

Skin Diseases

PRESENTED BY AMERICA'S BIOPHARMACEUTICAL RESEARCH COMPANIES

Biopharmaceutical Research Companies Are Developing Nearly 300 Medicines to Treat Diseases of the Skin

America's biopharmaceutical research companies are currently developing 277 medicines to help the more than 100 million Americans—one-third of the U.S. population—afflicted with at least one skin disease, ranging from acne to melanoma. All of the medicines are either in clinical trials or awaiting review by the U.S. Food and Drug Administration.

Skin is the largest organ of the human body and, along with hair and nails, protects the body. It prevents germs from entering the body and damaging internal organs, supports all other body parts, and plays a role in maintaining the immune system. The skin also helps to regulate body temperature through the sweat glands and is responsible for the sense of touch.

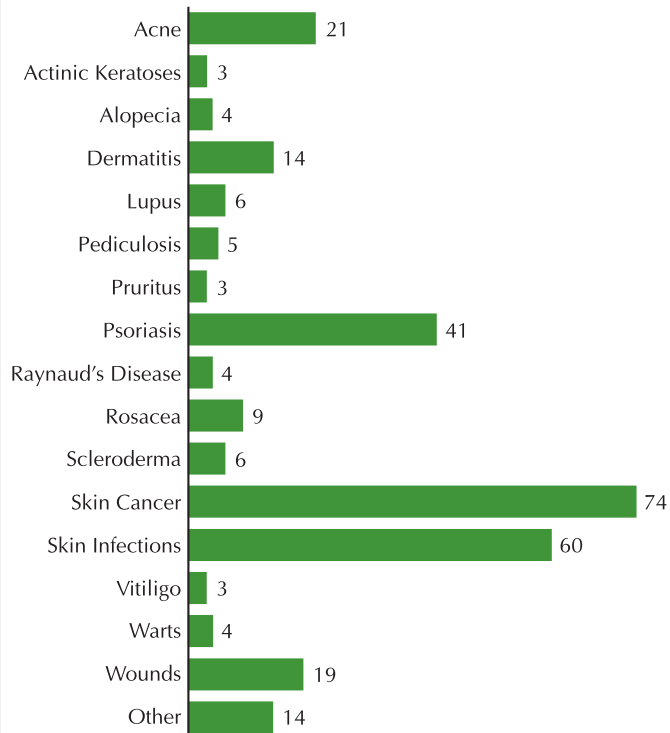
The new medicines today in the research and development pipeline offer hope for reducing the human and economic costs of the many skin diseases affecting Americans. They include:

- 74 for skin cancers, including 63 for melanoma, which affects more than 68,000 Americans each year.
- 60 for skin and soft tissue infections, which account for nearly 14 million outpatient visits each year.
- 41 for psoriasis, which affects about 7.5 million people in the United States. Most people, about 80 percent, have plaque psoriasis.
- 14 for dermatitis (eczema), which affects 90 percent of sufferers before the age of five. Worldwide, 10 percent to 20 percent of children have dermatitis.
- 9 for rosacea, which affects more than 14 million Americans.

Examples of some medicines now being tested to treat skin diseases include:

- A recombinant protein in development for skin cancer that is designed to trigger an immune response against specific tumor cells.
- A first-in-class medicine for drug-resistant skin infections with broad-spectrum antibacterial activity.
- A first-in-class medicine in development for psoriasis that inhibits an enzyme that plays a role in T-cell activation, an early step in autoimmune diseases.

MEDICINES IN DEVELOPMENT FOR SKIN DISEASES*



* Some medicines are listed in more than one category.

Researching and developing new medicines remains a risky investment and lengthy process—costing, on average, \$1.2 billion and taking between 10-15 years to bring a new medicine to patients. Advances in our understanding of diseases and how to treat them have allowed America's pharmaceutical research and biotechnology companies to conduct the cutting-edge research needed to reduce the destructive toll of skin diseases and to allow more patients to lead healthier, happier, more productive lives.

John J. Castellani
President and CEO
PhRMA

Medicines in Development for Skin Diseases

ACNE

Product Name	Company	Indication	Development Status*
ACAT inhibitor	Graceway Pharmaceuticals <i>Bristol, TN</i>	acne	Phase II (800) 328-0255
ANT-1207 (botulinum toxin A)	Anterios <i>New York, NY</i>	acne vulgaris	Phase II (212) 303-1683
ASC-J9	AndroScience <i>San Diego, CA</i>	acne vulgaris	Phase II (858) 638-7230
AUS-131/ doxycycline	Nexgen Dermatologics <i>Boynton Beach, FL</i>	acne vulgaris	Phase I/II
BLI-1100	Braintree Laboratories <i>Braintree, MA</i>	acne vulgaris	Phase II (800) 874-6756
CD-07223	Galderma R&D <i>Princeton, NJ</i>	acne	Phase II (609) 409-7701
CD-2475-101	Galderma Laboratories <i>Fort Worth, TX</i>	acne vulgaris	Phase I www.galdermausa.com
clindamycin/ tretinoin topical combination	Skinvisible Pharmaceuticals <i>Las Vegas, CA</i>	acne	in clinical trials (702) 433-7154
COL-177	Onset Dermatologics <i>Cumberland, RI</i>	acne vulgaris	in clinical trials (888) 713-8154
Duac® Topical Gel low-dose clindamycin/ benzoyl peroxide gel	Stiefel, a GSK company <i>Rsch. Triangle Park, NC</i>	acne vulgaris	application submitted (888) 825-5249
E-0301	Elorac <i>Vernon Hills, IL</i>	acne (see also rosacea)	Phase III (847) 362-8200
IDP 107	Valeant Pharmaceuticals International <i>Irvine, CA</i>	acne vulgaris	Phase II (800) 548-5100
isotretinoin	Cipher Pharmaceuticals <i>Mississauga, Canada</i>	acne	Phase III (905) 565-0043
JNJ-10229570	Johnson & Johnson Consumer & Personal Products <i>Skillman, NJ</i>	acne vulgaris	Phase II (800) 817-5286
Metvixia® methyl aminolevulinate	PhotoCure <i>Oslo, Norway</i>	acne (see also skin cancer)	Phase II www.photocure.com
NB-003 (nanoemulsion)	NanoBio <i>Ann Arbor, MI</i>	acne	Phase I (734) 302-4000
NVC-422	Galderma <i>Princeton, NJ</i> NovaBay Pharmaceuticals <i>Emeryville, CA</i>	acne	Phase I completed (609) 409-7701 (510) 899-8800

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

ACNE

Product Name	Company	Indication	Development Status
tazarotene foam (retinoid foam)	Stiefel, a GSK company <i>Rsch. Triangle Park, NC</i>	acne vulgaris	Phase III (888) 825-5249
tretinoin topical	Phosphagenics <i>Melbourne, Australia</i>	acne	Phase I www.phosphagenics.com
WC-3018	Warner Chilcott <i>Rockaway, NJ</i>	acne (see also skin infections)	Phase II (973) 442-3200
WC-3035	Warner Chilcott <i>Rockaway, NJ</i>	acne (see also rosacea)	Phase I (973) 442-3200

ACTINIC KERATOSES

Product Name	Company	Indication	Development Status
E-0611	Elorac <i>Vernon Hills, IL</i>	actinic keratosis	Phase III (847) 362-8200
PEP005 (ingenol mebutate)	LEO Pharma <i>Parsippany, NJ</i>	actinic keratosis (see also skin cancer)	application submitted (877) 494-4536
Veregen® sinecatechins	PharmaDerm <i>Florham Park, NJ</i>	actinic keratoses	Phase II (800) 645-9833

ALOPECIA

Product Name	Company	Indication	Development Status
bimatoprost topical	Allergan <i>Irvine, CA</i>	alopecia	Phase I (800) 433-8871
norepinephrine topical	ProCertus BioPharm <i>Madison, WI</i>	prevention of alopecia (see also other)	Phase I (608) 277-7950
NP-619	North Park Aesthetics <i>San Diego, CA</i>	alopecia	Phase II
PF-277343	Pfizer <i>New York, NY</i>	alopecia	Phase I (732) 860-5156

DERMATITIS

Product Name	Company	Indication	Development Status
alitretinoin oral (BAL4079)	Basilea Pharmaceutica <i>Basel, Switzerland</i>	chronic eczema	Phase III www.basilea.com
AMG 157	Amgen <i>Thousand Oaks, CA</i>	atopic dermatitis	Phase I (800) 772-6436
AN2728	Anacor Pharmaceuticals <i>Palo Alto, CA</i>	atopic dermatitis (see also psoriasis)	Phase I (650) 543-7500
AN2898	Anacor Pharmaceuticals <i>Palo Alto, CA</i>	atopic dermatitis (see also psoriasis)	Phase I (650) 543-7500
Elidel® pimecrolimus topical	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	seborrheic dermatitis (see also vitiligo)	Phase I/II (888) 669-6682

DERMATITIS

Product Name	Company	Indication	Development Status
GSK870086 (topical novel glucocorticoid agonist)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	atopic dermatitis	Phase II (888) 825-5249
KP-413 (topical ointment)	Kaken Pharmaceutical <i>New York, NY</i>	atopic dermatitis	Phase I/II (212) 372-8910
mapracorat	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i>	atopic dermatitis	Phase II (888) 842-2937
NF-kappa-B decoy oligonucleotide	Transcription Factor Therapeutics <i>Irvine, CA</i>	atopic dermatitis	Phase I/II
PH-10	Provectus Pharmaceuticals <i>Knoxville, TN</i>	atopic dermatitis (see also psoriasis)	Phase II completed (866) 594-5999
SAR231893 (anti-IL4 mAb)	Regeneron Pharmaceuticals <i>Tarrytown, NY</i> sanofi-aventis <i>Bridgewater, NJ</i>	atopic dermatitis	Phase I (877) 734-6777 (800) 633-1810
SAR302532 (REGN846)	Regeneron Pharmaceuticals <i>Tarrytown, NY</i> sanofi-aventis <i>Bridgewater, NJ</i>	atopic dermatitis	Phase I (877) 734-6777 (800) 633-1810
SMT-D002	Summit <i>Oxford, United Kingdom</i>	seborrhoeic dermatitis	Phase I www.summitplc.com
SUN-13834	Asubio Pharmaceuticals <i>Rochelle Park, NJ</i>	atopic dermatitis	Phase II (201) 368-5020

LUPUS, CUTANEOUS

Product Name	Company	Indication	Development Status
AMG 811	Amgen <i>Thousand Oaks, CA</i>	discoid lupus erythematosus	Phase I (800) 772-6436
CC-11050	Celgene <i>Summit, NJ</i>	cutaneous lupus erythematosus	Phase I (908) 673-9000
DV-1179	Dynavax Technologies <i>Berkeley, CA</i>	cutaneous lupus erythematosus	Phase I (877) 848-5100
JNK-930	Celgene <i>Summit, NJ</i>	discoid lupus erythematosus	Phase II (908) 673-9000
PF-04236921	Pfizer <i>New York, NY</i>	lupus vulgaris	Phase I (860) 732-5156
TRX1 antibody (RG7424)	Genentech <i>South San Francisco, CA</i> Tolerx <i>Cambridge, MA</i>	cutaneous lupus erythematosus	Phase I completed (800) 626-3553 (617) 354-8100

PEDICULOSIS (HEAD LICE)

Product Name	Company	Indication	Development Status
BGC-200582	BTG International <i>West Conshohocken, PA</i>	pediculosis	Phase II (610) 278-1660
DeOvo™ pediculosis therapy	Hatchtech <i>Melbourne, Australia</i>	pediculosis	Phase II www.hatchtech.com.au
ivermectin topical	Topaz Pharmaceuticals <i>Horsham, PA</i>	pediculosis	Phase III (267) 960-3330
malathion gel	Taro Pharmaceuticals U.S.A. <i>Hawthorne, NY</i>	pediculosis	Phase III (800) 544-1449
RESULTZ® pediculosis therapy	Piedmont Pharmaceuticals <i>Greensboro, NC</i>	pediculosis	Phase III (336) 544-0320

PRURITUS

Product Name	Company	Indication	Development Status
CR845	Cara Therapeutics <i>Shelton, CT</i>	pruritus	Phase II (203) 567-1500
E-3016 (naloxone topical)	Elorac <i>Vernon Hills, IL</i>	pruritus	Phase II (847) 362-8200
GSK705498 (topical TRPV1 antagonist)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	pruritus	Phase I (888) 825-5249

PSORIASIS

Product Name	Company	Indication	Development Status
AbGn-168	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	plaque psoriasis	Phase I (800) 243-0127
Amevive® alefacept	Astellas Pharma US <i>Deerfield, IL</i>	psoriasis in adolescents	Phase II (800) 727-7003
AMG 139	Amgen <i>Thousand Oaks, CA</i>	psoriasis	Phase I (800) 772-6436
AMG 827 (brodalumab)	Amgen <i>Thousand Oaks, CA</i>	psoriasis	Phase II (800) 772-6436
aminopterin	Syntrix Biosystems <i>Auburn, WA</i>	psoriasis	Phase II (253) 833-8009
AN2728	Anacor Pharmaceuticals <i>Palo Alto, CA</i>	psoriasis (see also dermatitis)	Phase II (650) 543-7500
AN2898	Anacor Pharmaceuticals <i>Palo Alto, CA</i>	psoriasis (see also dermatitis)	Phase I (650) 543-7500
apremilast	Celgene <i>Summit, NJ</i>	psoriasis	Phase III (908) 673-9000
ASP-015K	Astellas Pharma US <i>Deerfield, IL</i>	plaque psoriasis	Phase II (800) 727-7003

PSORIASIS

Product Name	Company	Indication	Development Status
BFH772 (betamethasone/ calcipotriol)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	psoriasis	Phase I/II completed (888) 669-6682
CCX-832	ChemoCentryx <i>Mountain View, CA</i> GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	psoriasis	Phase I (650) 210-2900 (888) 825-5249
CF-101	Can-Fite BioPharma <i>Waltham, MA</i>	psoriasis	Phase II (978) 456-9975
Cimzia [®] certolizumab pegol	UCB <i>Smyrna, GA</i>	psoriatic arthritis	Phase III (770) 970-7500
CM2489	CalciMedica <i>La Jolla, CA</i>	plaque psoriasis	Phase I (858) 952-5500
CNTO-1959	Centocor Ortho Biotech <i>Horsham, PA</i>	psoriasis	Phase I (800) 457-6399
CT327	Creabilis Therapeutics <i>Luxembourg, Luxembourg</i>	plaque psoriasis	Phase II completed www.creabilis-sa.com
desoximetasone topical spray	Taro Pharmaceuticals U.S.A. <i>Hawthorne, NY</i>	plaque psoriasis	Phase III (800) 544-1449
E-0116	Elorac <i>Vernon Hills, IL</i>	psoriasis	Phase III (847) 362-8200
Enbrel [®] etanercept	Amgen <i>Thousand Oaks, CA</i>	plaque psoriasis in adolescents and children	application submitted (800) 772-6436
fezakinumab (ILV-094)	Pfizer <i>New York, NY</i>	psoriasis	Phase I (860) 732-5156
GSK2245840 (SIRT1 activator)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	plaque psoriasis	Phase II (888) 825-5249
IDP 118	Valeant Pharmaceuticals International <i>Irvine, CA</i>	psoriasis	Phase I (800) 548-5100
INCB18424 (topical formulation)	Incyte <i>Wilmington, DE</i>	psoriasis	Phase II completed (302) 498-6700
LY2439821 (ixekizumab)	Eli Lilly <i>Indianapolis, IN</i>	psoriasis	Phase II (800) 545-5979
M-518101	Maruho <i>New York, NY</i>	plaque psoriasis	Phase II www.maruho.co.jp
MK-0873	Merck <i>Whitehouse Station, NJ</i>	psoriasis	Phase I (800) 672-6372
otelixizumab	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i> Tolerx <i>Cambridge, MA</i>	psoriasis	Phase I completed (888) 825-5249 (617) 354-8100
PF-05212368 (ILV-095)	Pfizer <i>New York, NY</i>	psoriasis	Phase I (860) 732-5156

PSORIASIS

Product Name	Company	Indication	Development Status
PH-10	Provectus Pharmaceuticals <i>Knoxville, TN</i>	psoriasis (see also dermatitis)	Phase II (866) 594-5999
RG4934 (IL-17 antagonist mAb)	Roche <i>Nutley, NJ</i>	psoriatic arthritis	Phase I (973) 235-5000
RO5310074	Roche <i>Nutley, NJ</i>	psoriatic arthritis	Phase I (973) 235-5000
SCH900222	Merck <i>Whitehouse Station, NJ</i>	plaque psoriasis	Phase II (800) 672-6372
secukinumab	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	plaque psoriasis, psoriatic arthritis	Phase II (888) 669-6682
SGN-70	Seattle Genetics <i>Bothell, WA</i>	plaque psoriasis	Phase I (425) 527-4000
sotrastaurin	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	psoriasis	Phase II (888) 669-6682
Stelara [®] ustekinumab	Centocor Ortho Biotech <i>Horsham, PA</i>	palmoplantar pustulosis, psoriatic arthritis	Phase III (800) 457-6399
Taclonex [®] calcipotriol/ betamethasone dipropionate	LEO Pharma <i>Parsippany, NJ</i>	psoriasis vulgaris on non-scalp regions of the body	Phase II (877) 494-4536
		psoriasis in adolescents	Phase II (877) 494-4536
tofacitinib	Pfizer <i>New York, NY</i>	plaque psoriasis (oral formulation)	Phase III (860) 732-5156
		plaque psoriasis (topical formulation)	Phase II (860) 732-5156
		psoriatic arthritis (oral formulation)	Phase II (860) 732-5156
topical methotrexate	MediQuest Therapeutics <i>Bothell, WA</i>	psoriasis	Phase II (425) 398-9580
ulobetasol propionate lotion	Therapeutics <i>San Diego, CA</i>	plaque psoriasis	Phase II (858) 571-1800
VB-201	VBL Therapeutics <i>Or Yehuda, Israel</i>	psoriasis	Phase II www.vblrx.com

RAYNAUD'S DISEASE

Product Name	Company	Indication	Development Status
MQX-503 (nitroglycerin topical)	MediQuest Therapeutics <i>Bothell, WA</i>	Raynaud's disease	Phase III (425) 398-9580
PF-00489791	Pfizer <i>New York, NY</i>	Raynaud's disease	Phase II (732) 860-5156

RAYNAUD'S DISEASE

Product Name	Company	Indication	Development Status
Revatio® sildenafil	Pfizer New York, NY	Raynaud's disease secondary to cutaneous systemic sclerosis	in clinical trials (732) 860-5156
SLX-2101	Kadmon Pharmaceuticals New York, NY Nano Terra Brighton, MA	Raynaud's disease	Phase II completed (212) 600-1902 (617) 621-8500

ROSACEA

Product Name	Company	Indication	Development Status
azelaic acid (foam formulation)	Intendis Morristown, NJ	rosacea	Phase II (866) 463-3634
CLS001	Cutanea Life Sciences Wayne, PA	rosacea	Phase II completed (484) 568-0100
COL-118	Galderma R&D Princeton, NJ	moderate to severe facial erythema associated with rosacea	Phase III (609) 409-7701
E-0301	Elorac Vernon Hills, IL	rosacea (see also acne)	Phase II (847) 362-8200
IDP 115	Valeant Pharmaceuticals International Irvine, CA	rosacea	Phase III (800) 548-5100
licochalcone A	Beiersdorf Research Center Hamburg, Germany	rosacea (see also other)	Phase I www.beiersdorf.com
matrix metalloproteinase inhibitor	Quick-Med Technologies Gainesville, FL	rosacea	in clinical trials (888) 835-2211
V-101	Vicept Therapeutics Malvern, PA	rosacea	Phase II completed (484) 324-2930
WC-3035	Warner Chilcott Rockaway, NJ	rosacea (see also acne)	Phase I (973) 442-3200

SCLERODERMA

Product Name	Company	Indication	Development Status
ARG201 (Orphan Drug)	arGentis Pharmaceuticals Memphis, TN	systemic scleroderma	Phase II (901) 552-4730
CC-10015	Celgene Summit, NJ	systemic scleroderma	Phase I (908) 673-9000
Gleevec® imatinib	Novartis Pharmaceuticals East Hanover, NJ	scleroderma	Phase II completed (888) 669-6682
MEDI-546 (anti-IFNalphaR mAb)	AstraZeneca Wilmington, DE Medarex Princeton, NJ MedImmune Gaithersburg, MD	scleroderma	Phase I (800) 236-9933 (609) 430-2880 (301) 298-0000

SCLERODERMA

Product Name	Company	Indication	Development Status
MEDI-551 (anti-CD19 mAb)	AstraZeneca <i>Wilmington, DE</i> MedImmune <i>Gaithersburg, MD</i>	scleroderma	Phase I (800) 236-9933 (301) 298-0000
treprostinil	United Therapeutics <i>Silver Spring, MD</i>	systemic scleroderma	Phase II (301) 608-9292

SKIN CANCER

Product Name	Company	Indication	Development Status
Abraxane® albumin-bound paclitaxel (Orphan Drug)	Celgene <i>Summit, NJ</i>	malignant melanoma	Phase III (908) 673-9000
ABT-888 (veliparib)	Abbott Laboratories <i>Abbott Park, IL</i>	malignant melanoma	Phase II (847) 937-6100
AD-1001	Intrexon <i>Germantown, MD</i> ZIOPHARM Oncology <i>New York, NY</i>	late-stage malignant melanoma	Phase I (646) 214-0700
ADI-PEG 20 (Orphan Drug)	Polaris Pharmaceuticals <i>San Diego, CA</i>	malignant melanoma	Phase II (858) 452-6688
Allovetin-7® velimogene aliplasimid (immunotherapeutic vaccine) (Orphan Drug)	Vical <i>San Diego, CA</i>	malignant melanoma (Fast Track)	Phase III (858) 646-1100
ALT-801	Altor BioScience <i>Miramar, FL</i>	malignant melanoma	Phase I/II (954) 443-8600
Amplimexon® imexon	AmpliMed <i>Tucson, AZ</i>	melanoma	Phase I/II completed (520) 529-1000
APN301 (hu14.18-IL2)	Apeiron Biologics <i>Vienna, Austria</i>	malignant melanoma	Phase II www.apeiron- biologics.com
ARC-100	Archer Biosciences <i>New York, NY</i>	malignant melanoma	Phase II (646) 747-9090
ATN-224	Tactic Pharma <i>Evanston, IL</i>	malignant melanoma	Phase II (847) 733-0940
Azixa™ (verubulin)	Myrexis <i>Salt Lake City, UT</i>	malignant melanoma	Phase II (801) 214-7800
BMS-908662 (RAF kinase inhibitor)	Bristol-Myers Squibb <i>Princeton, NJ</i> Exelixis <i>South San Francisco, CA</i>	malignant melanoma	Phase I (212) 546-4000
BMS-936558 (anti-PD1)	Bristol-Myers Squibb <i>Princeton, NJ</i>	inoperable/unresectable malignant melanoma	Phase I (212) 546-4000

SKIN CANCER

Product Name	Company	Indication	Development Status
c31510	Cytotech Labs <i>Natick, MA</i>	basal cell cancer	Phase II completed
		squamous cell cancer	Phase I/II completed
CDX-011 (glembatumumab vedotin)	Celldex Therapeutics <i>Needham, MA</i>	malignant melanoma	Phase II (781) 433-0771
CNTO-95 (intelumumab)	Centocor Ortho Biotech <i>Horsham, PA</i>	malignant melanoma	Phase I/II (800) 457-6399
CST-101	ConKwest <i>Del Mar, CA</i>	stage IV malignant melanoma	Phase I (858) 380-1999
CYT-107 (interleukin-7)	Cytheris <i>Rockville, MD</i>	malignant melanoma	Phase I (301) 231-0450
denenicokin (IL-21) (Orphan Drug)	Bristol-Myers Squibb <i>Princeton, NJ</i>	malignant melanoma	Phase II (212) 546-5000
ecromeximab	Life Sciences Pharmaceuticals <i>Darien, CT</i>	malignant melanoma	Phase II (203) 656-2500
Fodosine™ forodesine	BioCryst Pharmaceuticals <i>Birmingham, AL</i>	cutaneous T-cell lymphoma	Phase II (205) 444-4600
Folotyn® pralatrexate	Allos Therapeutics <i>Westminster, CO</i>	cutaneous T-cell lymphoma	Phase I (303) 426-6262
GC-1008 (fresolimumab)	Genzyme <i>Cambridge, MA</i>	malignant melanoma	Phase I (617) 252-7000
Genasense® oblimersen (Orphan Drug)	Genta <i>Berkeley Heights, NJ</i>	malignant melanoma (Fast Track)	Phase III (908) 286-9800
GSK1120212 (MEK1/2 inhibitor)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	metastatic melanoma	Phase III (888) 825-5249
GSK1120212/ GSK2118436 (MEK1/2 inhibitor+ BRAF protein kinase inhibitor)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	metastatic melanoma	Phase II (888) 825-5249
GSK2118436 (BRAF protein kinase inhibitor)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	metastatic melanoma	Phase III (888) 825-5249
GSK2241658A (NY-ESO-1 recombinant vaccine)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	metastatic melanoma	Phase I (888) 825-5249
GSK2302032A (PRAME immunotherapeutic recombinant vaccine)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	metastatic melanoma	Phase I (888) 825-5249

SKIN CANCER

Product Name	Company	Indication	Development Status
GSK2401502 (domain antibody targeted vaccine)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i> Lipotek <i>Acton, Australia</i>	malignant melanoma	Phase I (888) 825-5249
HyperAcute® Melanoma melanoma vaccine	NewLink Genetics <i>Ames, IA</i>	malignant melanoma	Phase II (515) 296-5555
IMC-20D7S	Eli Lilly <i>Indianapolis, IN</i> ImClone Systems <i>New York, NY</i>	malignant melanoma	Phase I (800) 545-5979 (212) 645-1405
IMC-gp100	Immunocore <i>Oxon, United Kingdom</i>	malignant melanoma	Phase 0 www.immunocore.com
Imprime PGG®	Biothera <i>Eagen, MN</i>	malignant melanoma	Phase I/II (651) 675-0300
ioflubenzamide I-131	Molecular Insight Pharmaceuticals <i>Cambridge, MA</i>	malignant melanoma	Phase I (617) 492-5554
Karenitecin® cositecan	BioNumerik Pharmaceuticals <i>San Antonio, TX</i>	malignant melanoma	Phase II completed (210) 614-1701
lenvatinib (E7080)	Eisai <i>Woodcliff Lake, NJ</i>	melanoma	Phase II (888) 422-4743
Levulan® Kerastick® aminolevulinic acid	DUSA Pharmaceuticals <i>Wilmington, MA</i>	prevention of skin cancer in solid organ transplant patients (see also warts)	Phase II (978) 657-7500
Lymphoseek™	Neoprobe <i>Dublin, OH</i>	diagnosis of malignant melanoma	Phase III (800) 793-0079
MAGE-A3 ASCI (astuprotimut-R)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	melanoma	Phase III (888) 825-5249
Marqibo® vincristine liposomal (Orphan Drug)	Talon Therapeutics <i>South San Francisco, CA</i>	malignant melanoma	Phase II (650) 588-6404
mechlorethamine gel (anti-CD70 ADC) (Orphan Drug)	Yaupon Therapeutics <i>Radnor, PA</i>	cutaneous T-cell lymphoma	Phase I (610) 975-9290
melanoma vaccine	Ichor Medical Systems <i>San Diego, CA</i> Memorial Sloan-Kettering Cancer Center <i>New York, NY</i>	malignant melanoma	Phase I (858) 550-2022
MEK162 (ARRY-162)	Array BioPharma <i>Boulder, CO</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	malignant melanoma	Phase II (877) 633-2436 (888) 669-6682

SKIN CANCER

Product Name	Company	Indication	Development Status
Metvixia® methyl aminolevulinate	PhotoCure <i>Oslo, Norway</i>	basal cell cancer (see also acne)	Phase III www.photocure.com
MKC1106-MT	MannKind <i>Valencia, CA</i>	malignant melanoma	Phase II (661) 775-5300
MORAb-004	Eisai <i>Woodcliff Lake, NJ</i>	melanoma	Phase I (888) 422-4743
MORAb-028	Eisai <i>Woodcliff Lake, NJ</i>	malignant melanoma	Phase I (888) 422-4743
muparfostat (PI-88) (Orphan Drug)	Progen Pharmaceuticals <i>Redwood City, CA</i>	malignant melanoma	Phase II (650) 610-7080
OncoVEX ^{GM-CSF}	BioVex <i>Woburn, MA</i>	malignant melanoma	Phase III (781) 376-4900
Ontak® denileukin diftitox	Eisai <i>Woodcliff Lake, NJ</i>	melanoma	Phase II (888) 422-4743
PEP005 (ingenol mebutate)	LEO Pharma <i>Parsippany, NJ</i>	basal cell carcinoma (see also actinic keratoses)	Phase II (877) 494-4536
PF-01367338	Pfizer <i>New York, NY</i>	malignant melanoma	Phase II (860) 732-5156
prophage (vitespen) (Orphan Drug)	Agenus <i>Lexington, MA</i>	malignant melanoma (Fast Track)	Phase III (781) 674-4400
PTC299	PTC Therapeutics <i>South Plainfield, NJ</i>	Kaposi's sarcoma	Phase I/II (908) 222-7000
PTI-188	Pain Therapeutics <i>San Mateo, CA</i>	malignant melanoma	Phase I completed (650) 624-8200
PV-10 (intralesional)	Provectus Pharmaceuticals <i>Knoxville, TN</i>	malignant melanoma	Phase II (866) 594-5999
RAF265	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	malignant melanoma	Phase I (888) 669-6682
ramucirumab	Eli Lilly <i>Indianapolis, IN</i> Imclone Systems <i>Bridgewater, NJ</i>	malignant melanoma	Phase II (800) 545-5979
Reolysin® reovirus	Oncolytics Biotech <i>Calgary, Canada</i>	malignant melanoma	Phase II (403) 670-7377
RG3616 (systemic hedgehog antagonist)	Curis <i>Lexington, MA</i> Roche <i>Nutley, NJ</i>	basal cell carcinoma	Phase II (973) 235-5000
RG7256 (BRAF kinase inhibitor)	Roche <i>Nutley, NJ</i>	BRAF-mutated malignant melanoma	Phase I (973) 235-5000

SKIN CANCER

Product Name	Company	Indication	Development Status
RG7420 (MEK protein kinase kinase inhibitor)	Exelixis <i>South San Francisco, CA</i> Genentech <i>South San Francisco, CA</i>	malignant melanoma	Phase I (650) 837-7000 (800) 626-3553
Tasigna® nilotinib	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	malignant melanoma	Phase III (888) 669-6682
Temodar® temozolomide	Merck <i>Whitehouse Station, NJ</i>	malignant melanoma	Phase I (800) 672-6372
tesetaxel (Orphan Drug)	Genta <i>Berkeley Heights, NJ</i>	advanced melanoma	Phase II (908) 286-9800
TNFrade™ golnerminogene pradenovec	GenVec <i>Gaithersburg, MD</i>	malignant melanoma	Phase II completed (240) 632-0740
tremelimumab (CP-675206)	Pfizer <i>New York, NY</i>	malignant melanoma	Phase II (860) 732-5156
TRX518	Tolerx <i>Cambridge, MA</i>	malignant melanoma	Phase I (617) 354-8100
vegin (VEGF antisense)	VasGene Therapeutics <i>Los Angeles, CA</i>	Kaposi's sarcoma	Phase I (323) 221-7818
vemurafenib (BRAF kinase inhibitor)	Plexxikon <i>Berkeley, CA</i> Roche <i>Nutley, NJ</i>	first-line malignant melanoma ----- second- and third-line malignant melanoma	application submitted (510) 647-4000 (973) 235-5000 Phase II (510) 647-4000 (973) 235-5000
Yervoy™ ipilimumab (Orphan Drug)	Bristol-Myers Squibb <i>Princeton, NJ</i>	metastatic melanoma, adjuvant therapy	Phase III (212) 546-4000
Zadaxin® thymalfasin (Orphan Drug)	SciClone Pharmaceuticals <i>Foster City, CA</i>	malignant melanoma	Phase II completed (650) 358-3456
zanolimumab	TenX Biopharma <i>Philadelphia, PA</i>	cutaneous T-cell lymphoma (Fast Track)	Phase III (215) 774-1176

SKIN INFECTIONS

Product Name	Company	Indication	Development Status
aciclovir extended-release	BioAlliance Pharma <i>Paris, France</i>	treatment of oral herpes	Phase III completed www.bioalliancepharma.com
AIC316	AiCuris <i>Wuppertal, Germany</i>	herpes simplex virus infections	Phase II www.aicuris.com
albaconazole	Stiefel, a GSK Company <i>Rsch. Triangle Park, NC</i>	onychomycosis	Phase II completed (888) 825-5249

SKIN INFECTIONS

Product Name	Company	Indication	Development Status
AN2690	Anacor Pharmaceuticals <i>Palo Alto, CA</i>	onychomycosis	Phase III (650) 543-7500
AN2718	Anacor Pharmaceuticals <i>Palo Alto, CA</i>	onychomycosis, skin fungal infections	Phase I (650) 543-7500
ARYS-01 (sorivudine topical)	aRigen Pharmaceuticals <i>Tokyo, Japan</i>	herpes zoster	Phase II/III www.arigen.jp
AZD9742 (BTGT4 IV)	AstraZeneca <i>Wilmington, DE</i>	methicillin-resistant <i>Staphylococcus aureus</i> (MRSA) infections	Phase I (800) 236-9933
BC-3781	Nabriva Therapeutics <i>Vienna, Austria</i>	skin and soft tissue infections	Phase II www.nabriva.com
BSYX-L210 (lysostaphin topical cream)	Biosynexus <i>Gaithersburg, MD</i>	staphylococcal infections	Phase I/II (301) 330-5800
BTL-TML001	Beech Tree Labs <i>Providence, RI</i>	recurrent oral herpes	Phase I/II (401) 273-2060
CEM-102 (fusidic acid oral)	Cempra Pharmaceuticals <i>Chapel Hill, NC</i>	skin and soft tissue infections	Phase II (919) 467-1716
CMX001	Chimerix <i>Durham, NC</i>	smallpox	Phase I completed (919) 806-1074
CXL104 (CEF104)	AstraZeneca <i>Wilmington, DE</i> Forest Laboratories <i>New York, NY</i>	MRSA infections	Phase II (800) 236-9933 (800) 947-5227
dalbavancin	Durata Therapeutics <i>Morristown, NJ</i>	skin and soft tissue infections	Phase III (646) 871-6480
delafloxacin	Rib-X Pharmaceuticals <i>New Haven, CT</i>	MRSA infections, skin and soft tissue infections	Phase II (203) 624-5606
E-0706	Elorac <i>Vernon Hills, IL</i>	skin fungal infections	Phase III (847) 362-8200
EDP-322	Enanta Pharmaceuticals <i>Watertown, MA</i>	MRSA infections	Phase I (617) 607-0800
Ertaczo™ sertaconazole (new delivery system)	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	tinea pedis	Phase III (800) 817-5286
Famvir® famciclovir	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	recurrent herpes labialis in adolescents	Phase II/III completed (888) 669-6682
FV-100	Inhibitex <i>Alpharetta, GA</i>	herpes zoster	Phase II (678) 746-1100
GSK1322322 (peptide deformylase inhibitor)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	skin and soft tissue infections	Phase II completed (888) 825-5249
GSK1437173A (zoster recombinant vaccine)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	prevention of varicella zoster virus infections	Phase III (888) 825-5249

SKIN INFECTIONS

Product Name	Company	Indication	Development Status
GSK2392102A vaccine	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	staphylococcal infections	Phase I (888) 825-5249
herpes simplex DNA vaccine	Pfizer <i>New York, NY</i>	herpes simplex virus infections	Phase I (732) 860-5156
herpes simplex vaccine	AuRx <i>Elkridge, MD</i>	herpes simplex virus infections	Phase I/II (410) 796-7559
HerpV	Agenus <i>Lexington, MA</i>	herpes simplex virus infections	Phase I completed (781) 674-4400
IDP 108	Valeant Pharmaceuticals International <i>Irvine, CA</i>	onychomycosis	Phase III (800) 548-5100
IDP 113	Valeant Pharmaceuticals International <i>Irvine, CA</i>	tinea capitis	Phase I (800) 548-5100
ImmunoVEXHSV2 vaccine	BioVex <i>Woburn, MA</i>	genital herpes prevention	Phase I (781) 376-4900
JNJ-32729463 (fluoroquinolone)	Furiex Pharmaceuticals <i>Morrisville, NC</i>	skin and soft tissue infections	Phase II (919) 456-7814
luliconazole	Topica Pharmaceuticals <i>Palo Alto, CA</i>	tinea pedis	Phase II (650) 473-3800
		onychomycosis	Phase I/II (650) 473-3800
NB-001 (nanoemulsion)	NanoBio <i>Ann Arbor, MI</i>	herpes simplex virus infections (recurrent herpes labialis)	Phase III (734) 302-4000
NB-002 (cetylpyridinium nanoemulsion)	NanoBio <i>Ann Arbor, MI</i>	distal subungual onychomycosis of the toenail	Phase II (734) 302-4000
NB-002T	NanoBio <i>Ann Arbor, MI</i>	onychomycosis, tinea capitis	Phase I (734) 302-4000
NB-004	NanoBio <i>Ann Arbor, MI</i>	molluscum contagiosum	Phase I (734) 302-4000
NDV-3	NovaDigm Therapeutics <i>Grand Forks, ND</i>	candidiasis, MRSA infections	Phase I (701) 757-5161
nitric oxide topical	NB Therapeutics <i>Bristol, PA</i>	skin and soft tissue infections	Phase II (866) 957-6200
omadacycline	Novartis Pharmaceuticals <i>East Hanover, NJ</i> Paratek Pharmaceuticals <i>Boston, MA</i>	skin and soft tissue infections	Phase III (888) 669-6682 (617) 275-0040
oritavancin	The Medicines Company <i>Parsippany, NJ</i>	skin and soft tissue infections	Phase III (800) 388-1183
pagibaximab (Orphan Drug)	Biosynexus <i>Gaithersburg, MD</i>	staphylococcal infections	Phase II/III (301) 330-5800
PF-05230894 (3-antigen <i>Staphylococcus aureus</i> vaccine)	Inhibitex <i>Alpharetta, GA</i>	staphylococcal infections	Phase I (678) 746-1100
	Pfizer <i>New York, NY</i>		(732) 860-5156

SKIN INFECTIONS

Product Name	Company	Indication	Development Status
PMX-30063	PolyMedix <i>Radnor, PA</i>	skin and soft tissue infections due to <i>Staphylococcus aureus</i>	Phase II (484) 598-2340
radezolid	Rib-X Pharmaceuticals <i>New Haven, CT</i>	skin and soft tissue infections	Phase II (203) 624-5606
Remicade® imatinib	Centocor Ortho Biotech <i>Horsham, PA</i>	pyoderma in patients with inflammatory bowel disease	Phase II (800) 457-6399
RPI-78M	ReceptoPharm <i>Plantation, FL</i>	herpes simplex virus infections	Phase I completed (954) 321-8988
rSEB (staphylococcal biodefense vaccine)	BioSante Pharmaceuticals <i>Lincolnshire, IL</i>	staphylococcal infections	Phase I/II (847) 478-0500
SPL-7013	Starpharma <i>Melbourne, Australia</i>	herpes simplex virus infections	Phase I www.starpharma.com
<i>Staphylococcus aureus</i> vaccine conjugate pentavalent	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	staphylococcal infections	Phase I/II (888) 825-5249
TD-1792	Theravance <i>South San Francisco, CA</i>	skin and soft tissue infections	Phase II (877) 275-8479
terbinafine iontophoretic	NB Therapeutics <i>Bristol, PA</i>	onychomycosis	Phase II (866) 957-6200
terbinafine lacquer	Apricus Biosciences <i>San Diego, CA</i>	onychomycosis	Phase III completed (858) 222-8041
terbinafine topical (TDT 067)	Celtic Pharma <i>Hamilton, Bermuda</i>	onychomycosis	Phase III (212) 616-4000
toezolid	Trius Therapeutics <i>San Diego, CA</i>	skin and soft tissue infections	Phase III (858) 452-0370
Tygacil® tigecycline	Pfizer <i>New York, NY</i>	skin and soft tissue infections in children	Phase II (860) 73215156
V212	Merck <i>Whitehouse Station, NJ</i>	herpes zoster prevention	Phase I (800) 672-6372
V710	Merck <i>Whitehouse Station, NJ</i>	staphylococcal infections prevention	Phase II/III (800) 672-6372
valomaciclovir	Epiphany Biosciences <i>San Francisco, CA</i>	herpes zoster	Phase II (415) 765-7200
varicella zoster immune globulin (Orphan Drug)	Cangene <i>Winnipeg, Canada</i>	varicella zoster virus infections	Phase III (204) 275-4200
WAP 8294A2	aRigen Pharmaceuticals <i>Tokyo, Japan</i>	MRSA infections	Phase I www.arigen.jp
WC-3018	Warner Chilcott <i>Rockaway, NJ</i>	skin and soft tissue infections (see also acne)	Phase II (973) 442-3200

VITILIGO

Product Name	Company	Indication	Development Status
afamelanotide	Clinuvel Pharmaceuticals New York, NY Melbourne, Australia	vitiligo (see also other)	in clinical trials (201) 633-4773
ATX-202	Kythera Biopharmaceuticals Calabasas, CA	vitiligo	Phase I (818) 587-4500
Elidel® pimecrolimus topical	Novartis Pharmaceuticals East Hanover, NJ	vitiligo (see also dermatitis)	in clinical trials (888) 669-6682

WARTS

Product Name	Company	Indication	Development Status
albuterpenoid ointment	ViroXis San Antonio, TX	common warts	Phase II (210) 558-8896
IDP 109	Valeant Pharmaceuticals International Irvine, CA	common warts	Phase I (800) 548-5100
iontophoretic warts gene therapy	NB Therapeutics Bristol, PA	non-genital warts	Phase II (866) 957-6200
Levulan® Kerastick® aminolevulinic acid	DUSA Pharmaceuticals Wilmington, DE	warts (see also skin cancer)	Phase I/II (978) 657-7500

WOUNDS

Product Name	Company	Indication	Development Status
azficel-T (fibroblast cell therapy)	Fibrocell Science Exton, PA	scars	Phase II/III (484) 713-6000
AZX100	Capstone Therapeutics Tempe, AZ	prevention of post-incision keloid scarring	Phase II (800) 937-5520
CODA 001 (antisense oligonucleotide)	CoDa Therapeutics San Diego, CA	leg ulcers	Phase II (858) 677-0474
Cogenzia® gentamicin topical	Innocoll Ashburn, VA	diabetic foot ulcers	Phase II www.innocollinc.com
doxycycline topical gel	Nanotherapeutics Alachua, FL	diabetic foot ulcers	Phase II (386) 462-9663
		wounds	Phase I (386) 462-9663
DSC-127	Derma Sciences Princeton, NJ	diabetic foot ulcers	Phase II (609) 514-4744
EXC-001	Excaliard Pharmaceuticals Carlsbad, CA	scars	Phase II (760) 431-1850

WOUNDS

Product Name	Company	Indication	Development Status
fibroblast growth factor 1 (FGF-1)	CardioVascular BioTherapeutics <i>Las Vegas, NV</i>	wounds in diabetes	Phase II completed (702) 839-7200
GBT 900	Garnet BioTherapeutics <i>Malvern, PA</i>	prevention of scarring	Phase II (610) 240-4150
GLYC-101	Glycotex <i>Rockville, MD</i>	wounds	Phase II completed (301) 670-2825
HP802-247 (allogeneic human skin replacement)	Healthpoint Biotherapeutics <i>Fort Worth, TX</i>	leg ulcers, skin ulcers	Phase II (800) 441-8227
iroxanadine	CytRx <i>Los Angeles, CA</i>	diabetic foot ulcers	Phase I (310) 826-5648
KUR-211	Baxter International <i>Deerfield, IL</i> Kuros Biosurgery <i>Zurich, Switzerland</i>	diabetic foot ulcers	Phase II (800) 422-9837
Locilex™ pexiganan	Dipexium Pharmaceuticals <i>White Plains, NY</i>	diabetic foot ulcers	application submitted (914) 949-3898
nemoxacin	Warner Chilcott <i>Rockaway, NJ</i>	diabetic foot ulcers	Phase II completed (973) 442-3283
StrataGraft® tissue repair cell therapy	Stratatech <i>Madison, WI</i>	chronic wounds	Phase I/II (608) 441-2750
thymosin beta-4 (RGN-137 topical)	RegeneRx Biopharmaceuticals <i>Rockville, MD</i>	pressure ulcers, venous stasis ulcers	Phase II completed (301) 208-9191
		epidermolysis bullosa	Phase II (301) 208-9191
topical talactoferrin alfa	Agennix <i>Princeton, NJ</i>	diabetic foot ulcers (Fast Track)	Phase II (609) 524-1000
WPP-201	Intralytix <i>Baltimore, MD</i>	leg ulcers	Phase I (877) 489-7424

OTHER

Product Name	Company	Indication	Development Status
afamelanotide (Orphan Drug)	Clinuvel Pharmaceuticals <i>New York, NY</i> <i>Melbourne, Australia</i>	erythropoietic protoporphyria (see also vitiligo)	Phase II completed (201) 633-4773
AM-001 (fibroblast growth factor inhibitor)	DermAct Pharmaceutical <i>Melville, NY</i>	undisclosed skin disease	Phase II (631) 577-4015
CellCept® mycophenolate mofetil (Orphan Drug)	Aspreva Pharmaceuticals <i>Basking Ridge, NJ</i>	pemphigus vulgaris	Phase III (908) 212-1020

OTHER

Product Name	Company	Indication	Development Status
COL-179	Onset Dermalogics <i>Cumberland, RI</i>	undisclosed skin disease	in clinical trials (888) 713-8154
E-1415	Elorac <i>Vernon Hills, IL</i>	rare dermatological condition	Phase III (847) 362-8200
licochalcone A	Beiersdorf Research Center <i>Hamburg, Germany</i>	sensitive skin (see also rosacea)	Phase I www.beiersdorf.com
menadione topical lotion	Talon Therapeutics <i>San Mateo, CA</i>	exanthema	Phase I completed (650) 588-6404
norepinephrine topical	ProCertus BioPharm <i>Madison, WI</i>	prevention of radiation- induced skin damage (see also alopecia)	Phase I/II (608) 277-7950
NX-101	NexGenix Pharmaceuticals <i>New York, NY</i>	dermal neurofibromatoses	Phase II (212) 974-3006
QAX576	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	keloids (prevention of relapse)	Phase II (888) 669-6682
Rituxan [®] rituximab	Biogen Idec <i>Cambridge, MA</i> Genentech <i>South San Francisco, CA</i>	dermatomyositis	Phase II (617) 679-2000 (800) 626-3553
RT001 (botulinum toxin A)	Revance Therapeutics <i>Newark, CA</i>	hyperhidrosis	Phase I/II (510) 742-3400
TD101	TransDerm <i>Santa Cruz, CA</i>	pachyonychia congenita	Phase I (831) 420-1684
Xolair [®] omalizumab	Genentech <i>South San Francisco, CA</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	urticaria	Phase II (800) 626-3553 (888) 669-6682

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 May 14, 2011.** The information in this report may not be comprehensive. For more specific information about a particular product, contact the individual company directly or go to www.clinicaltrials.gov. The entire series of *Medicines in Development* is available on PhRMA’s 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.

actinic keratoses—Roughness and thickening of the skin caused by overexposure to the sun's ultraviolet rays. It can degenerate into a skin cancer called squamous cell carcinoma.

alopecia—Hair loss or baldness, which can occur for many reasons. Some hair loss causes are considered natural, while others signal serious health problems.

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

atopic dermatitis—This form of **eczema** is a non-contagious disorder characterized by chronically inflamed skin and sometimes intolerable itching.

basal cell carcinoma—Cancer of the lower layers of the skin.

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.

chronic wounds—Wounds that have failed to proceed through an orderly and timely reparative process to produce anatomic and functional integrity over a period of 3 months. The most commonly encountered chronic wound is the lower extremity **ulcer**, which is generally vascular or diabetic related and accounts for up to 98 percent of all lower extremity wounds.

cutaneous lupus erythematosus—A form of lupus erythematosus in which the skin may be the only organ involved or in which skin involvement precedes the spread into other body systems.

dermal neurofibromas—Skin tumors in patients with neurofibromatosis type 1 (NF1).

dermatomyositis—An uncommon inflammatory disease marked by muscle weakness and a distinctive skin rash.

discoid lupus erythematosus—A chronic skin disease occurring primarily in women between the ages of 20 and 40, characterized by an eruption of red lesions over the cheeks and bridge of the nose.

eczema—A general term used to describe a variety of conditions that cause an itchy, inflamed skin rash.

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.

erythema—Redness of the skin, usually occurring in patches, caused by irritation or injury to the tissue.

exanthema—A disease characterized by the appearance of a skin rash, e.g., measles or scarlet fever.

herpes labialis—Infection of the lips, mouth, or gums with the **herpes simplex virus**. It leads to the development of small, painful blisters commonly called cold sores or fever blisters.

herpes simplex virus—Three strains of the herpes virus often occur in AIDS patients: **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.

hyperhidrosis—A disorder marked by excessive sweating. It usually begins at puberty and affects the palms, soles, and armpits.

Kaposi's sarcoma—A rare malignant skin tumor that occurs in some AIDS patients. It can be accompanied by fever, enlarged lymph nodes and gastrointestinal problems.

keloid—Scar tissue that grows excessively after an injury heals, sometimes extending beyond the original wound site. Even seemingly minor injuries such as acne and piercings can cause keloids, which frequently occur in families and are common in African Americans and people with darker skin.

lupus vulgaris—Tuberculosis of the skin in which reddish brown patches develop on the face, leading to tissue destruction and scarring.

melanoma—The most dangerous form of skin cancer is a malignancy of the melanocyte, the cell that produces pigment in the skin. Most melanomas present as a dark, mole-like spot that spreads and, unlike a mole, has an irregular border. Melanoma is most common in people with fair skin, but it can occur in people with all skin colors. Risk increases with overexposure to the sun and sunburn.

molluscum contagiosum—A relatively common viral infection of the skin that most often affects

children. It results in firm bumps (papules) that are painless and usually disappear within a year without treatment. If the papules are scratched or injured, the infection can spread to surrounding skin.

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

onychomycosis—Disease, deformity or wasting of the nails caused by fungal infection.

pachyonychia congenita—A rare, congenital disorder marked by great thickening of the nails, hyperkeratosis (an excessive thickening of the outer layer of the skin) of the palms and soles, and leukoplakia (a precancerous condition of small thickened white patches, usually inside the mouth or vulva).

pediculosis—Infestation with lice, which are ectoparasites that live on the body. *Pediculus humanus capitis* is the head louse, which is spread from person to person by close physical contact or combs, hats, and clothes. Overcrowding encourages the spread of lice. Pediculosis affects 6 million-12 million people annually and is common among U.S. school children.

pemphigus—A group of rare skin disorders that cause blisters of the skin or mucous membranes, such as inside the mouth or on the genitals. The most common form of pemphigus, **pemphigus vulgaris**, tends to be more common in people of Middle Eastern or Jewish descent, though it can affect people of all races.

Phase 0—First-in-human trials conducted in accordance with

FDA's 2006 guidance on exploratory Investigational New Drug (IND) studies designed to speed up development of promising drugs by establishing very early on whether the agent behaves in human subjects as was anticipated from preclinical studies.

Phase I—Safety testing and pharmacological profiling in humans.

Phase II—Effectiveness and safety testing in humans.

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

pruritus—Itching resulting from a drug reaction, food allergy, kidney or liver disease, cancers, parasites, aging or dry skin, contact skin reaction, such as poison ivy, and for unknown reasons.

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**, which is also called **psoriatic vulgaris**.

psoriatic arthritis—A type of chronic inflammatory arthritis associated with the chronic skin condition **psoriasis**, which is believed to be an inherited condition. Psoriatic arthritis typically appears about 10 years after the onset of psoriasis, which can manifest at any age, though most people are diagnosed between the ages of 15 and 35.

pyoderma—Any bacterial skin infection producing pus.

Raynaud's disease—condition that causes some areas of the body—fingers, toes, the tip of the nose, and ears—to feel numb and cool in response to cold temperatures or stress. In Raynaud's disease, the smaller arteries that supply blood to the skin narrow, thus limiting blood circulation to affected areas.

rosacea—A skin disease typically appearing in people during their 30s and 40s. It is marked by redness (erythema) of the face, flushing of the skin, and the presence of hard pimples (papules) or pus-filled pimples (pustules), and small visible spider-like veins called telangiectasias. In later stages of the disease, the face may swell and the nose may take on a bulb-like appearance called rhinophyma.

scleroderma—A group of rare, progressive diseases that involve the hardening and tightening of the skin and connective tissues—the fibers that provide the framework and support for the body. Localized scleroderma affects only the skin. Systemic scleroderma also harms internal organs, such as the heart, lungs, kidneys, and digestive tract.

seborrheic dermatitis—A common inflammatory disease of the skin characterized by scaly lesions usually on the scalp, hairline, and face. Seborrheic dermatitis appears as red, inflamed skin covered by greasy or dry scales that may be white, yellowish, or gray. It can affect the scalp, eyebrows, forehead, face, folds around the nose and ears, the chest, armpits, and groin. Dandruff and cradle cap are mild forms of seborrheic dermatitis.

skin and soft-tissue infections—Viruses, bacteria, and fungi generally cause skin and soft-tissue infections by entering the body at a spot where a cut, scrape, bite, or other wound has broken the skin; some infections are even the result of bacteria that normally live on the body. These infections can affect the layers of the skin or deeper tissues, such as muscle and connective tissue (the interlacing framework of tissue that forms ligaments, tendons, and other supporting structures of the body), and they may bring about symptoms in other parts of the body.

smallpox—A contagious, disfiguring and often deadly disease caused by the variola virus. The first symptoms of smallpox usually appear 12 to 14 days after infection. During the incubation period of seven to 17 days, an infected person looks and feels healthy and can't infect others.

Following the incubation period, a sudden onset of flu-like signs and symptoms occurs, including fever, headache, severe fatigue, and sometimes vomiting, diarrhea or both. A few days later, the characteristic smallpox rash appears as flat, red spots (lesions). Within a day or two, many of these lesions turn into small blisters filled with clear fluid (vesicles) and later with pus (pustules). The rash appears first on the face, hands and forearms, and later on the trunk. The distribution of lesions is a hallmark of smallpox and a primary way of diagnosing the disease.

staphylococcal infections—Caused by *Staphylococcus* bacteria, which are germs commonly found on the skin or in the nose of healthy individuals. Most of the time, these bacteria cause no problems or result in relatively minor skin infections, but staph infections don't always remain skin-deep. In some circumstances, they may invade the bloodstream, urinary tract, lungs or heart. Skin infections caused by staph bacteria include: boils, impetigo, cellulitis, and scalded skin syndrome.

tinea capitis—A disease caused by superficial fungal infection of the skin of the scalp, eyebrows, and eyelashes, with a propensity for

attacking hair shafts and follicles. Several synonyms are used, including ringworm of the scalp and tinea tonsurans.

tinea pedis—Also called "athlete's foot," a skin infection caused by a fungus.

ulcers—A skin ulcer is a type of wound that develops on the skin. A **venous skin ulcer** is a shallow wound that develops when the leg veins don't return blood back toward the heart as they normally would. A venous skin ulcer also is called a **stasis leg ulcer**. These ulcers usually develop on the sides of the lower leg, above the ankle and below the calf. Venous skin ulcers are slow to heal and often come back if you don't take steps to prevent them. Pressure sores or

pressure ulcers—also called bedsores—are injuries to skin and underlying tissues that result from prolonged pressure on the skin. Bedsores most often develop on skin that covers bony areas of the body, such as the heel, ankles, hips or buttocks. People most at risk of bedsores are those with a medical condition that limits their ability to change positions, requires them to use a wheelchair, or confines them to a bed for prolonged periods. Bedsores can develop quickly and are often difficult to treat.

urticaria—Also known as chronic hives, which are batches of raised, red or white itchy welts (wheals) of various sizes that appear and disappear. While most cases of hives go away within a few weeks or less, for some people they are a long-term

problem. Chronic hives are defined as those that last more than six weeks or hives that go away but recur frequently. In most cases of chronic hives, a cause is never clearly identified. In some cases, they may be related to an underlying autoimmune disorder, such as thyroid disease or lupus.

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

vitiligo—A condition in which the skin loses melanin, the pigment that determines the color of a person's skin, hair and eyes. Vitiligo occurs when the cells that produce melanin die or no longer form melanin, causing slowly enlarging white patches of irregular shapes to appear on the skin. Vitiligo affects all races, but it may be more noticeable and disfiguring in people with darker skin. Vitiligo usually starts as small areas of pigment loss that spread with time. There is no cure for vitiligo. The goal of treatment is to stop or slow the progression of pigment loss.

warts—Common warts are local growths in the skin that are caused by human papillomavirus (HPV) infection. Although they are considered to be contagious, it is very common for just one family member to have them. In addition, they often affect just one part of the body (such as the hands or the feet) without spreading over time to other areas.

SELECTED FACTS ABOUT SKIN DISEASES IN THE UNITED STATES

Overview¹

- More than 100 million Americans—one-third of the U.S. population—are afflicted with skin diseases.
-

Acne

- **Acne**, also known as **acne vulgaris**, is the most common skin condition in the United States. Up to 50 million Americans have acne at any one time.² Acne affects 85 percent to 100 percent of people at some point in their lives, and it usually begins at puberty. Acne can persist into the 30s and beyond. Some 5 percent of people over age 45 still have acne, which affects people of all ethnic backgrounds.³
-

Actinic Keratosis⁴

- **Actinic keratosis** is the most common precancer, affecting more than 58 million Americans. Approximately 65 percent of all squamous cell carcinomas arise in lesions that previously were diagnosed as actinic keratoses. In patients with a history of two or more skin cancers, 36 percent of **basal cell carcinomas** arise in lesions previously diagnosed as actinic keratoses.
-

Alopecia⁵

- In men, the age of onset for pattern hair loss (**alopecia**) usually is between the ages of 20 and 25, and the prevalence and severity of the condition increases with age. In general, 30 percent of white people are affected by age 30, 50 percent by age 50, and 80 percent by age 70. In women, the onset of hair loss is usually before age 40, and up to 13 percent of premenopausal women have some evidence of pattern hair loss. The incidence of hair loss increases in women around the time of menopause and may affect 70 percent of women over age 65.
-

Dermatitis²

- Around the world, between 10 percent and 20 percent of children have **atopic dermatitis (AD)**, a form of **eczema**. Most people (90 percent) get AD before their fifth birthday. About 1 percent to 3 percent of adults have AD, which affects people of all skin colors.
-

Lupus⁶

- At least 1.5 million Americans have **lupus**. It strikes mostly women of childbearing age (15-44); however, men, children, and teenagers develop lupus, too. Women of color are two to three times more likely to develop lupus. More than 16,000 new cases of lupus are reported annually across the country.
 - Approximately 10 percent of people who have **cutaneous lupus** will develop systemic lupus, although it is likely that these people already had systemic lupus, with the skin rash as their main symptom.
-

Pediculosis (Head Lice)⁷

- **Pediculosis—head lice** infection—is very common. It has been estimated that up to one in every 10 children in school acquires head lice at some time. One study found that the estimated annual cost of head lice infestations in this country was nearly \$1 billion dollars.
-

Pruritus (Itching)⁸

- **Pruritus** occurs in approximately 20 percent of adults. It is present in approximately 25 percent of patients with jaundice and in 50 percent of patients receiving renal dialysis.
-

SELECTED FACTS ABOUT SKIN DISEASES IN THE UNITED STATES

Psoriasis

- **Psoriasis** is the most prevalent autoimmune disease in this country. According to current studies, as many as 7.5 million Americans—approximately 2.2 percent of the population—have psoriasis. Psoriasis prevalence in African Americans is 1.3 percent compared to 2.5 percent of Caucasians.⁹
 - **Plaque psoriasis** is the most widespread form of psoriasis. About 1 percent to 2 percent of individuals in the United States suffer from plaque psoriasis—that’s about 5.5 million Americans. Females tend to develop plaque psoriasis sooner than males do. The first peak incidence of plaque psoriasis is in individuals ages 16 to 22. The second peak is in individuals ages 57 to 60.¹⁰
 - Up to 30 percent of people diagnosed with psoriasis actually have **psoriatic arthritis**. People of all ages can develop psoriatic arthritis, but the peak incidence is between the ages of 30 and 50. Up to 57 percent of patients experienced joint damage by the time they were diagnosed with psoriatic arthritis. In up to 70 percent of psoriatic arthritis patients, skin symptoms precede joint symptoms. In psoriatic arthritis, joint symptoms generally present 10 years after skin symptoms, but both may present simultaneously.¹¹
 - The total direct and indirect health care costs of **psoriasis** for patients are calculated at \$11.25 billion annually, with work loss accounting for 40 percent of the cost burden. Approximately 60 percent of psoriasis patients miss an average of 26 days of work a year due to their illness.⁹
-

Rosacea⁷

- As of 2010, **rosacea** was estimated to affect at least 16 million people in the United States alone and approximately 45 million worldwide. Most people with rosacea are Caucasians with fair skin; females, especially around menopause; and those ages 30-50.
-

Scleroderma

- **Scleroderma**, a group of rare, progressive diseases that causes hardening and tightening of the skin and connective tissues, occurs at least four times as often in women as it does in men and usually occurs between the ages of 30 and 50. Systemic scleroderma is more common in African Americans than it is in Americans of European descent, and those African-Americans are more likely to develop severe lung complications.¹² The number of adults with all forms of scleroderma in the United States is estimated to be approximately 300,000. Fifty percent of patients with the systemic form will die within 5 years of the onset of disease without treatment. With treatment, the 5-year survival is greater than 80 percent, although morbidity is considerable.¹³
-

Skin Cancer

- **Skin cancer** is the most common form of cancer in the United States. More than 3.5 million skin cancer cases in over 2 million people are diagnosed annually. Each year there are more new cases of skin cancer than the combined incidence of cancers of the breast, prostate, lung and colon. One in five Americans will develop skin cancer in the course of a lifetime. About 90 percent of nonmelanoma skin cancers are associated with exposure to ultraviolet (UV) radiation from the sun.⁴
- **Basal cell carcinoma (BCC)** is the most common form of skin cancer, with an estimated 2.8 million diagnosed annually in this country. BCCs are rarely fatal, but they can be highly disfiguring if allowed to grow. The number of women under age 40 diagnosed with basal cell carcinoma has more than doubled in the last 30 years. BCC is the most common cancer in Caucasians, Hispanics, Chinese, Japanese, and other Asian populations.⁴
- Before the AIDS epidemic, **Kaposi’s sarcoma (KS)** rarely occurred in the United States. About two new cases of KS were found for every 1 million people in this country annually. Now, most KS cases occur in people infected with HIV. It has been estimated that an HIV-infected person has a 20,000-fold increased risk of developing KS compared with people who do not have HIV. AIDS patients with KS increased the rate of KS in this country more than 20 times, peaking at 47 cases per 1 million people annually in the early 1990s. With new treatments for AIDS, KS has become less common, with about six cases per million people each year. KS is much more common in men than in women, and it is rarely seen in children.¹⁴

SELECTED FACTS ABOUT SKIN DISEASES IN THE UNITED STATES

Skin Cancer (continued)

- Nearly 800,000 Americans are living with a history of **melanoma** and 13 million are living with a history of nonmelanoma skin cancer, typically diagnosed as **basal cell carcinoma** or squamous cell carcinoma.⁴
- One in 55 people will be diagnosed with **melanoma** during their lifetime. Melanoma is the most common form of cancer for young adults ages 25-29 and the second most common form of cancer for young people ages 15-29. One in 39 Caucasian men and one in 58 Caucasian women will develop melanoma in their lifetimes.⁴
- An estimated 114,900 new cases of **melanoma** were diagnosed in the United States in 2010—46,770 noninvasive (in situ) and 68,130 invasive, with nearly 8,700 resulting in death. Melanoma accounts for less than 5 percent of skin cancer cases, but it causes more than 75 percent of skin cancer deaths.⁴
- One person dies of **melanoma** every hour (every 62 minutes). The survival rate for patients whose melanoma is detected early, before the tumor has penetrated the skin, is about 99 percent. The survival rate falls to 15 percent for those with advanced disease.⁴
- In 2004, the total direct cost associated with the treatment for nonmelanoma **skin cancer** was \$1.5 billion.⁴
- In adults 65 or older, **melanoma** treatment costs total about \$249 million annually. About 40 percent of the annual cost for melanoma goes to treating stage IV (advanced) cancers, although they account for only 3 percent of melanomas.⁴

Skin Infections

- Half of all nail disorders are caused by **onychomycosis**, a fungal infection, and it is the most common nail disease in adults. Toenails are much more likely to be infected than fingernails. The incidence of onychomycosis has been increasing and is related to diabetes, a suppressed immune system, and increasing age. Adults are 30 times more likely to have onychomycosis than children. In fact, only 2.6 percent of children younger than age 18 are reported to have onychomycosis, but as many as 90 percent of elderly people have the disorder.¹⁵ About 10 percent of the U.S. population has onychomycosis.¹⁶
- Hospitalizations caused by the **Staphylococcus aureus** bacteria are on the rise in the United States, increasing 62 percent between 1999 and 2005. During the same period, **methicillin-resistant Staphylococcus aureus**, or **MRSA**, accounted for a 119 percent jump in hospitalizations, growing from 127,036 to 278,203, according to the U.S. Centers for Disease Control. The estimated number of hospitalizations involving *S. aureus*-related infections also increased remarkably from 294,570 to 477,927 during the same period.¹⁷
- **Tinea capitis** is predominantly a disease of preadolescent children. It accounts for up to 92.5 percent of dermatophytoses (fungal infections) in children younger than age 10. Peak age range is in patients ages 3-7. The disease is rare in adults, although occasionally it may be found in elderly patients. Tinea capitis occurrence is widespread in some urban areas in the United States.⁸
- **Tinea pedis** (athlete's foot) is thought to be the world's most common dermatophytosis or fungal infection. Reportedly, 70 percent of the population will be infected with tinea pedis at some time. Tinea pedis has no predilection for any racial or ethnic group, but it more commonly affects males compared with females. The prevalence of tinea pedis increases with age. Most cases occur after puberty. Childhood tinea pedis is rare.¹³

Warts⁵

- Generally, common **warts** affect children ages 5 to 10 and young adults. Only 15 percent occur in patients older than age 35. Warts are also more prevalent in patients with suppressed immune systems, such as in HIV/AIDS, transplant recipients or patients receiving chemotherapy or other immunosuppressive medications.

SELECTED FACTS ABOUT SKIN DISEASES IN THE UNITED STATES

Wounds

- One of the most common complications of diabetes in the lower extremity is the **diabetic foot ulcer**. An estimated 15 percent of patients with diabetes will develop a lower extremity ulcer during the course of their disease. Infected and ischemic foot ulcers are major causes for diabetes-related hospitalization. The average hospital length of stay for diabetic patients with ulcer diagnoses was 59 percent longer than for those diabetes discharges without ulcers.¹⁸
- **Diabetic foot ulceration** is the precursor to approximately 85 percent of lower extremity amputations in people with diabetes. Costs for ulcer care in the United States have been estimated in the range of \$4,595 per ulcer episode to nearly \$28,000 for the 2 years after diagnosis. One report estimates 800,000 prevalent ulcer cases in the United States with costs averaging \$5,457 per year per patient, which amounts to total national annual costs of \$5 billion.¹⁸
- **Pressure ulcers** are among the most common conditions in patients acutely hospitalized or requiring long-term institutional care. Estimates of the rates of pressure ulcers vary widely by clinical setting. An estimated 2.5 million pressure ulcers are treated each year in U.S. acute care facilities alone. One study showed an 80 percent increase in the number of hospital stays involving pressure ulcers in the United States between 1993 and 2006. In more than 90 percent of cases, the pressure ulcer was not the cause of admission. Most patients with pressure ulcers were over age 65. More than half of the patients with pressure ulcers required subsequent care in long-term care facilities, as compared with 16.2 percent of patients without pressure ulcers.¹⁹ Several studies have noted the association of pressure ulcer development and mortality in both the hospital and nursing-home settings. The mortality rate has been as high as 60 percent for those older patients who develop a pressure ulcer within one year of hospital discharge.²⁰

Sources:

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18. American Professional Wound Care Association (www.apwca.org)
19. UpToDate, Inc. (www.uptodate.com)
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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 shows results of previous experiments; how, where and by whom the new studies will be conducted; the chemical structure of the compound; how it is thought to work in the body; any toxic effects found in the animal studies; and how the compound is manufactured. All clinical trials must be reviewed and approved by the Institutional Review Board (IRB) where the trials will be conducted. Progress reports on clinical trials must be submitted at least annually to FDA and the IRB.

Clinical Trials, Phase I. These tests usually involve

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

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

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

New Drug Application (NDA)/Biologic License Application (BLA). Following the completion of all three phases of clinical trials, a company analyzes all of the data and files an NDA or BLA with FDA if the data successfully demonstrate both safety and effectiveness. The applications contain all of the scientific information that the company has gathered. Applications typically run 100,000 pages or more. The average review time for the 21 new therapeutics approved by the FDA in 2010 was 14.8 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 \$67.4 billion in research and development in 2010.

TRACKING THE BIOPHARMACEUTICAL RESEARCH PIPELINE

Today, more than 3,000 new medicines are in development in the United States. Many of these potential new medicines will fail in clinical trials, but some may represent tomorrow's new treatments. Bringing each new medicine to patients will require, on average, 10 to 15 years of testing and review.

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

- **Cancer**—There are few things that cause patients more fear and uncertainty as a cancer diagnosis. Yet today—because of a steady stream of new and improved medicines and treatments—cancer can increasingly be managed and even beaten. *The 2010 report found 887 medicines in the pipeline.*
- **Heart Disease and Stroke**—Keeping up the momentum of drug discovery that has helped cut deaths from heart disease and stroke in half in the past three decades, biopharmaceutical companies are working on new medicines for these diseases. *The 2011 report found 299 medicines in the pipeline.*
- **Diabetes**—Approximately 4,110 people are diagnosed with diabetes every day. To help fight this disease, pharmaceutical and biotechnology researchers are working on new medicines to treat it and related conditions. *The 2010 report found 235 medicines in the pipeline.*
- **Mental Illnesses**—Pharmaceutical and biotechnology researchers are testing many new medicines to help the more than 450 million people worldwide who suffer from some form of mental illness. *The 2010 report found 313 medicines in the pipeline.*
- **Biotechnology**—Millions of people have already benefited from medicines and vaccines developed through biotechnology, and a new report offers hope that many more will benefit in the future. *The 2008 report found 633 medicines in the pipeline.*
- **Children**—Biopharmaceutical researchers are testing medicines to meet the special needs of children. These medicines offer hope that the significant improvements achieved in children's health over the past few decades will continue and even accelerate. *The 2010 report found 234 medicines in the pipeline.*
- **Infectious Diseases**—Throughout history, infectious diseases have taken a devastating toll on the lives and well-being of people around the world. Today, vaccines and antibiotics have proven to be effective treatments in many cases, but infectious diseases still pose a very serious threat to patients. Infectious diseases may never be eradicated. But, new knowledge, new technologies, and a huge commitment of resources by America's biopharmaceutical research companies and the government can help meet the continuing—and ever-changing—threat from infectious diseases. *The 2010 report found 395 medicines in the pipeline.*



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