfizer <i>New York, NY</i>	glaucoma	Phase III <i>36 weeks–18 years</i> (860) 732-5156
LX211 (voclosporin) (Orphan Drug)	Lux Biosciences <i>Jersey City, NJ</i>	uveitis	Phase III completed <i>13 years and older</i> (201) 946-0551
microplasmin (Orphan Drug)	ThromboGenics <i>New York, NY</i>	adjunct to conventional vitrectomy	Phase II <i>up to 16 years</i> (212) 201-0920
moxifloxacin AF ophthalmic solution (alternative formulation)	Alcon Research <i>Fort Worth, TX</i>	bacterial conjunctivitis	Phase III <i>1 month and older</i> (817) 293-0450
Patanase® olopatadine intranasal	Alcon Research <i>Fort Worth, TX</i>	allergic rhinitis	application submitted <i>6 months–12 years</i> (800) 862-5266
Zylet™ loteprednol etabonate 0.5%/ tobramycin 0.3% ophthalmic solution	Bausch & Lomb <i>Rochester, NY</i>	chalazion hordeolum (eye lid inflammation)	Phase IV completed <i>up to 6 years</i> (585) 338-6000
		----- blepharoconjunctivitis	Phase IV <i>up to 6 years</i> (585) 338-6000

GASTROINTESTINAL DISORDERS

Product Name	Sponsor	Indication	Development Status
Aciphex® rabeprazole sodium (delayed-release formulation)	Eisai <i>Woodcliff Lake, NJ</i> Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	gastroesophageal reflux disease (GERD)	Phase III 1 year–11 years (888) 422-4743 (800) 817-5286
		GERD	Phase I 1 month–11 months (888) 422-4743 (800) 817-5286
Asacol® mesalamine delayed-release tablets (400mg)	Procter & Gamble Pharmaceuticals <i>Cincinnati, OH</i>	ulcerative colitis	Phase III 5 years–17 years www.pgpharma.com
Asacol® HD mesalamine delayed-release tablets (800mg)	Procter & Gamble Pharmaceuticals <i>Cincinnati, OH</i>	ulcerative colitis	Phase III 5 years–17 years www.pgpharma.com
Axid® nizatidine oral solution	Braintree Laboratories <i>Braintree, MA</i>	GERD	Phase III completed up to 1 year (800) 874-6756
Bosatria™ mepolizumab	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	hypereosinophilic syndrome (see also respiratory)	Phase III 12 years and older (888) 825-5249
		eosinophilic esophagitis	Phase II completed 2 years–17 years (888) 825-5249
budesonide oral suspension (Orphan Drug)	Meritage Pharma <i>San Diego, CA</i>	eosinophilic esophagitis	Phase II 2 years–18 years (858) 436-1660
Cimzia™ certolizumab pegol	UCB <i>Smyrna, GA</i>	Crohn's disease	Phase II 6 years–17 years (770) 970-7500
Humira® adalimumab	Abbott Laboratories <i>Abbott Park, IL</i>	moderate to severe Crohn's disease (see also arthritis)	Phase III 7 years–18 years (847) 937-6100
Remicade® infliximab	Centocor Ortho Biotech <i>Horsham, PA</i>	ulcerative colitis	Phase III 6 years–17 years (610) 651-6000

GENETIC DISEASES

Product Name	Sponsor	Indication	Development Status
656933	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	cystic fibrosis	Phase I 16 years and older (888) 825-5249

GENETIC DISEASES

Product Name	Sponsor	Indication	Development Status
A-001	Anthera Pharmaceuticals <i>Hayward, CA</i>	acute chest syndrome in patients with sickle cell disease	Phase II <i>5 years and older</i> (510) 856-5600
ALD-101 (Orphan Drug)	Aldagen <i>Durham, NC</i>	inherited metabolic disorders	Phase III <i>up to 16 years</i> (919) 484-2571
arbaclofen (STX209)	Seaside Therapeutics <i>Cambridge, MA</i>	fragile X syndrome (see also psychiatric)	Phase II <i>6 years and older</i> (617) 374-9009
AT1001 (migalastat) (Orphan Drug)	Amicus Therapeutics <i>Cranbury, NJ</i>	Fabry disease	Phase III <i>16 years and older</i> (609) 662-2000
ataluren (Orphan Drug)	Genzyme <i>Cambridge, MA</i> PTC Therapeutics <i>South Plainfield, NJ</i>	cystic fibrosis	Phase III <i>6 years and older</i> (617) 252-7200 (908) 222-7000
		Duchenne/Becker muscular dystrophy (boys)	Phase II/III <i>7 years and older</i> (617) 252-7200 (908) 222-7000
BAY 79-4980	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i>	hemophilia A	Phase II <i>12 years and older</i> (800) 422-9374
cysteamine bitartrate delayed-release (RP-103) (Orphan Drug)	Raptor Pharmaceutical <i>Novato, CA</i>	cystinosis	Phase III <i>pediatric patients</i> (877) 727-8679
denufosal tetrasodium inhalation solution (Orphan Drug)	Inspire Pharmaceuticals <i>Durham, NC</i>	cystic fibrosis	Phase III <i>5 years and older</i> (919) 941-9777
DNA nanoparticle gene therapy (Orphan Drug)	Copernicus Therapeutics <i>Cleveland, OH</i>	cystic fibrosis	Phase I/II (216) 231-0227
Elaprase [®] idursulfase (intrathecal administration) (Orphan Drug)	Shire Human Genetic Therapies <i>Cambridge, MA</i>	mucopolysaccharidosis II (Hunter syndrome)	Phase I/II <i>3 years–8 years</i> (617) 349-0300
ENB-0040 (Orphan Drug)	Enobia Pharma <i>Montreal, Canada</i>	hypophosphatasia	Phase II <i>5 years–12 years</i> (514) 596-2901
Faslodex [®] fulvestrant	AstraZeneca <i>Wilmington, DE</i>	precocious puberty associated with McCune-Albright syndrome (girls)	Phase II <i>up to 10 years</i> (800) 236-9933

GENETIC DISEASES

Product Name	Sponsor	Indication	Development Status
Genz-112638	Genzyme Cambridge, MA	Gaucher disease, type 1	Phase III 16 years and older (617) 252-7200
HuCNS-SC	StemCells Palo Alto, CA	infantile or late infantile neuronal ceroid lipofuscinosis (intracerebral)	Phase I 18 months–12 years (650) 475-3100
		Pelizaeus-Merzbacher disease (implant)	Phase I 6 months–5 years (650) 475-3100
idebenone (Orphan Drug)	Santhera Pharmaceuticals Charlestown, MA	Friedreich's ataxia	Phase III 8 years–18 years (617) 886-5161
mannitol inhalation (therapeutic) (Orphan Drug)	Pharmaxis Exton, PA	cystic fibrosis	Phase III 6 years and older (610) 363-5120
mipomersen (Orphan Drug)	Genzyme Cambridge, MA Isis Pharmaceuticals Carlsbad, CA	familial hypercholesterolemia	Phase II 12 years and older (617) 252-7500 (760) 931-9200
new generation immunoglobulin (IVIG)	Octapharma USA Hoboken, NJ	primary immunodeficiency diseases	Phase III 2 years and older (201) 604-1130
NutreStore™ L-glutamine powder for oral solution (Orphan Drug)	Emmaus Medical Torrance, CA	sickle cell anemia, thalassemia	Phase II 5 years and older (877) 420-6493
Oxabect™ oxalobacter formigenes (Orphan Drug)	OxThera Alachua, FL	primary hyperoxaluria	Phase II/III 5 years and older (386) 418-1428
PEG-PAL (Orphan Drug)	BioMarin Pharmaceutical Novato, CA	phenylketonuria	Phase II 16 years and older (415) 506-6700
plasma-derived AT-III concentrate	Grifols Biologicals Los Angeles, CA	antithrombin III deficiency	Phase II/III 12 years and older (888) 474-3657
recombinant factor VII (long-acting)	Novo Nordisk Princeton, NJ	hemophilia A or B with inhibitors	Phase II 12 years and older (800) 727-6500
recombinant factor VIII	Octapharma Hoboken, NJ	hemophilia A	Phase II 12 years and older (201) 604-1130
recombinant factor VIII (N8)	Novo Nordisk Princeton, NJ	hemophilia A	Phase III 12 years and older (800) 727-6500

GENETIC DISEASES

Product Name	Sponsor	Indication	Development Status
recombinant human C1 inhibitor (Orphan Drug)	Pharming Technologies <i>Leiden, The Netherlands</i>	hereditary angioedema	Phase II <i>12 years and older</i> www.pharming.com
Replagal® agalsidase alfa (Orphan Drug)	Shire Human Genetic Therapies <i>Cambridge, MA</i>	Fabry disease	Phase II <i>7 years–17 years</i> (617) 349-0300
rFVIII-Fc	Biogen Idec <i>Cambridge, MA</i> Biovitrum <i>Stockholm, Sweden</i>	hemophilia A	Phase I <i>12 years and older</i> (617) 679-2000
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
UbiQGel® ubidecarenone (Orphan Drug)	Tishcon <i>Westbury, NY</i>	mitochondrial cytopathies	Phase III <i>12 months–17 years</i> (516) 333-3050
ULTRASE®MT 12 pancrelipase	Axcan Pharma <i>Birmingham, AL</i>	pancreatic insufficiency in cystic fibrosis patients	Phase III <i>2 years–6 years</i> (800) 472-2634
velaglucerase alfa	Shire Human Genetic Therapies <i>Cambridge, MA</i>	Gaucher disease, type 1	Phase III <i>2 years and older</i> (617) 349-0300
VX-770 (Orphan Drug)	Vertex Pharmaceuticals <i>Cambridge, MA</i> Cystic Fibrosis Foundation <i>Bethesda, MD</i>	cystic fibrosis	Phase III <i>6 years and older</i> (617) 444-6100
Xyntha™ morotocog alfa (Orphan Drug)	Pfizer <i>New York, NY</i>	hemophilia A (boys)	Phase III <i>younger than 6 years</i> (860) 732-5156
Zithromax® azithromycin	Pfizer <i>New York, NY</i> Cystic Fibrosis Foundation Therapeutics <i>Bethesda, MD</i>	cystic fibrosis	Phase III <i>6 years–18 years</i> (860) 732-5156 (800) 344-4823

GROWTH DISORDERS

Product Name	Sponsor	Indication	Development Status
Casodex® bicalutamide Arimidex® anastrozole combination	AstraZeneca <i>Wilmington, DE</i>	testotoxicosis (boys)	Phase II <i>2 years and older</i> (800) 236-9933
somatropin (ALTU-238)	Altus Pharmaceuticals <i>Waltham, MA</i>	growth hormone deficiency	Phase II <i>3 years–13 years</i> (781) 373-6432

GROWTH DISORDERS

Product Name	Sponsor	Indication	Development Status
somatropin (sustained-release)	LG Life Sciences <i>Seoul, South Korea</i>	insufficient secretion of growth hormone	Phase III 3 years–11 years

INFECTIOUS DISEASES

Product Name	Sponsor	Indication	Development Status
ADS-8902	Adamas Pharmaceuticals <i>Emeryville, CA</i>	influenza A	Phase II 1 year and older (510) 450-3500
anidulafungin/ voriconazole combination	Pfizer <i>New York, NY</i>	treatment of invasive aspergillosis	Phase III 16 years and older (860) 732-5156
Aptivus [®] tipranavir (low-dose)	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	HIV-1 infection treatment	Phase I/II 2 years–18 years (800) 243-0127
Baraclude [®] entecavir	Bristol-Myers Squibb <i>Princeton, NJ</i>	chronic hepatitis	in clinical trials <i>pediatric patients</i> (212) 546-4000
Cayston [®] aztreonam for inhalation solution	Gilead Sciences <i>Foster City, CA</i>	treatment of <i>Pseudomonas aeruginosa</i> in cystic fibrosis patients	application submitted 6 years and older (800) 445-3235
ceftobiprole	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	streptococcal infections	Phase I 3 months–18 years (800) 817-5286
ciprofloxacin PulmoSphere [®] inhalation powder	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i> Nektar Therapeutics <i>San Carlos, CA</i>	treatment of <i>Pseudomonas aeruginosa</i> in cystic fibrosis	Phase I 6 years–12 years (800) 422-9374 (650) 631-3100
Cubicin [®] daptomycin for injection	Cubist Pharmaceuticals <i>Lexington, MA</i>	skin and skin structure infections	Phase IV 2 years–17 years (781) 860-8660
		gram-positive bacterial infections	Phase I 3 months–24 months (781) 860-8660
Eraxis [®] anidulafungin	Pfizer <i>New York, NY</i>	candidiasis, including candidemia	Phase III 1 month–17 years (860) 732-5156
Famvir [®] famciclovir (pediatric formulation)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	herpes simplex infection	Phase III completed 1 year–12 years (888) 669-6682
fidaxomicin	Optimer Pharmaceuticals <i>San Diego, CA</i>	<i>Clostridium difficile</i> -associated diarrhea	Phase III 16 years and older (858) 909-0736

INFECTIOUS DISEASES

Product Name	Sponsor	Indication	Development Status
FluBlok® trivalent recombinant hemagglutinin vaccine	Protein Sciences <i>Meriden, CT</i>	prevention of influenza virus infection	application submitted <i>children</i> (800) 488-7099
Intelece™ etravirine	Tibotec Therapeutics <i>Titusville, NJ</i>	HIV-1 infection	Phase III <i>6 years–18 years</i> (609) 730-6000
Isentress® raltegravir	Merck <i>Whitehouse Station, NJ</i>	HIV-1 infection	Phase I/II <i>children, adolescents</i> (800) 672-6372
ivermectin cream	Topaz Pharmaceuticals <i>Horsham, PA</i>	pediculosis (head lice)	Phase II <i>2 years and older</i> (267) 960-3330
Lexiva® fosamprenavir	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	HIV infection	Phase II <i>4 weeks–2 years</i> (888) 825-5249
malathion gel (topical gel formulation)	Taro Pharmaceuticals USA <i>Hawthorne, NY</i>	pediculosis (head lice)	Phase III <i>2 years and older</i> (800) 544-1449
		pediculosis (head lice)	Phase II/III <i>6 months–24 months</i> (800) 544-1449
Mycamine® micafungin	Astellas Pharma US <i>Deerfield, IL</i>	candidiasis	Phase I <i>4 months–23 months</i> (800) 695-4321
		candidiasis	Phase I <i>2 years–16 years</i> (800) 695-4321
NatrOVA™ spinosad creme rinse	ParaPro <i>Carmel, IN</i>	pediculosis (head lice)	Phase I <i>6 months and older</i> (877) 542-3639
Numax® motavizumab intravenous	MedImmune <i>Gaithersburg, MD</i>	respiratory syncytial virus	Phase II <i>up to 12 months</i> (301) 398-0000
pagibaximab	Biosynexus <i>Gaithersburg, MD</i>	staphylococcal sepsis	Phase II/III <i>neonates up to 48 hours</i> (301) 330-5800
peramivir	BioCryst Pharmaceuticals <i>Birmingham, AL</i>	influenza	Phase III <i>12 years and older</i> (205) 444-4600
Prezista™ darunavir	Tibotec Therapeutics <i>Titusville, NJ</i>	HIV-1 infection	Phase II <i>6 years–17 years</i> (609) 730-6000

INFECTIOUS DISEASES

Product Name	Sponsor	Indication	Development Status
Reyataz [®] atazanavir	Bristol-Myers Squibb <i>Princeton, NJ</i>	HIV infection	in clinical trials <i>pediatric patients</i> (212) 546-4000
RI-001	ADMA Biologics <i>Hackensack, NJ</i>	respiratory tract infections	Phase II <i>2 years and older</i> (201) 478-5552
Selzentry [®] maraviroc	Pfizer <i>New York, NY</i>	HIV-1 infection	Phase IV <i>2 years–18 years</i> (860) 732-5156
Sustiva [®] efavirenz	Bristol-Myers Squibb <i>Princeton, NJ</i>	HIV infection	in clinical trials <i>pediatric patients</i> (212) 546-4000
Valcyte [®] valganciclovir (syrup)	Roche <i>Nutley, NJ</i>	cytomegalovirus disease in solid organ transplant patients	Phase II completed <i>3 months–16 years</i> (973) 235-5000
Valtrex [®] valaciclovir oral suspension	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	herpes simplex virus	Phase I completed <i>1 month–6 years</i> (800) 825-5249
Vfend [®] voriconazole	Pfizer <i>New York, NY</i>	aspergillosis	Phase III <i>2 years and older</i> (860) 732-5156
Viramune [®] XR nevirapine extended-release	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	HIV infection	Phase I <i>3 years–17 years</i> (800) 243-0127
Viread [®] tenofovir disoproxil fumarate	Gilead Sciences <i>Foster City, CA</i>	HIV-1 infection	Phase III <i>12 years–17 years</i> (800) 445-3235
		----- hepatitis B	Phase II/III <i>12 years–17 years</i> (800) 445-3235
zanamivir aqueous solution	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	influenza	Phase II <i>6 months and older</i> (888) 825-5249

NEUROLOGIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Acetavance [™] acetaminophen for injection	Cadence Pharmaceuticals <i>San Diego, CA</i>	treatment of acute pain and fever	application submitted <i>pediatric patients</i> (858) 436-1400
brivaracetam	UCB <i>Smyrna, GA</i>	partial-onset seizures	Phase III <i>16 years and older</i> (770) 970-7500
Ciltiari [®] eplivanserin	sanofi-aventis <i>Bridgewater, NJ</i>	insomnia	Phase I <i>6 years–17 years</i> (800) 633-1610

NEUROLOGIC DISORDERS

Product Name	Sponsor	Indication	Development Status
clobazam (Orphan Drug)	Lundbeck USA <i>Deerfield, IL</i>	Lennox-Gastaut syndrome	Phase III 2 years and older (866) 337-6996
Comfyde™ carisbamate	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	partial-onset seizures	Phase III 16 years and older (800) 817-5286
Enblex® darifenacin	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	neurogenic detrusor overactivity	Phase II 6 years–15 years (888) 669-6682
Epliga™ oxcarbazepine extended-release	Supernus Pharmaceuticals <i>Rockville, MD</i>	refractory partial epilepsy	Phase III 4 years–17 years (301) 838-2500
Flomax® tamsulosin	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	neurogenic bladder	application submitted 2 years–16 years (203) 798-9988
ganaxolone	Marinus Pharmaceuticals <i>Branford, CT</i>	infantile spasms	Phase II 4 months–24 months (203) 315-0566
glycopyrronium bromide (Orphan Drug)	Sciele Pharma <i>Atlanta, GA</i>	drooling associated with cerebral palsy and other neurologic conditions in children	application submitted 3 years–18 years (800) 461-3696
Keppra® levetiracetam intravenous	UCB <i>Smyrna, GA</i>	epilepsy	Phase II 4 years–16 years (770) 970-7500
Keppra® XR levetiracetam extended-release	UCB <i>Smyrna, GA</i>	epilepsy	Phase II 12 years–16 years (770) 970-7500
Lyrica® pregabalin	Pfizer <i>New York, NY</i>	partial-onset seizures	Phase III 1 month–16 years (860) 732-5156
Maxalt® rizatriptan benzoate	Merck <i>Whitehouse Station, NJ</i>	acute migraine	Phase III 6 years–17 years (800) 672-6372
MultiHance® gadobenate dimeglumine	Bracco Diagnostics <i>Princeton, NJ</i>	MRI contrast agent in central nervous system disorders	Phase III completed 2 years–17 years (800) 631-5245
Opana® oxymorphone immediate-release	Endo Pharmaceuticals <i>Newark, DE</i>	acute postoperative pain	Phase III 12 years–17 years (800) 462-3636
perampanel (E2007)	Eisai <i>Woodcliff Lake, NJ</i>	refractory partial seizures (adjunctive therapy)	Phase III 12 years and older (888) 422-4743
Rozerem® ramelteon	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	insomnia	Phase I 12 years and older (224) 554-6500

NEUROLOGIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Treximet™ sumatriptan/ naproxen sodium	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	migraine	Phase III 12 years–17 years (888) 825-5249
Vanquix® diazepam injection	King Pharmaceuticals <i>Bristol, TN</i>	epilepsy	Phase III 2 years and older (800) 776-3637
Vimpat® lacosamide	UCB <i>Smyrna, GA</i>	partial seizures	Phase II 2 years–17 years (770) 970-7500
Xatral® alfuzosin	sanofi-aventis <i>Bridgewater, NJ</i>	neurogenic urinary bladder	Phase III 2 years–16 years (800) 633-1610
YKP3089	SK Drug Development <i>Fair Lawn, NJ</i>	epilepsy	Phase II 16 years and older

PSYCHIATRIC DISORDERS

Product Name	Sponsor	Indication	Development Status
arbaclofen (STX209)	Seaside Therapeutics <i>Cambridge, MA</i>	autism spectrum disorder (see also genetic)	Phase II 6 years–17 years (617) 374-9009
Clonixel® clonidine HCl sustained-release	Addrenex Pharmaceuticals <i>Durham, NC</i>	attention-deficit/hyperactivity disorder	Phase III 6 years–17 years (919) 941-0800
CM-AT	Curemark <i>Rye, NY</i>	autism spectrum disorder	Phase III 3 years–8 years (914) 925-3450
Cymbalta® duloxetine	Eli Lilly <i>Indianapolis, IN</i>	major depressive disorder	Phase III 7 years–17 years (800) 545-5979
Geodon® ziprasidone oral capsules	Pfizer <i>New York, NY</i>	bipolar disorder	Phase III <i>children, adolescents</i> (860) 732-5156
Intuniv™ guanfacine extended-release tablets	Shire Pharmaceutical <i>Wayne, PA</i>	attention-deficit/hyperactivity disorder (combination therapy)	application submitted 6 years–17 years (484) 595-8800
Invega® paliperidone ER (extended-release)	Johnson and Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	schizophrenia	Phase III 12 years–17 years (800) 817-5286
JNJ-31001074	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	attention-deficit/hyperactivity disorder	Phase I 6 years–17 years (800) 817-5286

PSYCHIATRIC DISORDERS

Product Name	Sponsor	Indication	Development Status
Lamictal® lamotrigine	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	bipolar disorder	Phase III 10 years–17 years (888) 825-5249
LY2216684	Eli Lilly <i>Indianapolis, IN</i>	attention-deficit/hyperactivity disorder	Phase II/III 6 years–18 years (800) 545-5979
Namenda® memantine	Forest Laboratories <i>New York, NY</i>	autism spectrum disorder	Phase II 6 years–12 years (800) 947-5227
Pristiq® desvenlafaxine succinate sustained-release	Pfizer <i>New York, NY</i>	major depressive disorder	Phase II 7 years–18 years (860) 732-5156
Seroquel XR® quetiapine fumarate extended-release	AstraZeneca <i>Wilmington, DE</i>	bipolar depression	Phase III 10 years–17 years (800) 236-9933
SPN-810	Supernus Pharmaceuticals <i>Rockville, MD</i>	conduct disorder associated with attention-deficit/hyperactivity disorder	Phase II 6 years–12 years (301) 838-2500

RESPIRATORY DISORDERS

Product Name	Sponsor	Indication	Development Status
Aerosurf™ lucinactant for inhalation (Orphan Drug)	Discovery Laboratories <i>Warrington, PA</i>	neonatal respiratory distress syndrome	Phase II <i>neonates</i> (215) 488-9300
Astepro® azelastine nasal spray	Meda Pharmaceuticals <i>Somerset, NJ</i>	perennial allergic rhinitis	Phase III 6 years–12 years (732) 564-2200
beclomethasone nasal aerosol	Teva Pharmaceuticals USA <i>North Wales, PA</i>	seasonal allergic rhinitis	Phase III 12 years and older (215) 591-3000
Bosatria™ mepolizumab	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	severe asthma (see also gastrointestinal)	Phase II 12 years and older (888) 825-5249
fluticasone furoate/ 642444 combination (inhalation powder)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	asthma	Phase III 12 years and older (888) 825-5249
INO® nitric oxide for inhalation	Ikuria <i>Clinton, NJ</i>	bronchopulmonary dysplasia	Phase III up to 14 days (908) 238-6600
mannitol inhalation diagnostic	Pharmaxis <i>Frenchs Forest, Australia</i>	detection of bronchial hyperresponsiveness in patients with asthma	application submitted 6 years and older www.pharmaxis.com.au

RESPIRATORY DISORDERS

Product Name	Sponsor	Indication	Development Status
motavizumab	MedImmune <i>Gaithersburg, MD</i>	respiratory syncytial virus (RSV)	Phase II <i>up to 12 months</i> (301) 398-0000
Omnaris® ciclesonide HFA nasal spray	Sepracor <i>Marlborough, MA</i>	seasonal allergic rhinitis	Phase III <i>12 years and older</i> (800) 739-0565
QMF149 Twisthaler®	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	asthma	Phase II <i>12 years and older</i> (888) 669-6682
Surfaxin® lucinactant (Orphan Drug)	Discovery Laboratories <i>Warrington, PA</i>	neonatal respiratory distress syndrome (prevention)	application submitted <i>premature infants</i> (215) 488-9300
		bronchopulmonary dysplasia, acute respiratory failure	Phase II <i>neonates, children</i> (215) 488-9300
Symbicort® budesonide/ formoterol	AstraZeneca <i>Wilmington, DE</i>	asthma	application submitted <i>6 years–11 years</i> (800) 236-9933
Veramyst® fluticasone furoate nasal spray	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	asthma	Phase II <i>12 years and older</i> (888) 825-5249
Xolair® omalizumab	Genentech <i>South San Francisco, CA</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	moderate to severe allergic asthma	Phase III <i>6 years–11 years</i> (650) 225-1000 (888) 669-6682
Xopenex® HFA® MDI levalbuterol	Sepracor <i>Marlborough, MA</i>	asthma	Phase III <i>up to 48 months</i> (508) 481-6700

SKIN DISORDERS

Product Name	Sponsor	Indication	Development Status
Amevive® alefacept	Astellas Pharma US <i>Deerfield, IL</i>	psoriasis	Phase II <i>12 years–17 years</i> (800) 695-4321
CIP-isotretinoin	Cipher Pharmaceuticals <i>Mississauga, Canada</i>	severe nodular acne	Phase III <i>12 years and older</i> (905) 602-5840
Enbrel® etanercept	Amgen <i>Thousand Oaks, CA</i>	plaque psoriasis	application submitted <i>pediatric patients</i> (800) 772-6436
Metvixia® methyl aminolevulinate cream (photodynamic therapy)	PhotoCure <i>Oslo, Norway</i>	acne vulgaris	Phase II <i>9 years and older</i> www.photocure.com

SKIN DISORDERS

Product Name	Sponsor	Indication	Development Status
Retin-A Micro® tretinoin gel microsphere	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	acne vulgaris	Phase II 9 years–11 years (800) 817-5286
Taclonex® calcipotriene and betamethasone dipropionate ointment	LEO Pharma <i>Ballerup, Denmark</i>	psoriasis vulgaris	Phase II 12 years–17 years www.leo-pharma.com
tazarotene foam	Stiefel, a GSK Company <i>Coral Gables, FL</i>	acne vulgaris	Phase III 12 years and older (305) 443-3800
Vectical™ calcitriol ointment	Galderma Laboratories <i>Fort Worth, TX</i>	plaque psoriasis	Phase II completed 12 years–17 years (817) 961-5000

TRANSPLANTATION

Product Name	Sponsor	Indication	Development Status
Certican® everolimus	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	de novo renal transplantation	Phase III up to 16 years (888) 669-6682
CYT107 (IL-7)	Cytheris <i>Rockville, MD</i>	bone marrow transplant rejection	Phase I 15 years and older (301) 231-0451
Prochymal® human mesenchymal stem cells (Orphan Drug)	Osiris Therapeutics <i>Baltimore, MD</i>	graft vs. host disease (see also diabetes)	Phase III completed 6 months and older (410) 522-5005

VACCINES

Product Name	Sponsor	Indication	Development Status
134612 (MenACWY-TT conjugated vaccine)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	<i>Neisseria meningitis</i> groups A, C, W and Y disease prophylaxis	Phase III <i>pediatric patients</i> (800) 825-5249
792014 (Hib-MenCY-TT conjugated vaccine)	GlaxoSmithKline <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
1557482A influenza vaccine	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	prevention of influenza	Phase III 3 years–17 years (888) 825-5249

VACCINES

Product Name	Sponsor	Indication	Development Status
2340273A (H1N1 influenza vaccine)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	prevention of H1N1 influenza	Phase II <i>6 months–8 years</i> (888) 825-5249
2340274A (H1N1 influenza vaccine)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	prevention of H1N1 influenza	Phase II <i>6 months–8 years</i> (888) 825-5249
Adacel [®] tetanus toxoid, reduced diphtheria toxoid and acellular pertussis vaccine adsorbed	sanofi pasteur <i>Swiftwater, PA</i>	diphtheria, tetanus and pertussis	Phase III <i>4 years–6 years</i> (570) 839-7187
H1N1 influenza vaccine	Novartis Vaccines <i>East Hanover, NJ</i>	prevention of H1N1 influenza	Phase III <i>6 months–35 months</i> (888) 669-6682
MEDI-534 (RSV/PIV-3 vaccine)	MedImmune <i>Gaithersburg, MD</i>	respiratory syncytial virus and parainfluenza virus type 3 infections	Phase I/II <i>2 months–23 months</i> (301) 398-0000
MEDI-559 (RSV vaccine)	MedImmune <i>Gaithersburg, MD</i>	respiratory syncytial virus infections	Phase I/II <i>5 months–23 months</i> (301) 398-0000
Menactra [®] meningococcal (groups A, C, Y and W-135) polysaccharide diphtheria toxoid conjugate vaccine	sanofi pasteur <i>Swiftwater, PA</i>	meningococcal disease	Phase III <i>infant–toddler</i> <i>(9–12 months)</i> (570) 839-7187
menACWY conjugate vaccine	Novartis Vaccines <i>East Hanover, NJ</i>	prevention of meningococcal group A, C, W-135, Y infections	application submitted <i>11 years and older</i> (888) 669-6682
		prevention of meningococcal group A, C, W-135, Y infections	Phase III <i>2 months–10 years</i> (888) 669-6682
meninge ACYW conjugate vaccine (2nd generation)	sanofi pasteur <i>Swiftwater, PA</i>	meningitis	Phase I <i>infants</i> (570) 839-7187
Pneumo (monovalent)	sanofi pasteur <i>Swiftwater, PA</i>	meningitis, pneumonia	Phase I <i>infants</i> (570) 839-7187
Prevnar 13 [™] 13-valent pneumococcal conjugate vaccine	Pfizer <i>New York, NY</i>	prevention of pneumococcal infections	application submitted <i>infants, children,</i> <i>adolescents</i> (860) 732-5156

VACCINES

Product Name	Sponsor	Indication	Development Status
Simplirix™ herpes simplex vaccine	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	prevention of herpes simplex virus infections	Phase III completed 10 years–17 years (888) 825-5249
V503	Merck <i>Whitehouse Station, NJ</i>	human papillomavirus infections (prevention of cervical and vulvovaginal cancers)	Phase III 9 years and older (800) 672-6372

OTHER

Product Name	Sponsor	Indication	Development Status
Analatro™ black widow spider antivenom (Orphan Drug)	Instituto Bioclon <i>Toriello Guerra, Mexico</i>	systemic latrodectism (spider bite pain)	Phase III 10 years and older www.bioclon.com
atacept	EMD Serono <i>Rockland, MA</i> ZymoGenetics <i>Seattle, WA</i>	systemic lupus erythematosus	Phase II/III 16 years and older (800) 283-8088 (800) 775-6686
Caldolor® ibuprofen intravenous	Cumberland Pharmaceuticals <i>Nashville, TN</i>	fever	Phase III up to 16 years (877) 484-2700
Emend® eprepitant	Merck <i>Whitehouse Station, NJ</i>	chemotherapy-induced and post-operative nausea and vomiting	Phase I 6 months–17 years (800) 672-6372
HPN-100	Hyperion Therapeutics <i>South San Francisco, CA</i>	urea cycle disorders	Phase II 6 years–17 years (888) 897-4276
IVIG3I 10%	Grifols USA <i>Los Angeles, CA</i>	idiopathic thrombocytopenic purpura	Phase III 3 years and older (888) 474-3657
liprotamase	Alnara Pharmaceuticals <i>Cambridge, MA</i>	exocrine pancreatic insufficiency	Phase III 7 years and older (617) 349-3690
Nplate® romiplostim	Amgen <i>Thousand Oaks, CA</i>	immune thrombocytopenic purpura	Phase III 1 year–17 years (800) 772-6436
omr-IgG-am IGIV	FFF Enterprises <i>Temecula, CA</i> OMRIX Biopharmaceuticals <i>Somerville, NJ</i>	immunologic deficiency syndromes	Phase III 3 years and older (800) 843-7477
OMS103HP	Omeros <i>Seattle, WA</i>	anterior cruciate ligament (ACL) reconstruction (knee)	Phase III 15 years and older (206) 676-5000
Precedex® dexmedetomidine	Hospira <i>Lake Forest, IL</i>	sedation	Phase II 2 years–16 years (800) 615-0187

OTHER

Product Name	Sponsor	Indication	Development Status
Promacta [®] eltrombopag	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	immune thrombocytopenic purpura	Phase II <i>1 years–17 years</i> (888) 825-5249
Recothrom [®] thrombin, topical (recombinant)	ZymoGenetics <i>Seattle, WA</i>	control bleeding during skin-graft surgery	Phase IV <i>up to 17 years</i> (206) 442-6600
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 (cyclic pelvic pain)	Phase III <i>up to 17 years</i> (610) 747-2600
Soliris [®] eculizumab	Alexion Pharmaceuticals <i>Cheshire, CT</i>	atypical hemolytic uremic syndrome	Phase II <i>12 years–18 years</i> (203) 272-2596
Stanate [®] stansoporphin	Infacare <i>Plymouth Meeting, PA</i>	hyperbilirubinemia	Phase II <i>neonates (up to 7 days)</i> (610) 260-1485
Toviaz [®] fesoterodine	Pfizer <i>New York, NY</i>	overactive bladder	Phase II <i>11 years–17 years</i> (860) 732-5156
Vascana [™] nitroglycerin topical	MediQuest Therapeutics <i>Bothell, WA</i>	treatment and prevention of Raynaud's disease	application submitted <i>15 years and older</i> (425) 398-9580
Venofer [®] iron sucrose injection	Luitpold Pharmaceuticals <i>Shirley, NY</i>	anemia in chronic kidney disease	Phase II <i>12 years–16 years</i> (631) 924-4000

The content of this report has been obtained through public government and industry sources, and the Adis “R&D Insight” database based on the latest information. **Report current as of January 11, 2010.** 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. The entire series of *Medicines in Development* is available on PhRMA's web site.

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acne vulgaris—The common form of acne seen most often in teenagers or young adults, which is the result of overactive oil glands that become plugged, red, and inflamed. Most outbreaks of acne can be treated by keeping the skin clear and avoiding irritating soaps, foods, drinks, and cosmetics. Severe acne and acne in those who are prone to scarring can be treated with topical creams and anti-inflammatory medications.

acute chest syndrome (ACS)—The second most common cause of hospitalization in sickle cell patients, accounting for more than 25 percent of premature deaths in sickle cell disease. ACS is unpredictable—it can come on quite suddenly and range from being very mild to fatal. Its main signs and symptoms include: fever, chest pain, cough, and trouble breathing.

angioedema—Swelling of the mucous membranes, tissues beneath the skin, or an internal organ due to an allergic reaction.

antithrombin (AT) deficiency—A genetic disorder leading to poorly functioning or diminished levels of antithrombin (an anticoagulant), a condition that can lead to excessive clotting. Historically called ATIII deficiency, AT deficiency is estimated to affect more than 200,000 people in Europe, Canada and the United States. It is one of a group of related conditions called thrombophilia. Individuals with AT deficiency are at risk for blood clots, organ damage or even death. Sometimes the clots can form spontaneously, putting an individual at sudden and unexpected risk. Patients are perhaps at greatest risk during events which are independently associated with a probability of thrombosis, such as surgery and delivery.

application submitted—An application for marketing has been submitted by the company to the

Food and Drug Administration (FDA).

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

atherosclerosis—A common disease in which deposits of plaque containing calcium and fatty substances, such as cholesterol, are formed within the inner layers of the arteries. It is a condition that progresses over decades, chiefly affecting the arteries of the heart, brain and extremities. Its complications include heart attacks and strokes.

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.

atypical hemolytic uremic syndrome (aHUS)—An extremely rare disease characterized by hemolytic anemia, low platelet count (thrombocytopenia), and acute renal

failure. (It is a distinctly different illness from hemolytic uremic syndrome caused by particular strains of the bacterium *E.coli*.) There is substantial evidence that aHUS is a genetic disorder. Atypical hemolytic uremic syndrome may become a chronic condition, and patients with aHUS may experience repeated attacks. Children with aHUS are much more likely to develop chronic serious complications such as kidney failure and severe high blood pressure.

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.

Becker muscular dystrophy (BMD)—One of nine types of muscular dystrophy, a group of genetic, degenerative diseases primarily affecting voluntary muscles. It’s caused by an insufficient production of dystrophin, a protein that helps keep muscle cells intact. Onset can occur

during adolescence or adulthood. Symptoms include generalized weakness and wasting, which first affects the muscles of the hips, pelvic area, thighs and shoulders. BMD is similar to Duchenne MD but often much less severe. The disease progresses slowly and with variability but can affect all voluntary muscles. BMD primarily affects boys and men, who inherit the disease through their mothers. Most with BMD survive well into mid- to late adulthood.

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

candidemia—A potentially life-threatening fungal infection and the most deadly of the common hospital-acquired bloodstream infections, with a mortality rate of approximately 40 percent. In the United States, an estimated 60,000 cases of candidemia occur each year.

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

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.

conjunctivitis—Swelling (inflammation) or infection of the membrane lining the eyelids (conjunctiva).

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.

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

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

de novo—A latin phrase meaning anew; afresh; beginning again; from the start; new; not present previously; just beginning.

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.

diphtheria—A bacterial infection characterized by mucus coating the membranes of the nose and throat, causing breathing problems, a fever, sore throat, vomiting, stomach pains, and chills.

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.

epilepsy—Recurrent seizures—transient neurological abnormalities caused by abnormal electrical activity in the brain—or temporary alteration in one or more brain functions. Seizures are a symptom of brain dysfunction and can result from a wide variety of diseases or injury.

Epstein-Barr virus (EBV)—A member of the herpes virus family and one of the most common human viruses. The virus occurs worldwide, and most people become infected with EBV sometime during their lives. When infection with EBV occurs during adolescence or young adulthood, it causes infectious mononucleosis up to 50 percent of the time.

esophagitis—Inflammation of the mucous membrane in the esopha-

gus, which can be a sometime side-effect of chemotherapy or caused by gastroesophageal reflux (GERD). Symptoms may include heartburn and painful or difficult swallowing.

exocrine pancreatic insufficiency (EPI)—The inability to properly digest food due to a lack of digestive enzymes made by the pancreas. EPI is found in humans afflicted with cystic fibrosis and Shwachman-Diamond syndrome. Chronic pancreatitis is the most common cause of EPI in humans. Loss of digestive enzymes leads to maldigestion and malabsorption of nutrients.

Fabry disease—A rare hereditary disorder that leads to an accumulation of glycolipid, a product of fat metabolism.

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

Friedreich's ataxia—An inherited disease that causes progressive damage to the nervous system

resulting in symptoms ranging from gait disturbance and speech problems to heart disease. "Ataxia," which refers to coordination problems such as clumsy or awkward movements and unsteadiness, occurs in many different diseases and conditions.

Gaucher disease—A chronic, progressive, inherited genetic disorder. People with Gaucher disease lack sufficient levels of a particular enzyme. As a result of this enzyme deficiency, a fatty material, or lipid, accumulates in the body. Lipid accumulation in organs and bones can cause mild to severe symptoms that can appear at any time throughout life, from infancy to adulthood.

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

Gram-positive bacteria—Gram's stain is a method of staining bacteria in order to identify them. Gram-positive bacteria stain violet.

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

head lice—See **pediculosis**.

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

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.

human papillomavirus (HPV)—Viral agent of genital warts, believed to be contagious and usually harmless, but some strains of HPV can lead to cervical cancer.

Hunter syndrome—A rare, life-threatening genetic condition, also called **mucopolysaccharides II (MPS II)**, that results from absence or insufficient levels of the lysosomal enzyme iduronate-2. Without this enzyme, cellular waste products accumulate in tissue and organs, which then begin to malfunction.

hyperbilirubinemia—Abnormally high amounts of bile pigment (bilirubin) in the blood causing jaundice. Most newborns have a rise in bilirubin in the first days following birth.

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.

hypereosinophilic syndrome—A condition in which the number of eosinophils (a type of white blood cell) in the blood is markedly and persistently elevated with no apparent cause, such as an allergic disease or a parasitic infection. These patients have either an inflammatory disease, the hypereosinophilic syndrome (HES), or chronic eosinophilic leukemia.

hyperoxaluria—An hereditary disorder that causes a special type of stone to form in the kidney and the urine beginning in childhood. Also known as oxalosis.

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

hypoxic—Refers to the lack of oxygen.

idiopathic—Of, relating to, or designating a disease having no known cause.

immune/idiopathic thrombocytopenia purpura—A condition that may follow a viral infection, which can trigger destruction of blood platelets by the immune system. The reduced number of platelets may result in abnormal bleeding into the skin and from other parts of the body (purpura).

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, 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.

lupus nephritis—Damaging inflammation of the kidneys that can occur in people with lupus. If not controlled, it may lead to total kidney failure. Lupus nephritis affects approximately 3 out of every 10,000 people. In children with lupus, about half will have some form or degree of kidney involvement. More than half of patients have not had other symptoms of lupus when they are diagnosed with lupus nephritis.

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 unsteadiness, 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).

meningococcal disease—Describes infections caused by the bacterium *Neisseria meningitidis* (also termed meningococcus). It carries a high mortality rate if untreated. While it is best known as a cause of meningitis, it also causes widespread blood infection (sepsis), which is more damaging and dangerous. Meningitis and meningococcal sepsis are major causes of illness, death, and disability in both developed and underdeveloped countries worldwide.

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

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.

MRI (magnetic resonance imaging)—A diagnostic technique that provides high quality crosssectional images of organs and structures within the body without x-rays or other radiation.

mucopolysaccharidoses II—See **Hunter syndrome**.

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

Neisseria meningitides—The gram-negative bacterium that causes meningococcal meningitis in humans, the only natural hosts in which it causes disease. Meningococci enter the nasal passage and may cause no symptoms (up to 30 percent of the population may harbor them between epidemics), or they may enter the bloodstream and produce the symptoms of meningitis.

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.

neurogenic detrusor overactivity—Urine voiding dysfunction secondary to neurologic injury or disease. The neurogenic detrusor overactivity syndrome, which may include urinary frequency, urgency, and incontinence, frequently contributes to a loss of independence or even institutionalization. It can occur in patients with primary diagnoses as diverse as Parkinson's disease, cerebral palsy, multiple sclerosis, spinal cord injury, and spina bifida.

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

nodular acne—A severe form of acne characterized by the presence of inflammation, nodular breakouts and cysts. It can be painful and often leaves scarring. A dermatologist is needed to treat this form of acne, often with both topical and systemic medications.

osteosarcoma—Cancer of the bone that occurs predominantly in adolescents and young adults. It accounts for 5 percent of cancer in children.

paraganglioma—A rare, usually benign tumor that develops from cells of the paraganglia, which is a collection of cells that came from embryonic nervous tissue and are found near the adrenal glands and some blood vessels and nerves.

parainfluenza virus 3—One in a group of four RNA viruses that rank second only to respiratory syncytial virus (RSV) as a common cause of lower respiratory tract disease in young children. Like RSV, human parainfluenza viruses (HPIVs) can cause repeated infections throughout life, which are usually manifested by an upper respiratory tract illness (such as a cold or sore throat). HPIVs can also cause

serious lower respiratory tract disease with repeat infection (including pneumonia, bronchitis, and bronchiolitis), especially among the elderly and patients with compromised immune systems. There are four serotypes of HPIV (1 through 4). Each of the four HPIVs has different clinical and epidemiologic features. HPIV-3 is more often associated with bronchiolitis and pneumonia.

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.

Pelizaeus-Merzbacher disease (PMD)—A rare, progressive, degenerative central nervous system disorder in which coordination, motor abilities, and intellectual function deteriorate. The disease is one of a group of gene-linked disorders known as the leukodystrophies, which affect growth of the myelin sheath—the fatty covering that wraps around and protects nerve fibers in the brain.

Phase I—Human clinical trials, usually 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.

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.

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.

pneumococcal infections—Caused by *Streptococcus pneumoniae*, or **pneumococcus**, a gram-positive human pathogenic bacterium. The organism causes many types of pneumococcal infections, including pneumonia, otitis media, meningitis, sepsis, endocarditis, and brain abscess. *S. pneumoniae* is the most common cause of bacterial meningitis in adults and children and one of the top two isolates found in ear infection (otitis media). Pneumococcal pneumonia is more common in the very young and the very old.

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.

primary immunodeficiency disease (PID)—An inherited disorder that affects some 50,000 people in the United States. The disorder requires regular immunoglobulin replacement therapy to prevent potentially serious or life-threatening infections.

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.

psoriasis—A common skin disease characterized by thickened patches of inflamed, red skin (plaques) often covered by silvery scales. About 80 percent of people who have psoriasis develop **plaque psoriasis**.

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

GLOSSARY

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.

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.

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.

staphylococcal sepsis—A severe bacterial infection involving the bloodstream, caused by *Staphylococcus aureus*, a common bacterium that is a frequent cause of hospital infections. Sepsis has a high mortality rate unless promptly treated with intravenous antibiotics.

stomatitis—Inflammation of the mucous lining of any of the structures in the mouth, which may involve the cheeks, gums, tongue,

lips, and roof or floor of the mouth. (The word “stomatitis” literally means inflammation of the mouth.) The inflammation can be caused by conditions in the mouth itself, such as poor oral hygiene or by conditions that affect the entire body, such as medications, allergic reactions, or infections. Stomatitis is usually a painful condition, associated with redness, swelling, and occasional bleeding from the affected area. It affects all age groups, from infants to the elderly.

streptococcal infections—Also called “strep,” which can cause a variety of health problems. There are two types: group A and group B. Antibiotics are used to treat both. Group A strep causes strep throat, scarlet fever, impetigo, toxic shock syndrome, and cellulitis and necrotizing fasciitis (the flesh-eating disease). Group B strep can cause blood infections, pneumonia and meningitis in newborns. Adults can also get group B strep infections, especially if they are elderly or already have health problems. Strep B can cause urinary tract infections, blood infections, skin infections and pneumonia in adults.

systemic lupus erythematosus (SLE)—The most serious form of lupus, a chronic autoimmune disorder causing inflammation and damage to multiple organs. SLE affects many systems of the body, including the joints, kidneys and brain.

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

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

thrombosis—The formation of a blood clot within the heart or a blood vessel.

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.

urea cycle disorders—Inborn errors in metabolism that can lead to brain damage and death. They involve a deficiency in one of the enzymes required by the urea cycle that removes ammonia from the blood. Ammonia accumulates in toxic levels if the urea cycle does not convert nitrogen from protein metabolism into urea for excretion into the urine. A series of biochemical reactions are necessary to complete the urea cycle. When an enzyme is missing or deficient, the cycle is interrupted and nitrogen accumulates in the form of ammonia. It cannot be excreted from the body and enters the blood stream, damaging nervous tissues, including the brain. Seizures, poor muscle tone, respiratory distress, and coma follow if an affected infant is not treated.

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

vitrectomy—A surgical procedure performed by a retinal specialist to remove the vitreous gel from the inside of the eye. Vitreous gel fills the inside cavity of the eye, taking up about two-thirds of the eye’s volume. Normally, the vitreous is clear and allows light to easily pass to the back of the eye. A vitrectomy is sometimes needed if bleeding occurs in the vitreal cavity, blocking clear vision. A vitrectomy is sometimes used to repair a retinal detachment or to remove fibrous tissue that sometimes forms as a part of diabetic retinopathy. A silicone oil or gas is injected into the eye to fill the space that the vitreous once occupied.

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Ten Leading Causes of Death in Children:

1-4 years (all causes 4,651)	5-14 years (all causes 6,091)	15-24 years (all causes 33,788)
1. Accidents (unintentional injuries).....(1,566)	1. Accidents (unintentional injuries).....2,157	1. Accidents (unintentional injuries).....(15,356)
2. Congenital malformations, deformations, and chromosomal abnormalities(506)	2. Cancer(929)	2. Assault (homicide)(5,284)
3. Assault (homicide)(365)	3. Congenital malformations, deformations, and chromosomal abnormalities(356)	3. Intentional self-harm (suicide)(4,030)
4. Cancer(361)	4. Assault (homicides)(337)	4. Cancer(1,609)
5. Diseases of heart.....(163)	5. Diseases of heart.....(209)	5. Diseases of heart.....(991)
6. Influenza and pneumonia.....(106)	6. Intentional self-harm (suicide)(195)	6. Congenital malformations, deformations, and chromosomal abnormalities(373)
7. Certain conditions originating in the perinatal period(77)	7. Influenza and pneumonia.....(111)	7. Cerebrovascular diseases.....(197)
8. Septicemia(74)	8. Chronic lower respiratory diseases(98)	8. Pregnancy, childbirth(166)
9. In situ neoplasms, benign neoplasms, and neoplasms of uncertain or unknown behavior.....(55)	9. Cerebrovascular diseases.....(85)	9. Septicemia(156)
10. Cerebrovascular diseases.....(52)	10. In situ neoplasms, benign neoplasms, and neoplasms of uncertain or unknown behavior.....(82)	10. Influenza and pneumonia.....(154)

Source: Xu J, Kochanek KD, Tejada-Vera B. *Deaths: Preliminary Data for 2007*. National Vital Statistics Reports. Vol. 58, No. 1. National Center for Health Statistics. 2009

Arthritis¹

- **Juvenile arthritis** is one of the most common childhood diseases in the United States. Some 294,000 children under the age of 18 are affected by pediatric arthritis and rheumatologic conditions, which result in an annual average of 827,000 ambulatory care visits.
- Arthritis and related conditions, such as **juvenile arthritis**, cost the U.S. economy nearly \$128 billion per year in medical care and indirect expenses, including lost wages and productivity.

Cancer²

- While childhood cancers are rare, representing less than 1 percent of all new cancer diagnoses, an estimated 10,730 new cases were expected to occur among children ages 0-14 in 2009. Some 1,380 deaths due to cancer were expected among that age group that year. Mortality rates for childhood cancer have declined by 50 percent since 1975, and the 5-year relative survival rate has increased to 80 percent.
- **Leukemia** accounts for about one-third of all childhood cancers and cancer deaths.
- Other childhood cancers include: **brain** and other **nervous system** (20.7 percent); **neuroblastoma** (6.9 percent); **Wilms tumor** (4.8 percent); **non-Hodgkin lymphoma** (4.3 percent) and **Hodgkin lymphoma** (3.6 percent); **rhabdomyosarcoma** (3.5 percent); **retinoblastoma** (2.7 percent); **osteosarcoma** (2.7 percent); and **Ewing sarcoma** (1.4 percent).

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Cardiovascular Disease

- In 2006, about 210,000 **cardiovascular** procedures were performed on children age 15 or younger.³
 - **Homozygous familial hypercholesterolemia**, a rare inherited disease of metabolism, occurs in fewer than one 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.⁴
 - About five of every 100 children have higher than normal blood pressure, although fewer than one in 100 has medically significant **hypertension**.⁵
-

Diabetes⁶

- Some 186,300 people under age 20 have **diabetes**, representing 0.22 percent of all people in that age group. About one in every 400 to 600 children and adolescents has type 1 diabetes.
 - The total economic cost of **diabetes** in 2007 was an estimated \$174 billion, or \$1 out of every \$10 spent on health care in the United States.
-

Eye Disorders

- **Conjunctivitis** is considered extremely common in the United States. Three percent of all ED visits are ocular related, and conjunctivitis is responsible for some 30 percent of all eye complaints. Approximately 15 percent of the population will have an allergic conjunctivitis episode at some time.⁷
 - Childhood **glaucoma** develops in 1 out of 10,000 children.⁸
 - More than 12.1 million school-age children, or one in four, have a **vision impairment**. Among preschool-age children, more than one in 20 has a vision problem that can cause permanent sight loss if left untreated. The most common types of eye problems seen in children are: myopia (nearsightedness), strabismus (crossed eyes), and lazy eye (amblyopia).⁹
-

Gastrointestinal Disorders

- Twenty percent of all cases of **Crohn's disease** affect children. Crohn's disease is seen in children as young as age 7. Males and females are affected equally. It appears to run in some families, with about 20 percent of people with Crohn's disease having a blood relative with some form of inflammatory bowel disease.¹⁰
 - **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**.¹¹
 - Some researchers believe as many as 7 million U.S. children have **gastroesophageal reflux disease (GERD)**.¹² Vomiting is the most common symptom of GERD in infants. During the first 3 months of life, recurrent vomiting occurs in 50 percent of infants. The incidence of recurrent vomiting peaks at age 4 months. Less than 5 percent of infants with GERD carry the problem into their childhood.¹³
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Genetic Diseases

- **Acute chest syndrome (ACS)** is the second most common cause of hospitalization in **sickle cell patients**, accounting for more than 25 percent of premature deaths in sickle cell disease. ACS is unpredictable—it can come on quite suddenly and range from being very mild to fatal. Its main signs and symptoms include: fever, chest pain, cough, and trouble breathing.¹⁴
- **Cystic fibrosis (CF)** affects approximately 30,000 children and adults (and 70,000 worldwide). About 1,000 new cases of CF are diagnosed each year. More than 70 percent of patients are diagnosed by age 2.¹⁵
- The median age of survival for a person with **CF** is more than 37 years. Today, more than 45 percent of the CF population is age 18 and older.¹⁵
- **Fragile X syndrome** is the most common inherited cause of mental impairment. The syndrome occurs in approximately 1 in 3,600 males and 1 in 4,000 to 6,000 females.¹⁶
- **Friedreich's ataxia**, an inherited disease that causes progressive damage to the nervous system, affects about 1 in every 50,000 people in the United States. Males and females are affected equally.¹⁷

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Genetic Diseases (continued)

- **Hereditary angioedema (HAE)** is a very rare and potentially life-threatening genetic condition that occurs in about 1 in 10,000 to 1 in 50,000 people. HAE symptoms include episodes of edema (swelling) in various body parts including the hands, feet, face, and airway. Before therapy became available, the mortality rate from airway obstruction was reportedly as high as 30 percent. A child has a 50 percent chance of inheriting this disease if one of his or her parents has it. Because the disease is very rare, it is not uncommon for patients to remain undiagnosed for many years. The age of HAE onset varies considerably. In one study, half of the patients reported onset of their symptoms by age 7, and more than two-thirds became symptomatic by age 13. There also seems to be an increased frequency of attacks during puberty or adolescence.¹⁸
- **Mucopolysaccharidosis type II (MPS II),** also called **Hunter syndrome,** is an inherited disorder. The syndrome is most often seen in males, although rare female cases have been reported. Hunter syndrome can occur in any ethnic group. It occurs in 1 in 100,000 to 1 in 150,000 males. Hunter syndrome is divided into two types. Type A is the severe form, which usually is diagnosed in children ages 18-36 months. It is considered the classic form. Symptoms in type A may include short stature, progressive and profound mental retardation, skin lesions, skeletal changes, deafness, and visual impairment. Children with type A may survive into the second and third decades of life. Type B Hunter syndrome is much milder than type A and may not be diagnosed until adulthood. Individuals with type B may live into their 70s.¹⁹
- **Sickle cell disease** affects millions of people throughout the world and is particularly common among those whose ancestors come from sub-Saharan Africa, Spanish-speaking regions in the Western Hemisphere, Saudi Arabia, India, and some Mediterranean countries. In the United States, more than 70,000 people have sickle cell disease, which occurs in 1 in every 500 African-American births. Some 2 million people have sickle cell trait, while 1 in 12 African Americans has the trait. From 1989 through 1993, sickle cell disease caused an average of 75,000 hospitalizations, costing the United States approximately \$475 million.²⁰

Infectious Diseases/Vaccines

AIDS Cases through December 2007²⁰

9,209 (<age 13); 7,258 (13-19 years)

AIDS Deaths through December 2007²⁰

4,891 (<age 13); 1,435 (13-19 years)

- In 2006, people ages 13-29 accounted for the largest number (19,200) of new **HIV infections,** or 34 percent of new cases.²⁰
- From 2004 through 2007, the estimated number of newly diagnosed **HIV/AIDS cases** decreased among children under age 13, remained stable among those ages 13-14, and increased among those ages 15-19.²⁰
- **Chickenpox,** caused by the **varicella zoster virus,** was until recently one of the most common childhood diseases. Before there was a vaccine, about 4 million cases a year occurred in the United States. Chickenpox is usually mild, but it occasionally causes serious problems. Among infants under age 1 who get the disease, about 1 in 250,000 dies. For older children, about 1 in 100,000 dies. If a woman gets chickenpox just before or after giving birth, her baby can get very sick, and about 1 in 3 of those babies will die if not treated quickly. About 1 child in 500 who contracts chickenpox is hospitalized.²⁰
- In 2005, more than 25,000 cases of **pertussis** were reported. Even though that number is down considerably from the estimated 150,000 cases that occurred annually before a vaccine was introduced, pertussis still is one of the most common vaccine-preventable childhood diseases in the United States. About 1 child in 10 who gets pertussis also gets pneumonia, and about 1 in 50 will have convulsions. The brain is affected in about 1 person out of 250. Pertussis causes about 10–20 deaths each year in this country.²⁰
- *Streptococcus pneumoniae* bacteria cause a range of **pneumococcal infections** throughout life, especially affecting children under age 2 and the elderly. The World Health Organization (WHO) estimates that more than 1.6 million people—including more than 800,000 children under age 5—die every year from pneumococcal infections. Pneumococcal meningitis is the most severe form of pneumococcal disease and one of the most fatal childhood illnesses. In developing countries, it kills or disables up to 70 percent of children who get it. Since 2000, when U.S. infants began receiving routine vaccination against pneumococcal disease, the country has nearly eliminated childhood pneumococcal disease.²¹
- **Respiratory syncytial virus (RSV)** is the most common cause of severe lower respiratory tract disease among infants and children under age 2. It affects boys 1.7 times more often than girls. An estimated 75,000 to 125,000 children are hospitalized due to RSV annually and approximately 0.2 percent to 7 percent of them die each year.²²

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Infectious Diseases/Vaccines (continued)

- The annual cost of medical care for treating **infectious diseases** in the United States alone is estimated to exceed \$120 billion.²¹
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Neurologic Disorders

- 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 15 have epilepsy. Epilepsy and seizures affect almost 3 million Americans of all ages, at an estimated annual cost of \$15.5 billion in direct and indirect costs.²³
 - **Duchenne muscular dystrophy (DMD)** is the most severe and also the most common form of muscular dystrophy. It is more commonly found in boys at a rate of 1 in 3,500. DMD causes progressive weakness in the skeletal muscles, and most DMD children require a wheelchair by age 11. DMD later results in respiratory muscle and heart muscle failure, eventually leading to death sometime between the teenage years and early 30s.²⁴
 - 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
 - As many as 5 percent of children in grade school have **migraine headaches**.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
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Psychiatric Disorders

- Research shows that half of all lifetime cases of **mental illness** begin by age 14.²⁷ Studies indicate that 1 in 5 children and adolescents (20 percent) may have a diagnosable mental disorder. Estimates of the number of children who have mental disorders range from 7.7 million to 12.8 million. An estimated two-thirds of all young people with mental health problems are not getting the help they need.²⁸
- Approximately 50 percent of students with a **mental health condition** age 14 or older drop out of school, the highest dropout rate for any disability group. Seventy percent of youth involved in state and local juvenile justice systems suffer from mental health conditions.²⁸
- **Autism** is diagnosed in 1 out of 150 children today making autism more prevalent than childhood cancers, multiple sclerosis, and cystic fibrosis combined.²⁹ Autism spectrum disorders (ASDs) occur in all racial, ethnic, and socioeconomic groups, but are four times more likely to occur in boys than in girls. If 4 million children are born in the United States every year, approximately 26,670 children will eventually be diagnosed with an ASD. Assuming the prevalence rate has been constant over the past two decades, an estimated 560,000 individuals between the ages of 0 to 21 have an ASD. The estimated lifetime cost to care for an individual with an ASD is \$3.2 million.²⁰
- **Attention-deficit/hyperactivity disorder (ADHD)** is one of the most common reasons children are referred for mental health services. It affects as many as one in every 20 children. Although boys are three to four times more likely than girls to experience ADHD, the disorder affects both genders. Although most children with ADHD have normal or above-normal intelligence, 40 to 60 percent have serious learning difficulties. Children and adolescents with ADHD are more likely than children without the disorder to suffer from other mental disorders. About one-half of all young people with ADHD have oppositional defiant disorder; about one-quarter have an **anxiety disorder**; as many as one-third have **depression**; and one-fifth have **bipolar disorder**. Nine out of 10 children respond to medication, and 50 percent of children who do not respond to an initial medication will respond to a second.²⁸
- At any given time, as many as one in every 33 children may have clinical **depression**. The rate of depression among adolescents may be as high as one in eight. Recent studies have shown that more than 20 percent of adolescents in the general population have emotional problems, and one-third of adolescents attending psychiatry clinics suffer from depression. About 8 percent of youth ages 12 to 17 experienced at least one major depressive episode in the past year. Almost one-third of children ages 6 to 12 who have been diagnosed with major depression will develop bipolar disorder within a few years. Most teenagers with major depression never receive treatment.²⁸

SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

Psychiatric Disorders (continued)

- **Schizophrenia** is rare in children under 12, but it occurs in about 3 out of every 1,000 adolescents.²⁸
 - The current cost of treating children and adolescents for **mental health conditions** is estimated at nearly \$12 billion. Most of the money is spent on outpatient rather than inpatient care. Hispanic young people are the least likely of all groups to access specialty care, even though they and African-American children have the highest rates of need. Around 7 percent of all families cite financial barriers for not getting their troubled youth the mental health care they need.³⁰
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Respiratory Disorders²²

- An estimated 6.7 million children under age 18 have **asthma**, the most common chronic disorder in childhood, and 3.8 million suffered from an asthma attack or episode in 2007.
 - **Asthma** is the third leading cause of hospitalization among children under age 15. And in 2005, asthma resulted in approximately 679,000 emergency room visits by children in that age group.
 - In 2005, 3,884 deaths were attributed to **asthma**. However, deaths due to asthma are rare among children. In 2005, 138 children under age 15 died from asthma compared to 740 adults over 85. [lungusa.org]
 - The annual total cost of **asthma** to the U.S. economy is \$19.7 billion—about \$14.7 billion in direct health-care costs and \$5 billion in indirect costs (e.g., lost productivity).
 - **Bronchopulmonary dysplasia (BPD)** is a neonatal chronic lung disease that predominately develops in premature infants weighing less than 1,500 grams 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 12,000 new cases of BPD occur each year. White, male infants seem to be at greater risk for developing BPD.
 - **Respiratory syncytial virus (RSV)** is most common in infants and young children and is the most common cause of severe lower respiratory tract disease among infants and children under age 2. RSV affects boys 1.7 times more often than girls. Annually, an estimated 75,000-125,000 children are hospitalized due to RSV, and approximately 0.2 percent to 7 percent die each year.
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Skin Disorders

- **Atopic dermatitis** is the most severe and chronic kind of **eczema**. It almost always begins in childhood, usually during infancy. An estimated 65 percent of eczema patients are diagnosed in the first year of life, and 90 percent of patients experience it before age 5. It is estimated that atopic dermatitis affects more than 30 million Americans.³¹
 - **Psoriasis** affects as many as 7.5 million people in the United States, about 2.6 percent of the population. It occurs nearly equally in men and women across all socioeconomic groups, and it occurs in all races, though Caucasians are slightly more affected. Every year, some 20,000 children under age 10 are diagnosed with psoriasis. People usually have their first outbreak between the ages of 15 and 35, but it can appear at any age.³²
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Transplantation

- The total number of pediatric candidates on the U.S. **transplantation** waiting list increased steadily from 1,739 in 1997 to a peak of 2,340 in 2001 and has since leveled, with 2,124 candidates in 2006. Distribution by age of pediatric candidates on the waiting list has remained consistent, averaging 6.6 percent for those under age 1, 26.9 percent for ages 1-5, 19.8 percent for ages 6-10, and 46.7 percent for ages 11-17. The overall percentage of pediatric candidates on the waiting list has declined somewhat, from 3.3% in 1997 to 2.3% in 2006.³³
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SELECTED FACTS ABOUT CHILDREN AND DISEASE IN THE UNITED STATES

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30. Rand Corporation (www.rand.org)
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THE DRUG DISCOVERY, DEVELOPMENT AND APPROVAL PROCESS

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

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

THE DRUG DEVELOPMENT AND APPROVAL PROCESS

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

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

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

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

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

Investigational New Drug Application (IND). After completing preclinical testing, a company files an IND with the U.S. Food and Drug Administration (FDA) to begin to test the drug in people. The IND 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 24 new therapeutics approved by the FDA in 2008 was 17.8 months.

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

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

TRACKING THE PHARMACEUTICAL AND BIOTECHNOLOGY RESEARCH PIPELINES

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

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

- **Cancer**—There are few things that cause patients more fear and uncertainty as a cancer diagnosis. Yet today—because of a steady stream of new and improved medicines and treatments—cancer can increasingly be managed and even beaten. *The 2009 report found 861 medicines in the pipeline.*
- **Heart Disease and Stroke**—Keeping up the momentum of drug discovery that has helped cut deaths from heart disease and stroke in half in the past three decades, biopharmaceutical companies are working on new medicines for these diseases. *The 2009 report found 312 medicines in the pipeline.*
- **Diabetes**—Approximately 4,110 people are diagnosed with diabetes every day. To help fight this disease, pharmaceutical and biotechnology researchers are working on new medicines to treat it and related conditions. *The 2009 report found 183 medicines in the pipeline.*
- **Mental Illnesses**—Pharmaceutical and biotechnology researchers are testing many new medicines to help the more than 450 million people worldwide who suffer from some form of mental illness. *The 2008 report found 301 medicines in the pipeline.*
- **Biotechnology**—Millions of people have already benefited from medicines and vaccines developed through biotechnology, and a new report offers hope that many more will benefit in the future. *The 2008 report found 633 medicines in the pipeline.*
- **Children**—Pharmaceutical researchers are testing medicines to meet the special needs of children. These medicines offer hope that the significant improvements achieved in children's health over the past few decades will continue and even accelerate. *The 2010 report found 234 medicines in clinical trials.*
- **Older Americans**—The population of Americans over 65 is surging, and the pace will only increase over the coming years. As life expectancy continues to expand, older Americans face new and growing challenges to their health, productivity and independence. *The 2008 report found 1,026 medicines in the pipeline.*



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