

# 100 GREAT INVESTIGATIONAL DRUGS

- AAE581** — Osteoporosis  
**ACAM2000** — Smallpox  
**ACC-001** — Alzheimer's disease  
**AEB071** — Organ transplant rejection  
**Aerugen** — *Pseudomonas aeruginosa* infection  
**AGI-1067** — Atherosclerosis  
**AGI-1096** — Organ transplant rejection  
**AMN107** — Chronic myeloid leukemia, acute lymphoblastic leukemia  
**Amplimexon** — Pancreatic cancer, melanoma, multiple myeloma, ovarian cancer, lung cancer, breast cancer, prostate cancer  
**AP23573** — Solid tumors, blood malignancies, soft tissue and bone sarcomas, prostate cancer, endometrial cancer  
**Arimoclomol** — Amyotrophic lateral sclerosis, type 2 diabetes  
**Asenapine** — Acute mania, bipolar disorder, schizophrenia  
**AT-101** — Cancer  
**AT-1001** — Celiac disease, type 1 diabetes  
**Avicine** — Colorectal cancer, pancreatic cancer  
**AVP-13358** — Allergies and asthma  
**Brecanavir** — HIV infection  
**Brivaracetam** — Symptomatic myoclonus  
**Ceftobiprole** — Complicated skin and skin-structure infections, nosocomial pneumonia  
**Cethrin** — Spinal cord injuries  
**CGX-1160** — Chronic intractable pain  
**Chrysalin** — Bone fractures, diabetic ulcers, myocardial revascularization, cartilage repair  
**Cimzia** — Rheumatoid arthritis, Crohn's disease, psoriasis  
**Cloretazine** — Acute myelogenous leukemia, brain tumor, chronic lymphocytic leukemia, small cell lung cancer, Richter syndrome  
**CPI-226** — Catheter-related bloodstream infections  
**Depelestat** — Cystic fibrosis, acute respiratory distress syndrome  
**DX-9065a** — Angina  
**EDP-420** — Community-acquired pneumonia  
**EGEN-001** — Ovarian cancer  
**Eniluracil** — Colorectal cancer, liver cancer, solid tumors  
**Epratuzumab** — Non-Hodgkin's lymphoma, systemic lupus erythematosus, Sjogren's syndrome  
**Ethynylcytidine** — Solid tumors  
**Favid** — Non-Hodgkin's lymphoma  
**Fodosine** — Acute lymphoblastic leukemia, T-cell leukemia  
**FX-1006a** — Familial genetic disorders  
**Genexol-PM** — Cancer  
**GRC 3886** — Chronic obstructive pulmonary disease, asthma  
**GS 9137** — HIV infection  
**GV1001** — Pancreatic cancer  
**HCT 3012** — Osteoarthritis  
**HuMax EGFr** — Head and neck cancer  
**IL-21** — Metastatic melanoma, renal cell carcinoma  
**Indacaterol** — Asthma, chronic obstructive pulmonary disease  
**INGN 241** — Solid tumors, malignant melanoma  
**INS37217 Respiratory** — Cystic fibrosis  
**ISIS 301012** — High cholesterol  
**KP-1461** — HIV infection  
**Liraglutide** — Type 2 diabetes  
**Lobeline** — Methamphetamine addiction  
**Maribavir** — Cytomegalovirus infection  
**MLN2704** — Prostate cancer  
**MT103** — Non-Hodgkin's lymphoma  
**NicVAX** — Nicotine addiction  
**OMS103HP** — Pain  
**OrBec** — Graft vs. Host disease  
**Org 25969** — Neuromuscular blocker  
**Org 50081** — Insomnia, menopausal symptoms  
**Peramivir** — Influenza  
**Picoplatin** — Small cell lung cancer, colorectal cancer  
**Pixantrone** — Non-Hodgkin's lymphoma, acute myelogenous leukemia  
**PolyHeme** — Blood substitute  
**Posurdex** — Macular edema  
**Prochymal** — Graft vs. Host disease  
**Proxinium** — Head and neck cancer  
**PRX-03140** — Alzheimer's disease  
**PSMA Vaccine** — Prostate cancer  
**PT-141** — Erectile dysfunction, female sexual dysfunction  
**R788** — Rheumatoid arthritis  
**RC-1291** — Cachexia and anorexia  
**Remoxy** — Pain  
**Respicort** — Asthma  
**REV131** — Allergic rhinitis  
**Rexin-G** — Pancreatic cancer  
**Rifalazil** — Gastritis and peptic ulcer disease, nongonococcal urethritis, peripheral arterial disease, *Clostridium difficile*-associated disease  
**RNAPc2** — Acute coronary syndromes  
**RSV604** — Respiratory syncytial virus  
**SC12267** — Autoimmune diseases  
**SCH 503034** — Hepatitis C  
**SGN-40** — Multiple myeloma, non-Hodgkin's lymphoma, chronic lymphocytic leukemia  
**Sirna-027** — Age-related macular degeneration  
**StemEx** — Hematological malignancies  
**SUN N8075** — Acute cerebral infarction  
**Tavocept** — Paclitaxel-related toxicities  
**Telavancin** — Hospital-acquired pneumonia, complicated skin and skin-structure infections  
**Terlipressin** — Type 1 hepatorenal syndrome  
**TG-873870** — Drug-resistant bacterial infections  
**TheraCLEC** — Malabsorption due to pancreatic insufficiency  
**Tilarginine Acetate Injection** — Cardiogenic shock  
**Traficet-EN** — Inflammatory bowel disease  
**TransMID** — Glioblastoma multiforme  
**Valortim** — Anthrax  
**Velafermin** — Inflammatory bowel disease, oral mucositis  
**Vivagel** — HIV infection  
**VRX496** — HIV infection  
**XL880** — Solid tumors  
**XP12B** — Heavy menstrual bleeding  
**YM443** — Functional dyspepsia  
**Yondelis** — Ovarian cancer  
**Zactima** — Nonsmall cell lung cancer, thyroid cancer  
**ZIO-101** — Solid tumors, hematologic cancer, myeloma

## DEVELOPERS OF 100 GREAT INVESTIGATIONAL DRUGS

**Novartis Pharmaceuticals Corp.**

**AAE581** — Osteoporosis  
**AEB071** — Organ transplant rejection  
**AMN107** — Chronic myeloid leukemia, acute lymphoblastic leukemia  
**Indacaterol** — Asthma, chronic obstructive pulmonary disease  
**RSV604** — Respiratory syncytial virus

**Allergan Inc.**

**Posurdex** — Macular edema  
**Sirna-027** — Age-related macular degeneration  
**YM443** — Functional dyspepsia

**Organon Pharmaceuticals USA**

**Asenapine** — Acute mania, schizophrenia  
**Org 25969** — Neuromuscular blocker  
**Org 50081** — Insomnia, menopausal symptoms

**Astellas Pharma US Inc.**

**AGI-1096** — Organ transplant rejection  
**Telavancin** — Hospital-acquired pneumonia, complicated skin and skin-structure infections

**BioCryst Pharmaceuticals Inc.**

**Fodosine** — Acute lymphoblastic leukemia, T-cell leukemia  
**Peramivir** — Influenza

**GlaxoSmithKline Inc.**

**Breacanvir** — HIV infection  
**Ethinylcytidine** — Solid tumors

**Johnson & Johnson**

**Pharmaceutical**  
**Ceftobiprole** — Complicated skin and skin-structure infections, nosocomial pneumonia  
**Yondelis** — Ovarian cancer

**UCB Pharma Inc.**

**Brivaracetam** — Symptomatic myoclonus  
**Cimzia** — Rheumatoid arthritis, Chron's disease

**Acambis Plc.**

**ACAM2000** — Smallpox

**ActivBiotics Inc.**

**Rifalazil** — Gastritis and peptic ulcer disease, non-gonococcal urethritis, peripheral arterial disease, Clostridium difficile-associated disease

**Adherex Technologies Inc.**

**Eniluracil** — Colorectal cancer, liver cancer, solid tumors

**Alba Therapeutics Corp.**

**AT-1001** — Celiac disease

**Altus Biologics Inc.**

**TheraCLEC** — Malabsorption due to pancreatic insufficiency

**Amplimed Corp.**

**Amplimexon** — Pancreatic cancer, melanoma, multiple myeloma, malignant melanoma, lung cancer, breast cancer, prostate cancer

**Arginox Pharmaceuticals Inc.**

**Tilarginine Acetate Injection** — Cardiogenic shock

**Ariad Pharmaceuticals Inc.**

**AP23573** — Solid tumors, blood malignancies, soft tissue and bone sarcomas, prostate cancer, endometrial cancer

**Ascenta Therapeutics Inc.**

**AT-101** — Cancer

**AstraZeneca Plc.**

**AGI-1067** — Atherosclerosis  
**Zactima** — Nonsmall cell lung cancer, thyroid cancer

**AtheroGenics Inc.**

**AGI-1067** — Atherosclerosis  
**AGI-1096** — Organ transplant rejection

**Avanir Pharmaceuticals Inc.**

**AVP-13358** — Allergies

**AVI BioPharma Inc.**

**Avicine** — Colorectal cancer, pancreatic cancer

**Basilea Pharmaceutica Ltd.**

**Ceftobiprole** — Complicated skin and skin-structure infections, nosocomial pneumonia

**Baxter Healthcare Corp.**

**ACAM2000** — Smallpox

**Berna Biotech AG**

**Aerugen** — Pseudomonas aeruginosa infection

**BioXone Therapeutic Inc.**

**Cethrin** — Spinal cord injury

**BioNumerik Pharmaceuticals Inc.**

**Tavocept** — Breast cancer

**Cadence Pharmaceuticals Inc.**

**CPI-226** — Catheter-related infection

**Cell Therapeutics Inc.**

**Pixantrone** — Relapsed indolent non-Hodgkin's lymphoma, relapsed aggressive non-Hodgkin's lymphoma, aggressive non-Hodgkin's lymphoma, acute myelogenous leukemia

**ChemoCentryx Inc.**

**Traficet-EN** — Inflammatory bowel disease

**Chrysalis Biotechnology Inc.**

**Chrysalin** — Bone fractures, diabetic ulcers, myocardial revascularization

**Cognetix Inc.**

**CGX-1160** — Chronic intractable pain

**CuraGen Corp.**

**Velafermin** — Mucositis, inflammatory bowel disease, oral mucositis

**CyRx Inc.**

**Arimoclamol** — Amyotrophic lateral sclerosis

**Daiichi Pharmaceutical Corp.**

**DX9065a** — Angina

**Daiichi Santory Pharma Co.**

**SUN N8075** — Acute cerebral infarction

**Debiopharma SA**

**Depelestat** — Cystic fibrosis

**DOR Biopharma Inc.**

**OrBec** — Graft vs. host disease

**Elan Pharmaceuticals Inc.**

**ACC-001** — Alzheimer's disease

**Enanta Pharmaceuticals Inc.**

**EDP-420** — Community-acquired pneumonia

**Epelus Biotechnologies Corp.**

**Rexin-G** — Pancreatic cancer

**ESP Pharma Inc.**

**Terlipressin** — Type 1 hepatorenal syndrome

**Evolutec Ltd.**

**REV131** — Allergic rhinitis

**Exelixis Inc.**

**XL880** — Solid tumors

**Expression Genetics Inc.**

**EGEN-001** — Ovarian cancer

**Favrille Inc.**

**Favld** — Non-Hodgkin's lymphoma

**FoldRx Pharmaceuticals Inc.**

**FX-1006a** — Genetic disorders

**Forest Laboratories Inc.**

**GRC 3886** — Chronic obstructive pulmonary disease, asthma

**Gamida-Cell Ltd.**

**StemEx** — Hematological malignancies

**Genmab A/S**

**HuMax EGFr** — Head and neck cancer

**Gilead Sciences Inc.**

**GS 9137** — HIV infection

**Immunomedics Inc.**

**Epratuzumab** — Non-Hodgkin's lymphoma, systemic lupus erythematosus, Sjogren syndrome

**Introgen Therapeutics Inc.**

**INGN 241** — Solid tumors, melanoma

**Isis Pharmaceuticals Inc.**

**ISIS 301012** — High cholesterol

**Ivax Corp.**

**Respicort** — Asthma

**King Pharmaceuticals Inc.**

**Remoxy** — Pain

**Koronis Pharmaceuticals Inc.**

**KP-1461** — HIV infection

**KS Biomedix Holdings Plc.**

**TransMID** — Glioma

**Medarex Inc.**

**Valortim** — Anthrax infection

**Medimmune Inc.**

**MT103** — Non-Hodgkin's lymphoma

**Micromet AG**

**MT103** — Non-Hodgkin's lymphoma

**Millenium Pharmaceuticals Inc.**

**MLN2704** — Prostate cancer

**Nabi Biopharmaceuticals Inc.**

**NicVAX** — Nicotine addiction

**NeoRx Corp.**

**Picoplatin** — Small cell lung cancer, colorectal cancer

**NicOx Inc.**

**HCT 3012** — Osteoarthritis

**Northfield Laboratories Inc.**

**PolyHeme** — Blood substitute

**Novo Nordisk Inc.**

**Liraglutide** — Type 2 diabetes

**Nuvelo Inc.**

**RNAPc2** — Acute coronary syndromes

**Omeros Medical Systems Inc.**

**OMS103HP** — Pain

**Orphan Therapeutics LLC.**

**Terlipressin** — Type 1 hepatorenal syndrome

**Orlho-McNeil Pharmaceutical**

**Ceftobiprole** — Complicated skin and skin-structure infections, nosocomial pneumonia

**Osirus Therapeutics Inc.**

**Prochymal** — Graft vs. host disease

**Pain Therapeutics Inc.**

**Remoxy** — Pain

**Palatin Technologies Inc.**

**PT-141** — Erectile dysfunction, female sexual dysfunction

**PharmaMar SA**

**Yondelis** — Ovarian cancer

**Pharmathene Inc.**

**Valortim** — Anthrax infection

**Pharmexa-Epimmune Inc.**

**GV 1001** — Pancreatic cancer

**Pfizer Inc.**

**Asenapine** — Acute mania, schizophrenia

**Predix Pharmaceuticals Inc.**

**PRX-03140** — Alzheimer's disease

**PSMA Development Co. LLC.**

**PSMA Vaccine** — Prostate cancer

**Rejuvenon Corp.**

**RC-1291** — Cachexia and anorexia

**Rigel Pharmaceuticals Inc.**

**R788** — Rheumatoid arthritis

**Samyang Corp.**

**Genexol-PM** — Cancer

**Schering-Plough Corp.**

**SCH 503034** — Hepatitis C

**Seattle Genetics Inc.**

**SGN-40** — Multiple myeloma, non-Hodgkin lymphoma, chronic lymphocytic leukemia

**Serono Inc.**

**SC12267** — Autoimmune diseases

**Sirna Therapeutics Inc.**

**Sirna-027** — Age-related macular degeneration

**Solvay Pharmaceuticals Inc.**

**Aerugen** — Pseudomonas aeruginosa infection

**Starpharma Ltd.**

**Vivagel** — HIV infection

**SuperGen Inc.**

**Avicine** — Colorectal cancer, pancreatic cancer

**TaiGen Biotechnology Co.**

**TG-873870** — Bacterial infections

**Taisho Pharmaceutical Co.**

**SUN N8075** — Acute cerebral infarction

**Takeda Pharmaceuticals**

**North America Inc.**  
**Tavocept** — Breast cancer

**Teva Pharmaceuticals USA**

**StemEx** — Hematological malignancies

**Theravance Inc.**

**Telavancin** — Hospital-acquired pneumonia, complicated skin and skin-structure infections

**Vertex Pharmaceuticals Inc.**

**Breacanvir** — HIV

**Vion Pharmaceuticals Inc.**

**Cloretazine** — Acute myelogenous leukemia, brain tumor, chronic lymphocytic leukemia, small cell lung cancer

**Virxsys Corp.**

**VRX496** — HIV infection

**Viropharma Inc.**

**Maribavir** — Cytomegalovirus infection

**Viventia Biotech Inc.**

**Proxinium** — Head and neck cancer

**Xanodyne Pharmaceuticals Inc.**

**XP12B** — Heavy menstrual bleeding

**Yaupon Therapeutics Inc.**

**Lobeline** — Methamphetamine addiction

**Ziopharm Oncology Inc.**

**ZIO-101** — Solid tumors, hematologic cancer, myeloma

**ZymoGenetics Inc.**

**IL-21** — Malignant melanoma, renal cell cancer