

# Diabetes

PRESENTED BY AMERICA'S PHARMACEUTICAL RESEARCH COMPANIES

## Biopharmaceutical Companies Developing a Record 235 Medicines to Treat Diabetes and Related Conditions

**D**iabetes is increasing at an alarming rate in America. Each day, approximately 4,110 people are diagnosed with diabetes. To help fight this potentially devastating disease, America's pharmaceutical research and biotechnology companies are working on 235 new medicines to treat diabetes and related conditions. All of the medicines in this report are either in clinical trials or awaiting approval by the U.S. Food and Drug Administration.

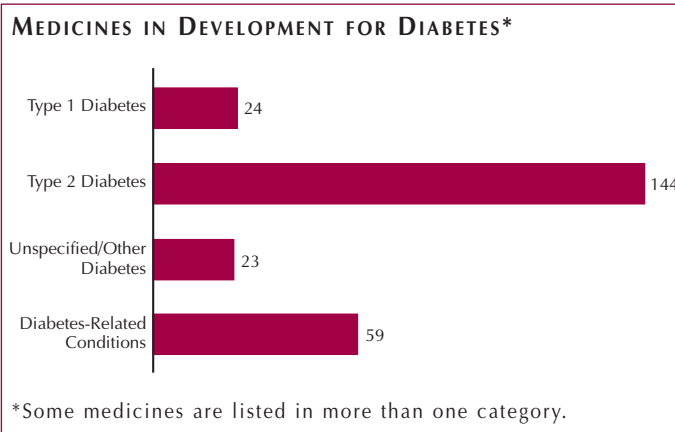
According to the American Diabetes Association (ADA), diabetes affects more than 20 million Americans—about 8 percent of the U.S. population—and a third are unaware they have the disease. Unfortunately, the rate of new cases and the death rate due to diabetes has been rising. The rate of new cases rose by more than 90 percent among adults over the last 10 years, according to a 2008 study by the U.S. Centers for Disease Control and Prevention. Since 1987, the death rate due to diabetes has increased by 45 percent, even as death rates due to heart disease, stroke, and cancer have declined.

With the stakes this high, America's pharmaceutical companies are committed to developing new medicines to treat diabetes.

According to the ADA, most Americans with diabetes have type 2, in which relative insulin deficiency combines with the body failing to properly use insulin. Between 5 percent and 10 percent of Americans with diabetes have type 1, in which the body fails to produce insulin.

Diabetes is associated with an increased risk for a number of serious, sometimes life-threatening, complications. These include heart disease and stroke, high blood pressure, blindness, kidney disease, nervous system disease, amputations, dental disease, complications of pregnancy, and sexual dysfunction. For example, adults with diabetes have heart disease death rates about two to four times higher than adults without diabetes.

While lifestyle changes such as weight loss combined with moderate physical activity can reduce the risk for



developing type 2 diabetes, medicines have been critical to improving and lengthening the lives of those suffering from it.

The new medicines in development include:

- A once-weekly medicine that is an analog of a natural human hormone that plays a significant role in blood sugar regulation.
- A drug that addresses the underlying cause of type 2 diabetes by modulating genes responsible for insulin sensitization.
- A once-daily medicine that selectively inhibits a protein associated with glucose metabolism, but not other related proteins associated with other biologic activity.

While diabetes remains a formidable foe, America's pharmaceutical research and biotechnology companies are continuing their efforts to develop novel and more effective therapies to contain the disease and increase patients' quality of life.

Billy Tauzin  
President and CEO  
PhRMA

# Medicines in Development for Diabetes

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status*
11βHSD inhibitor	Bristol-Myers Squibb <i>Princeton, NJ</i>	diabetes	in clinical trials (212) 546-4000
376501 (PPAR gamma partial agonist)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	type 2 diabetes	Phase I (888) 825-5249
625019 (PPAR agonist)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	metabolic syndrome, type 2 diabetes	Phase I (888) 825-5249
756050	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	type 2 diabetes	Phase II (888) 825-5249
1292263 (bombesin receptor agonist)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	type 2 diabetes	Phase I (888) 825-5249
1362885 (glycogen phosphorylase inhibitor)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	type 2 diabetes	Phase I completed (888) 825-5249
1614235 (SGLT1 inhibitor)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	type 2 diabetes	Phase I (888) 825-5249
<b>Afrezza™</b> insulin inhalation	MannKind <i>Valencia, CA</i>	type 1 diabetes, type 2 diabetes	application submitted (661) 775-5300
AI-401	AutoImmune <i>Pasadena, CA</i>	type 1 diabetes (prevention)	Phase III (626) 792-1235
<b>Albulin™</b> albumin/insulin	Teva Pharmaceuticals USA <i>North Wales, PA</i>	type 1 diabetes, type 2 diabetes	Phase I (215) 591-3000
aleglitazar (RG1439)	Roche <i>Nutley, NJ</i>	type 2 diabetes	Phase III (973) 235-5000
alogliptin	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	type 2 diabetes (once-daily treatment)	application submitted (224) 554-6500
alogliptin/ metformin (fixed-dose combination)	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	diabetes	Phase III (224) 554-6500
alogliptin/ pioglitazone (fixed-dose combination)	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	type 2 diabetes	application submitted (224) 554-6500
<b>Alveair™</b> inhaled insulin	Coremed <i>Lake Bluff, IL</i>	diabetes	Phase I (847) 549-9098
AMG 151	Amgen <i>Thousand Oaks, CA</i> Array BioPharma <i>Boulder, CO</i>	type 2 diabetes	Phase I (805) 447-1000 (877) 633-2436

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

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
AMG 221	Amgen <i>Thousand Oaks, CA</i>	type 2 diabetes	Phase I (805) 447-1000
AMG 222	Amgen <i>Thousand Oaks, CA</i>	type 2 diabetes	Phase II (805) 447-1000
APD597	Arena Pharmaceuticals <i>San Diego, CA</i> Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	type 2 diabetes	Phase I (858) 453-7200 (800) 817-5286
AR9281	Arete Therapeutics <i>Hayward, CA</i>	type 2 diabetes	Phase II (510) 785-7060
ARI-2243	Arisaph Pharmaceuticals <i>Boston, MA</i>	type 2 diabetes	Phase I (617) 292-3322
ASP-1941	Astellas Pharma US <i>Deerfield, IL</i>	type 2 diabetes	Phase II (800) 695-4321
ASP-4178	Astellas Pharma US <i>Deerfield, IL</i>	type 2 diabetes	Phase I (800) 695-4321
AT1391 (daily insulin skin patch)	Altea Therapeutics <i>Atlanta, GA</i>	type 1 diabetes ----- type 2 diabetes	Phase I/II (404) 835-6310 ----- Phase I (404) 835-6310
ATL-844	PGxHealth <i>New Haven, CT</i>	type 2 diabetes	Phase II (877) 274-9432
<b>Avandamet® XR</b> rosiglitazone/ metformin extended-release	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	type 2 diabetes	Phase III (888) 825-5249
AZD1656	AstraZeneca <i>Wilmington, DE</i>	type 2 diabetes ----- type 1 diabetes	Phase II (800) 236-9933 ----- Phase I (800) 236-9933
AZD4017	AstraZeneca <i>Wilmington, DE</i>	type 2 diabetes	Phase I (800) 236-9933
AZD6370	AstraZeneca <i>Wilmington, DE</i>	type 2 diabetes	Phase II (800) 236-9933
AZD6714	AstraZeneca <i>Wilmington, DE</i>	type 2 diabetes	Phase I (800) 236-9933
AZD7687	AstraZeneca <i>Wilmington, DE</i>	type 2 diabetes	Phase I (800) 236-9933
AZD8329	AstraZeneca <i>Wilmington, DE</i>	type 2 diabetes	Phase I (800) 236-9933

# DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
AZP-01	Alize Pharma <i>Ecully, France</i> Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase I (800) 545-5979
basal insulin analog	Eli Lilly <i>Indianapolis, IN</i>	diabetes	Phase I (800) 545-5979
BGP-15	N-Gene Research Laboratories <i>New York, NY</i>	type 2 diabetes	Phase II (212) 605-0225
BI-10773	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	type 2 diabetes	Phase II (800) 243-0127
BLX 1002	BEXEL Pharmaceuticals <i>Union City, CA</i>	type 2 diabetes	Phase II (510) 324-0015
<b>Byetta</b> <sup>®</sup> exenatide	Amylin Pharmaceuticals <i>San Diego, CA</i> Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes in adolescents	Phase III (858) 552-2200 (800) 545-5979
canagliflozin	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i> Mitsubishi Pharma America <i>Warren, NJ</i>	type 2 diabetes	Phase III (800) 817-5286 (908) 607-1950
CCR2 antagonist	Bristol-Myers Squibb <i>Princeton, NJ</i>	diabetes	in clinical trials (212) 546-4000
CCX140	ChemoCentryx <i>Mountain View, CA</i>	type 2 diabetes	Phase II (650) 210-2900
cetilistat	Norgine <i>Amsterdam, The Netherlands</i>	type 2 diabetes in clinically obese patients	Phase II <a href="http://www.norgine.com">www.norgine.com</a>
CORT001	Cortendo <i>Halmstad, Sweden</i>	metabolic syndrome	Phase II <a href="http://www.cortendo.com">www.cortendo.com</a>
CRx-401	CombinatoRx <i>Cambridge, MA</i>	type 2 diabetes	Phase II (617) 301-7000
dapagliflozin (SGLT2 inhibitor)	AstraZeneca <i>Wilmington, DE</i> Bristol-Myers Squibb <i>Princeton, NJ</i>	type 2 diabetes	Phase III (800) 236-9933 (212) 546-4000
dapagliflozin/ metformin (fixed-dose combination)	AstraZeneca <i>Wilmington, DE</i> Bristol-Myers Squibb <i>Princeton, NJ</i>	type 2 diabetes ----- type 2 diabetes (with extended-release metformin)	Phase III (212) 546-4000  Phase I (212) 546-4000
DC9703	Obio Pharmaceutical (Omega Bio-Pharma) <i>Wilmington, DE</i>	type 2 diabetes	Phase I <a href="http://www.obiopharma.com">www.obiopharma.com</a>

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
<b>Diamyd®</b> autoimmune diabetes vaccine <b>(Orphan Drug)</b>	Diamyd <i>Pittsburgh, PA</i>	type 1 diabetes	Phase III (412) 770-1310
<b>DiaPep277®</b>	Andromeda Biotech <i>Ness Ziona, Israel</i>	latent autoimmune diabetes in adults	Phase II
DSP-8658	Dainippon Sumitomo Pharma America <i>Fort Lee, NJ</i>	type 2 diabetes	Phase I (201) 592-2050
dutogliptin	Forest Laboratories <i>New York, NY</i> Phenomix <i>San Diego, CA</i>	type 2 diabetes	Phase III (800) 947-5227 (858) 731-5200
<b>Enbrel®</b> etanercept	Amgen <i>Thousand Oaks, CA</i>	metabolic syndrome	Phase II (805) 447-1000
EX-1000	Novo Nordisk <i>Princeton, NJ</i>	type 2 diabetes	Phase II (800) 727-6500
exenatide intranasal	Amylin Pharmaceuticals <i>San Diego, CA</i> MDRNA <i>Bothell, WA</i>	type 2 diabetes	Phase I completed (858) 552-2200 (425) 908-3600
exenatide once weekly	Alkermes <i>Cambridge, MA</i> Amylin Pharmaceuticals <i>San Diego, CA</i> Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	application submitted (617) 494-0170 (858) 552-2200 (800) 545-5979
exenatide once weekly (suspension)	Amylin Pharmaceuticals <i>San Diego, CA</i> Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase I/II (858) 552-2200 (800) 545-5979
exenatide transdermal	Altea Therapeutics <i>Atlanta, GA</i> Amylin Pharmaceuticals <i>San Diego, CA</i> Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase I (404) 835-6310 (858) 552-2200 (800) 545-5979
FGF-21 variant	Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase I (800) 545-5979
<b>Glinsuna™</b> mitiglinide	Elixir Pharmaceuticals <i>Cambridge, MA</i>	type 2 diabetes	Phase III completed (617) 995-7000
GLP-1 (oral)	Emisphere Technologies <i>Cedar Knolls, NJ</i>	type 2 diabetes	Phase I/II completed (973) 532-8000
GLP-1 FC	Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase III (800) 545-5979
GLP-1 PEG	Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase II (800) 545-5979

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
glucokinase activator	Bristol-Myers Squibb <i>Princeton, NJ</i>	diabetes	in clinical trials (212) 546-4000
GS 9667	Gilead Sciences <i>Foster City, CA</i>	diabetes/hypertriglyceridemia	Phase I (800) 445-3235
HDV-insulin	Diasome Pharmaceuticals <i>Conshohocken, PA</i>	type 2 diabetes	Phase II (609) 923-9443
HE3286	Harbor BioSciences <i>San Diego, CA</i>	type 2 diabetes	Phase II (858) 587-9333
HSD016	Pfizer <i>New York, NY</i>	diabetes	Phase I (860) 732-5156
IL-1 $\beta$ antibody	Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase II (800) 545-5979
<b>Ilaris</b> <sup>®</sup> canakinumab	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	type 2 diabetes	Phase II/III (888) 669-6682
INCB-13739	Incyte <i>Wilmington, DE</i>	type 2 diabetes	Phase II (302) 498-6700
INCB-20817	Incyte <i>Wilmington, DE</i>	type 2 diabetes	Phase I completed (302) 498-6700
INGAP peptide	Exsulin <i>Minneapolis, MN</i>	type 1 diabetes, type 2 diabetes	Phase II completed <a href="http://www.exsulin.com">www.exsulin.com</a>
insulin inhalation	Baxter Healthcare <i>Deerfield, IL</i>	diabetes	Phase I (800) 422-9837
insulin inhalation	MicroDose Therapeutx <i>Monmouth Junction, NJ</i>	diabetes	Phase I completed (732) 355-2100
insulin oral	Emisphere Technologies <i>Cedar Knolls, NJ</i>	type 2 diabetes	Phase I/II (973) 532-8000
insulin oral	Generex Biotechnology <i>Toronto, Canada</i>	type 1 diabetes	Phase III (416) 364-2551
insulin transdermal	Dermisonics <i>Irvine, CA</i>	type 1 diabetes, type 2 diabetes	Phase I (888) 401-3376
INT-131	InteKrin Therapeutics <i>Los Altos, CA</i>	type 2 diabetes	Phase II (650) 941-5501
INT-747	Intercept Pharmaceuticals <i>New York, NY</i>	type 2 diabetes	Phase II (646) 747-1000
<b>Intesulin</b> <sup>™</sup> insulin oral	Coremed <i>Lake Bluff, IL</i>	diabetes	Phase I (847) 772-6868
ISIS-113715	Isis Pharmaceuticals <i>Carlsbad, CA</i>	type 2 diabetes (combination therapy)	Phase II (800) 679-4747
ISIS-GCGRx	Isis Pharmaceuticals <i>Carlsbad, CA</i>	type 2 diabetes	Phase I (800) 679-4747
ISIS-SGLT2rx	Isis Pharmaceuticals <i>Carlsbad, CA</i>	type 2 diabetes	Phase I (800) 679-4747
ITCA 650	Intarcia Therapeutics <i>Hayward, CA</i>	type 2 diabetes	Phase II (510) 782-7800

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
Januvia® sitagliptin	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes (monotherapy)	Phase III (800) 672-6372
		type 2 diabetes (in adolescents)	Phase I (800) 672-6372
JNJ-16268110	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	type 2 diabetes	Phase II (800) 817-5286
JNJ-38431055	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	type 2 diabetes	Phase I (800) 817-5286
JTT-654	Akros Pharma <i>Princeton, NJ</i> Japan Tobacco <i>Tokyo, Japan</i>	type 2 diabetes	Phase I (609) 919-9570 www.jt.com
K-111	Kowa Pharmaceuticals America <i>Montgomery, AL</i>	type 2 diabetes	Phase II (334) 288-1288
KRP-104	ActivX Biosciences <i>La Jolla, CA</i>	type 2 diabetes	Phase II (858) 558-5558
LCQ908	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	type 2 diabetes	Phase II (888) 669-6682
lisofylline	DiaKine Therapeutics <i>Charlottesville, VA</i>	type 1 diabetes (newly diagnosed)	Phase I (434) 975-2001
<b>LixiLan</b> lixisenatide/ insulin glargine combination	sanofi-aventis <i>Bridgewater, NJ</i>	type 2 diabetes	Phase I (800) 633-1610
lixisenatide (AVE0010; GLP1 agonist)	sanofi-aventis <i>Bridgewater, NJ</i>	type 2 diabetes	Phase III (800) 633-1610
LX-4211	Lexicon Pharmaceuticals <i>The Woodlands, TX</i>	type 2 diabetes	Phase II (281) 863-3000
LY2405319	Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase I (800) 545-5979
LY2599506 (glukokinase activator)	Eli Lilly <i>Indianapolis, IN</i> (OSI) Prosidion <i>Melville, NY</i>	type 2 diabetes	Phase II (800) 545-5979 (631) 962-2000
LY2605541	Eli Lilly <i>Indianapolis, IN</i>	type 2 diabetes	Phase II (800) 545-5979
MAR701	Marcadia Biotech <i>Carmel, IN</i>	type 2 diabetes	Phase I (317) 566-2266
MB07803	Metabasis Therapeutics <i>La Jolla, CA</i>	type 2 diabetes	Phase II completed (858) 587-2770

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
MBX-102 (JNJ-39659100)	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i> Metabolex <i>Hayward, CA</i>	type 2 diabetes	Phase II/III completed (800) 817-5286 (510) 293-8800
MBX-2044	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i> Metabolex <i>Hayward, CA</i>	type 2 diabetes	Phase II (800) 817-5286 (510) 293-8800
MBX-2982	Metabolex <i>Hayward, CA</i>	type 2 diabetes	Phase II (510) 293-8800
<b>Metgluna™</b> mitiglinide/ metformin (fixed-dose combination)	Elixir Pharmaceuticals <i>Cambridge, MA</i>	type 2 diabetes	Phase III (617) 995-7000
<b>Mitoglitazone™</b> MSDC-0160	Metabolic Solutions Development <i>Kalamazoo, MI</i>	type 2 diabetes	Phase II completed (616) 464-5071
MK-0599	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase I (800) 672-6372
MK-0893	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase II (800) 672-6372
MK-0941	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase II (800) 672-6372
MK-1006	Merck <i>Whitehouse Station, NJ</i>	diabetes	Phase I (800) 672-6372
MK-3102	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase I (800) 672-6372
MK-3577	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase II (800) 672-6372
MK-4074	Merck <i>Whitehouse Station, NJ</i>	diabetes	Phase I (800) 672-6372
MK-8245	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase II (800) 672-6372
MKC253 (glucagon-like peptide-1 inhalation)	Mannkind <i>Valencia, CA</i>	type 2 diabetes	Phase I (661) 775-5300
<b>Naturlose™</b> tagatose	Spherix <i>Bethesda, MD</i>	type 2 diabetes	Phase III (301) 897-2540
NBI-6024	Neurocrine Biosciences <i>San Diego, CA</i>	type 1 diabetes	Phase II (858) 658-7600

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
NN1250 (degludec)	Novo Nordisk <i>Princeton, NJ</i>	type 1 diabetes, type 2 diabetes	Phase III (800) 727-6500
NN1952	Novo Nordisk <i>Princeton, NJ</i>	diabetes	Phase I (800) 727-6500
NN5401 (degludec plus)	Novo Nordisk <i>Princeton, NJ</i>	type 1 diabetes, type 2 diabetes	Phase III (800) 727-6500
NN9068	Novo Nordisk <i>Princeton, NJ</i>	type 2 diabetes	Phase I (800) 727-6500
NN9535 (semaglutide)	Novo Nordisk <i>Princeton, NJ</i>	type 2 diabetes	Phase II (800) 727-6500
NN9924	Novo Nordisk <i>Princeton, NJ</i>	type 2 diabetes	Phase I (800) 727-6500
NN9925	Novo Nordisk <i>Princeton, NJ</i>	type 2 diabetes	Phase I (800) 727-6500
<b>Norditropin®</b> somatropin	Novo Nordisk <i>Princeton, NJ</i>	insulin resistance	Phase I (800) 727-6500
NOX-700	Medinox <i>Carlsbad, CA</i>	type 2 diabetes	Phase I (760) 603-8989
NP-500	Napo Pharmaceuticals <i>South San Francisco, CA</i>	metabolic syndrome, type 2 diabetes	Phase I (650) 616-1900
OAP-189	Pfizer <i>New York, NY</i>	type 2 diabetes	Phase I (860) 732-5156
<b>Ondero®</b> linagliptin	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	type 2 diabetes	Phase III (800) 243-0127
otelixizumab <b>(Orphan Drug)</b>	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i> Tolerx <i>Cambridge, MA</i>	type 1 diabetes	Phase III (888) 825-5249 (617) 354-8100
PEG-encapsulated islet cell transplantation therapy	Novocell <i>San Diego, CA</i> Pfizer <i>New York, NY</i>	type 1 diabetes	Phase I/II (858) 455-3708 (860) 732-5156
PF-4620110 (DGAT)	Bristol-Myers Squibb <i>Princeton, NJ</i> Pfizer <i>New York, NY</i>	type 2 diabetes	Phase I (212) 546-4000 (860) 732-5156
PF-4856883	Pfizer <i>New York, NY</i>	type 2 diabetes	Phase I (860) 732-5156
PF-4937319	Pfizer <i>New York, NY</i>	type 2 diabetes	Phase I (860) 732-5156
PF-4971729	Pfizer <i>New York, NY</i>	type 2 diabetes	Phase II (860) 732-5156
PF-5212389	Pfizer <i>New York, NY</i>	diabetes	Phase I (860) 732-5156

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
PLX-204	Plexxikon <i>Berkeley, CA</i>	type 2 diabetes	Phase II (510) 647-4000
<b>Prochymal™</b> human mesenchymal stem cell therapy	Osiris Therapeutics <i>Columbia, MD</i>	type 1 diabetes	Phase II (443) 545-1800
pyrazinoyl- guanidine	SuperGen <i>Dublin, CA</i>	type 2 diabetes	Phase II (925) 560-0100
<b>Qnexa®</b> phentermine/ topiramate combination	VIVUS <i>Mountain View, CA</i>	type 2 diabetes in obese patients	Phase II (650) 934-5200
RG4929	Roche <i>Nutley, NJ</i>	type 2 diabetes	Phase I (973) 235-5000
RG7089	Roche <i>Nutley, NJ</i>	type 2 diabetes	Phase I (973) 235-5000
RG7201	Roche <i>Nutley, NJ</i>	type 2 diabetes	Phase II (973) 235-5000
RG7234	Roche <i>Nutley, NJ</i>	type 2 diabetes	Phase I (973) 235-5000
RG7426 (BHT-3021)	Bayhill Therapeutics <i>Palo Alto, CA</i> Genentech <i>South San Francisco, CA</i>	type 1 diabetes	Phase I/II (800) 626-3553
rHuPH20	Halozyme Therapeutics <i>San Diego, CA</i>	type 1 diabetes, type 2 diabetes	Phase II (858) 794-8889
RO-4998452	Roche <i>Nutley, NJ</i>	type 2 diabetes	Phase II (973) 235-5000
SAR16127 (very long-acting insulin analog)	sanofi-aventis <i>Bridgewater, NJ</i>	diabetes	Phase I (800) 633-1610
SAR176975 (insulin sensitizer)	sanofi-aventis <i>Bridgewater, NJ</i>	type 2 diabetes	Phase II (800) 633-1610
saxagliptin/ metformin combination therapy	AstraZeneca <i>Wilmington, DE</i> Bristol-Myers Squibb <i>Princeton, NJ</i>	type 2 diabetes	application submitted (800) 236-9933 (212) 546-4000
sitagliptin/ pioglitazone (fixed-dose combination)	Merck <i>Whitehouse Station, NJ</i>	type 2 diabetes	Phase III (800) 672-6372
SLV337	Abbott Laboratories <i>Abbott Park, IL</i>	type 2 diabetes	Phase I/II (847) 937-6100
SLV341	Abbott Laboratories <i>Abbott Park, IL</i>	type 2 diabetes	Phase I (847) 937-6100

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
sodelglitazar	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	metabolic syndrome, type 2 diabetes	Phase II (888) 825-5249
SRT501	Sirtris (GlaxoSmithKline) <i>Cambridge, MA</i>	type 2 diabetes	Phase II completed (617) 252-6920
SRT2104	Sirtris (GlaxoSmithKline) <i>Cambridge, MA</i>	type 2 diabetes	Phase II (617) 252-6920
SRT2379	Sirtris (GlaxoSmithKline) <i>Cambridge, MA</i>	type 2 diabetes	Phase I (617) 252-6920
<b>Syncrion</b> <sup>®</sup> albiglutide	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	type 2 diabetes	Phase III (888) 825-5249
SYR-472	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	type 2 diabetes	Phase II (224) 554-1200
TA-6666	Mitsubishi Pharma America <i>Warren, NJ</i>	type 2 diabetes	Phase II (908) 607-1950
TAK-875	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	type 2 diabetes	Phase II (224) 554-1000
tasoglutide (RG1583)	Roche <i>Nutley, NJ</i>	type 2 diabetes	Phase III (973) 235-5000
teneligliptin	Mitsubishi Pharma America <i>Warren, NJ</i>	type 2 diabetes	Phase I (908) 607-1950
teplizumab <b>(Orphan Drug)</b>	Eli Lilly <i>Indianapolis, IN</i> Macrogenics <i>Rockville, MD</i>	type 1 diabetes	Phase III (800) 545-5979 (301) 251-5172
THR-0921	Theracos <i>Sunnyvale, CA</i>	type 2 diabetes	Phase II (408) 992-1100
TT-223 + EGF analog	Transition Therapeutics <i>Toronto, Canada</i>	type 1 diabetes, type 2 diabetes	Phase II (416) 260-7770
TT-223 + GLP-1 analog	Transition Therapeutics <i>Toronto, Canada</i>	type 1 diabetes, type 2 diabetes	Phase I (416) 260-7770
TT-223 + metformin	Transition Therapeutics <i>Toronto, Canada</i>	type 2 diabetes	Phase I (416) 260-7770
TTP054	TransTech Pharma <i>High Point, NC</i>	type 2 diabetes	Phase I/II (336) 841-0300
TTP399	TransTech Pharma <i>High Point, NC</i>	type 2 diabetes	Phase I/II (336) 841-0300
TTP814	TransTech Pharma <i>High Point, NC</i>	type 2 diabetes	Phase I/II (336) 841-0300

## DIABETES, TYPE 1 AND TYPE 2

Product Name	Sponsor	Indication	Development Status
VGX-1027	VGX Pharmaceuticals <i>Blue Bell, PA</i>	type 1 diabetes	Phase I completed (267) 440-4200
<b>VIAject™</b> insulin injectable	Biodel <i>Danbury, CT</i>	type 1 diabetes, type 2 diabetes	application submitted (203) 796-5000
<b>VIAtab™</b> insulin oral sublingual	Biodel <i>Danbury, CT</i>	type 1 diabetes	Phase I (203) 796-5000
vildagliptin	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	type 2 diabetes	Phase III (888) 669-6682
		type 2 diabetes in treatment-naive patients	Phase II/III (888) 669-6682
vildagliptin/ metformin combination	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	type 2 diabetes	application submitted (888) 669-6682
XOMA052	XOMA <i>Berkeley, CA</i>	type 2 diabetes	Phase II (510) 204-7200

## DIABETES-RELATED CONDITIONS

Product Name	Sponsor	Indication	Development Status
962040 (motilin receptor agonist)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	diabetic gastroparesis	Phase II (888) 825-5249
ABT-335	Abbott Laboratories <i>Abbott Park, IL</i>	diabetic macular edema	Phase II (847) 937-6100
AEG33773	Aegera Therapeutics <i>Montreal, Canada</i>	painful diabetic neuropathies	Phase II (514) 288-5532
aflibercept (VEGF-trap) (intravitreal)	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i> Regeneron Pharmaceuticals <i>Tarrytown, NY</i>	diabetic macular edema	Phase II (888) 842-2937 (914) 345-7400
AGN-203818	ACADIA Pharmaceuticals <i>San Diego, CA</i> Allergan <i>Irvine, CA</i>	diabetic neuropathies	Phase II completed (858) 558-2871 (714) 246-4500
AL-39324	Alcon <i>Fort Worth, TX</i>	diabetic retinopathy	Phase I/II (800) 862-5266
APN01	Apeiron Biologics <i>Vienna, Austria</i> GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	diabetic nephropathies	Phase I www.apeiron-biologics.com (888) 825-5249

## DIABETES-RELATED CONDITIONS

Product Name	Sponsor	Indication	Development Status
ARC-4558 (clonidine topical gel)	Arcion Therapeutics <i>Baltimore, MD</i>	diabetic neuropathies	Phase II (410) 522-8701
<b>Arxxant™</b> ruboxistaurin	Alcon <i>Fort Worth, TX</i> Eli Lilly <i>Indianapolis, IN</i>	diabetic retinopathy	application submitted (800) 545-5979
		diabetic macular edema	Phase III (800) 545-5979
AS-3201	Eisai <i>Woodcliff Lake, NJ</i>	diabetic neuropathy	Phase II/III (888) 422-4743
ATG-3	CoMentis <i>South San Francisco, CA</i>	diabetic macular edema	Phase II (650) 869-7600
AZD2066	AstraZeneca <i>Wilmington, DE</i>	painful diabetic neuropathies	Phase II (800) 236-9933
bardoxolone methyl	Reata Pharmaceuticals <i>Irving, TX</i>	diabetic nephropathies	Phase II (972) 865-2219
carisbamate	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	diabetic neuropathies	Phase II (800) 817-5286
coleneuramide	Mitsubishi Tanabe Pharma America <i>Warren, NJ</i>	diabetic neuropathies	Phase II www.mt-pharma- america.com
<b>CollaRx®</b> gentamicin implant	Baxter Healthcare <i>Deerfield, IL</i>	diabetic foot ulcer	Phase II (800) 422-9837
<b>Diovan®</b> valsartan	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	diabetic nephropathies	Phase III (888) 669-6682
DSC-127	Derma Sciences <i>Princeton, NJ</i>	diabetic foot ulcer	Phase II (609) 514-4744
EVK-001	Evoke Pharma <i>San Diego, CA</i>	diabetic gastroparesis	Phase II (858) 967-5454
FG 3019	FibroGen <i>San Francisco, CA</i>	diabetic nephropathies	Phase II (415) 978-1200
FGF-1	CardioVascular BioTherapeutics <i>Las Vegas, NV</i>	diabetic foot ulcer	Phase I (702) 839-7200
<b>Gabapentin GR™</b> gabapentin controlled-release	Depomed <i>Menlo Park, CA</i>	diabetic neuropathies	Phase II (650) 462-5900
GAM 501	Cardium Therapeutics <i>San Diego, CA</i>	diabetic foot ulcer	Phase II completed (858) 436-1000

## DIABETES-RELATED CONDITIONS

Product Name	Sponsor	Indication	Development Status
GLY-230	Glycadia Pharmaceuticals <i>Philadelphia, PA</i>	diabetic nephropathy	Phase II (215) 557-8021
		diabetic retinopathy	Phase I (215) 557-8021
ICO-007	iCo Therapeutics <i>Vancouver, Canada</i>	diabetic macular edema	Phase I (604) 602-9414
<b>Iluvien™</b> fluocinolone acetone ophthalmic	Alimera Sciences <i>Alpharetta, GA</i> pSivida <i>Watertown, MA</i>	diabetic macular edema	Phase III (678) 990-5740 (617) 926-5000
indantadol (V3881)	Vernalis <i>Winnersh, United Kingdom</i>	diabetic neuropathic pain	Phase II www.vernalis.com
INV-064	InVasc Therapeutics <i>Tucker, GA</i>	diabetic complications	Phase II (678) 736-5900
iroxanadine	CytRx <i>Los Angeles, CA</i>	diabetic foot ulcer, diabetic neuropathies	Phase I (310) 826-5648
<b>Lipitor®</b> atorvastatin	Pfizer <i>New York, NY</i>	diabetic foot ulcer	Phase II (860) 732-5156
<b>Lucentis®</b> ranibizumab	Genentech <i>South San Francisco, CA</i>	diabetic macular edema	Phase III (800) 626-3553
LY545694	Eli Lilly <i>Indianapolis, IN</i>	diabetic peripheral neuropathic pain	Phase II (800) 545-5979
<b>Macugen®</b> pegaptanib	Eyetech <i>Palm Beach Gardens, FL</i> Pfizer <i>New York, NY</i>	diabetic macular edema, diabetic retinopathy	Phase III www.eyetech.com (860) 732-5156
<b>NanoDOX™</b> doxycycline topical gel	Nanotherapeutics <i>Alachua, FL</i>	diabetic foot ulcer	Phase II (386) 462-9663
nemonoxacin	TaiGen Biotechnology <i>Taipei, Taiwan</i>	infections associated with diabetic foot ulcer	Phase II completed www.taigenbiotech.com
Nova63035	Novagali Pharma <i>Evry, France</i>	diabetic macular edema	Phase I www.novagali.com
<b>Optina™</b>	Ampio Pharmaceuticals <i>Greenwood Village, CO</i>	diabetic macular edema	Phase II (303) 418-1000
PF-3882845	Pfizer <i>New York, NY</i>	diabetic nephropathies	Phase I (860) 732-5156
PF-4494700	Pfizer <i>New York, NY</i>	diabetic nephropathies	Phase II (860) 732-5156
PF-4523655	Pfizer <i>New York, NY</i>	diabetic macular edema	Phase II (860) 732-5156
PF-4856880 (ADL-5859)	Adolor <i>Exton, PA</i> Pfizer <i>New York, NY</i>	neuropathic pain in patients with diabetic peripheral neuropathy	Phase II completed (484) 595-1500 (860) 732-5156

## DIABETES-RELATED CONDITIONS

Product Name	Sponsor	Indication	Development Status
<b>Posurdex</b> <sup>®</sup> dexamethasone ophthalmic	Allergan <i>Irvine, CA</i>	diabetic macular edema	Phase III (800) 433-8871
PS433540	Ligand Pharmaceuticals <i>La Jolla, CA</i>	diabetic nephropathies	Phase I (858) 550-7500
<b>Pyridorin</b> <sup>™</sup> pyridoxamine	NephroGenex <i>Princeton, NJ</i>	diabetic nephropathies	Phase II (609) 986-1780
QR-333	Quigley Pharma <i>Doylestown, PA</i>	diabetic neuropathies	Phase II completed (267) 880-1100
SAR 1118	SARcode <i>San Francisco, CA</i>	diabetic macular edema	Phase I www.sarcode.com
SB-509	Sangamo BioSciences <i>Richmond, CA</i>	diabetic neuropathies	Phase II (510) 970-6000
sirolimus ophthalmic	MacuSight <i>Union City, CA</i>	diabetic macular edema (subconjunctival)	Phase II (510) 400-1440
		diabetic macular edema (intravitreal)	Phase I (510) 400-1440
SKL-NP	SK Drug Development <i>Fair Lawn, NJ</i>	diabetic neuropathic pain	Phase I
<b>Solzira</b> <sup>™</sup> gabapentin enacarbil	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i> XenoPort <i>Santa Clara, CA</i>	diabetic neuropathies	Phase II (888) 825-5249
stem cell therapy	Stemedica Cell Technologies <i>San Diego, CA</i>	diabetic retinopathy	in clinical trials (858) 658-0910
TAK-428	Takeda Pharmaceuticals North America <i>Deerfield, IL</i>	diabetic neuropathies	Phase II (877) 582-5332
talactoferrin (topical)	Agennix <i>Princeton, NJ</i>	diabetic foot ulcer	Phase II (609) 524-1000
<b>Tekturna</b> <sup>®</sup> aliskiren	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	diabetic nephropathies (adjunctive treatment)	Phase III (888) 669-6682
		diabetic macular edema	Phase II (888) 669-6682
<b>Topamax</b> <sup>®</sup> topiramate	Johnson & Johnson Pharmaceutical Research & Development <i>Raritan, NJ</i>	diabetic neuropathies	Phase III (800) 817-5286
<b>Tygacil</b> <sup>®</sup> tigecycline	Pfizer <i>New York, NY</i>	diabetic foot ulcer	Phase III completed (860) 732-5156
<b>Vasaloc</b> <sup>™</sup>	Ampio Pharmaceuticals <i>Greenwood Village, CO</i>	diabetic nephropathies	Phase II (303) 418-1000

## DIABETES-RELATED CONDITIONS

Product Name	Sponsor	Indication	Development Status
<b>Vitreosolve™</b> diabetic retinopathy therapeutic	Vitreoretinal Technologies <i>Irvine, CA</i>	diabetic retinopathy	Phase III (877) 478-3301
<b>Zenvia™</b> dextromethorphan/ quinidine fixed-dose combination	Avanir Pharmaceuticals <i>Aliso Viejo, CA</i>	diabetic peripheral neuropathic pain	Phase III (949) 389-6700

The content of this report has been obtained through industry sources and the Adis “R&D Insight” database based on the latest information. **Report current as of April 21, 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](http://www.clinicaltrials.gov). The entire series of *Medicines in Development* is available on PhRMA’s web site.

A publication of PhRMA’s Communications & Public Affairs Department. (202) 835-3460

[www.phrma.org](http://www.phrma.org) | [www.innovation.org](http://www.innovation.org) | [www.pparx.org](http://www.pparx.org) | [www.buysafedrugs.info](http://www.buysafedrugs.info) | [www.sharingmiracles.com](http://www.sharingmiracles.com)

Provided as a Public Service by PhRMA. Founded in 1958 as the Pharmaceutical Manufacturers Association.

Copyright © 2010 by the Pharmaceutical Research and Manufacturers of America. Permission to reprint is awarded if proper credit is given.

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

**diabetes**—A chronic disease in which the body does not produce or properly use insulin, a hormone that is needed to convert sugar, starches and other food into energy needed for daily life. Symptoms may include excessive thirst, hunger, urination and weight loss. The cause of diabetes continues to be a mystery, although both genetics and environmental factors such as obesity and lack of exercise appear to play roles. **Type 1 diabetes**, the more severe form, results from the body's failure to produce insulin, which "unlocks" the cells of the body, allowing glucose to enter and fuel them. It is estimated that 5 percent to 10 percent of Americans who are diagnosed with diabetes have type 1, which requires insulin treatment. **Type 2 diabetes** results from insulin resistance (a condition in which the body fails to properly use insulin), combined with relative insulin deficiency. Most Americans who are diagnosed with diabetes have type 2, which in most cases can be controlled by a combination of dietary measures, weight loss, and oral medication.

**diabetic peripheral neuropathy**—Nerve damage in the arms, hands, legs, and feet caused by diabetes. The condition develops slowly and worsens over time. Depending on the types of nerves involved, one or more signs and symptoms may be present in diabetic peripheral neuropathy. *Sensory neuropathy* results in numbness or tingling in the feet or pain or discomfort in the feet or legs, including prickly, sharp pain or burning feet. *Motor neuropathy* involves muscle weakness and loss of muscle tone in the feet and lower legs, loss of balance, or changes in foot shape that can lead to areas of increased pressure. *Autonomic neuropathy* results in dry feet and cracked skin. The loss of sensation and other problems associated with nerve damage make a patient prone to developing skin ulcers (open sores) that can become infected and may not heal. This serious complication of diabetes

can lead to the loss of a foot, a leg, or even a life.

**diabetic retinopathy**—The most common diabetic eye disease and a leading cause of blindness in American adults. It is caused by changes in the blood vessels of the retina, the light-sensitive tissue at the back of the eye that is necessary for good vision. In some people with diabetic retinopathy, blood vessels may swell and leak fluid. In others, abnormal new blood vessels grow on the surface of the retina. Over time, diabetic retinopathy, which usually affects both eyes, can worsen and cause vision loss. The condition has four stages: 1) mild nonproliferative retinopathy, the earliest stage during which microaneurysms occur in the retina's tiny blood vessels; 2) moderate nonproliferative retinopathy, during which some blood vessels that nourish the retina are blocked; 3) severe nonproliferative retinopathy, when many more blood vessels are blocked and deprive several areas of the retina with their blood supply; and 4) proliferative retinopathy, the advanced stage when signals sent by the retina for nourishment trigger the growth of new blood vessels, which grow along the retina and the surface of the clear, vitreous gel that fills the inside of the eye. If those thin, fragile blood vessels leak blood, severe vision loss and even blindness can result. Up to 45 percent of Americans diagnosed with diabetes have some stage of diabetic retinopathy.

**insulin resistance**—A generalized metabolic disorder caused by the body's inability to use insulin efficiently. Some people are genetically predisposed to insulin resistance. Acquired factors, such as excess body fat and physical inactivity, also can elicit insulin resistance. Most people with insulin resistance have abdominal obesity. The biologic mechanisms at the molecular level between insulin resistance and metabolic risk factors aren't fully understood and appear to be complex.

**macular edema**—A condition in which fluid can leak into the center of the macula, the part of the eye where sharp, straight-ahead vision occurs.

The fluid makes the macula swell, thus blurring vision. It can occur at any stage of **diabetic retinopathy**, although it is more likely to occur as the disease progresses. About half of the people with proliferative retinopathy also have macular edema.

**metabolic syndrome**—A group of metabolic risk factors that raise the risk of type 2 diabetes in one person, including: abdominal obesity (excessive fat tissue in and around the abdomen); atherogenic dyslipidemia (blood fat disorders—high triglycerides, low HDL cholesterol, and high LDL cholesterol—that foster plaque buildups in artery walls); elevated blood pressure; insulin resistance or glucose intolerance (the body can't properly use insulin or blood sugar); prothrombotic state (e.g., high fibrinogen or plasminogen activator inhibitor-1 in the blood); proinflammatory state (e.g., elevated C-reactive protein in the blood). People with metabolic syndrome are at increased risk of coronary heart disease and other diseases related to plaque buildup in artery walls (e.g., stroke and peripheral vascular disease) and type 2 diabetes. Metabolic syndrome has become increasingly common in the United States—more than 50 million Americans are estimated to have it. The dominant underlying risk factors for the syndrome appear to be abdominal obesity and insulin resistance. Insulin resistance is a generalized metabolic disorder in which the body can't use insulin efficiently. That is why metabolic syndrome is also called *insulin resistance syndrome*. Other conditions associated with metabolic syndrome include physical inactivity, aging, hormonal imbalance, and genetic predisposition.

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

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

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

# SELECTED FACTS ABOUT DIABETES IN THE UNITED STATES\*

---

## Diabetes

- In the United States, 23.6 million people, or nearly 8 percent of the population, have diabetes. An estimated 17.9 million have been diagnosed, but 5.7 million people are not aware that they have the disease. Another 57 million have pre-diabetes.
  - If present trends continue, 1 in 3 Americans and 1 in 2 minorities born in 2000 will develop diabetes in their lifetime. Each day, approximately 4,110 people are diagnosed with diabetes. In 2007, 1.6 million new cases of diabetes were diagnosed in people age 20 or older.
  - In the age group under age 20, 186,300, or 0.22 percent, have diabetes. About 1 in every 400-600 children and adolescents has type 1 diabetes, which requires insulin. Another 2 million adolescents (or 1 in 6 overweight adolescents) ages 12-19 have pre-diabetes.
  - Among those age 20 and older, 23.5 million, or 10.7 percent, have diabetes. Among those age 60 and older, 12.2 million, or 23.1 percent, have diabetes.
  - Among men age 20 and older, 12 million, or 11.2 percent, have diabetes. Among women in the same age group, 11.5 million, or 10.2 percent, have diabetes. The prevalence of diabetes is nearly twice as high among non-Hispanic blacks, and Hispanic Americans as it is among non-Hispanic whites.
  - In 2007, diabetes accounted for 15 million work days absent, 120 million work days with reduced performance, 6 million reduced productivity days for those not in the workforce, and an additional 107 million work days lost due to unemployment disability attributed to diabetes. Diabetes caused 445,000 cases of unemployment disability in 2007.
  - Diabetes is the fifth deadliest disease in the United States. Since 1987, the death rate due to diabetes has increased by 45 percent, while the death rates due to heart disease, stroke, and cancer have declined. Based on death certificate data, diabetes contributed to 233,619 deaths in 2005. (Diabetes is generally under-reported on death certificates, particularly in the cases of older people who have multiple chronic conditions. As a result, the diabetes death toll is believed to be much higher than officially reported.)
- 

## Costs

- The total annual economic cost of diabetes in 2007 was estimated to be \$174 billion. Medical expenditures totaled \$116 billion, including \$27 billion for diabetes care, \$58 billion for chronic diabetes-related complications, and \$31 billion for excess general medical costs. Indirect costs resulting from increased absenteeism, reduced productivity, disease-related unemployment disability, and loss of productive capacity due to early mortality totaled \$58 billion.
  - People with diagnosed diabetes, on average, have medical expenditures that are some 2.3 times higher than for those without diabetes. The 2007 per capita annual costs of health care for people with diabetes is \$11,744 a year, of which \$6,649 (57 percent) is attributed to diabetes.
  - Diabetes-related hospitalizations totaled 24.3 million days in 2007, an increase of 7.4 million from the 16.9 million days in 2002. The average cost for a hospital inpatient day due to diabetes is \$1,853, and another \$2,281 is due to diabetes-related chronic conditions, including neurological, peripheral vascular, cardiovascular, renal, metabolic, and ophthalmic complications.
  - One out of every five health-care dollars is spent caring for someone with diagnosed diabetes, and one in 10 health-care dollars is attributed to diabetes.
- 

\***Source:** American Diabetes Association, [www.diabetes.org](http://www.diabetes.org)

## THE DRUG DISCOVERY, DEVELOPMENT AND APPROVAL PROCESS

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

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

## THE DRUG DEVELOPMENT AND APPROVAL PROCESS

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

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

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

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

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

**Investigational New Drug Application (IND).** After completing preclinical testing, a company files an IND with the U.S. Food and Drug Administration (FDA) to begin to test the drug in people. The IND 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 25 new therapeutics approved by the FDA in 2009 was 13.3 months.

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

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

# TRACKING THE 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 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 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**—Biopharmaceutical researchers are testing medicines to meet the special needs of children. These medicines offer hope that the significant improvements achieved in children's health over the past few decades will continue and even accelerate. *The 2010 report found 234 medicines in clinical trials.*
- **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.*



New Medicines. New Hope.®

Pharmaceutical Research and Manufacturers of America  
950 F Street, NW  
Washington, DC 20004