Top 50 orphan drug thought-leaders
The top 50 thought-leaders in orphan drugs and rare disease

The following report recognizes the top 50 thought-leaders in orphan drugs and rare disease. Thought-leaders include representatives from industry, patient advocacy groups, research institutions, government and media.

In addition to their biographies, I’ve included a brief paragraph highlighting the main reasons why they have been included in this report.

Please note, thought leaders will appear in alphabetical order and have been selected based on the following criteria:

- Research & methodology
- Leadership
- Awards & recognition
- Community engagement & impact

Think we missed an important thought-leader? Let us know, we’d love to hear from you

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Chris Austin, Director, National Center for Advancing Translational Sciences, NIH

In September 2012, Christopher P. Austin, M.D., was appointed the first permanent director of the National Center for Advancing Translational Sciences (NCATS).

Austin is leading NCATS in its mission to catalyze the generation of innovative methods and technologies that will enhance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions.

Currently, many costly, time-consuming bottlenecks exist in the translational process. Austin is applying his experience across the spectrum of the research pipeline to develop, demonstrate, and disseminate innovative technologies and paradigms that increase the efficiency of translation, and thus speed the discovery and delivery of interventions that improve human health.

Austin came to NIH in 2002 from Merck, where his work focused on genome-based discovery of novel targets and drugs. He began his NIH career as the senior advisor to the director for translational research at the National Human Genome Research Institute, where he initiated the Knockout Mouse Project and the Molecular Libraries Roadmap Initiative. Other NIH roles have included serving as director of the Therapeutics for Rare and Neglected Diseases program as well as the NIH Chemical Genomics Center and as scientific director of the NIH Center for Translational Therapeutics.

Austin earned an A.B. summa cum laude in biology from Princeton University and an M.D. from Harvard Medical School. He completed clinical training in internal medicine and neurology at Massachusetts General Hospital, and a research fellowship in genetics at Harvard.

Why we chose Dr. Austin: As the first Director of NIH/NCATS, and having previously served as the Director for the agency’s TRND program which bridges the gap between basic research discovery and testing of new drugs in humans, Dr. Austin has led multiple initiatives to expedite orphan drugs to patients. The division has recently announced its first Federal Register notice concerning a novel selective series of non-inhibitory chaperones of glucocerebrosidase (GCase) for the treatment of Gaucher Disease.
Ségolène Aymé, Chair, European Union Committee of Experts on Rare Diseases

Ségolène Aymé is a medical geneticist, and Director of Research at the French Institute of Health and Medical Research (INSERM). She is also the executive manager of the INSERM department dedicated to information on rare diseases in Paris.

This service established Orphanet (www.orpha.net) in 1997, the European database of rare diseases and orphan drugs which is currently funded by the French Ministry of Health, the INSERM and the European commission through a Joint Action of the EU Member States. She chairs the newly formed European Union Committee of Experts on Rare Diseases. She is also the editor-in-chief of the Orphanet Journal of Rare Diseases (www.ojrd.com) and the chair of the WHO Topical Advisory Group for Rare Diseases.

Why we chose Dr. Aymé:
Dr. Aymé is recognized worldwide for her efforts in establishing Orphanet, a web portal with an invaluable rare disease database. This database was the first comprehensive registry for rare disorders, and has helped to shape most current patient registries. And as if that wasn’t enough, Dr. Aymé recently was appointed to chair EUCERD, which along with EURORDIS is the most prominent rare disease organization in Europe.
Hal Barron, CMO and Head, Global Product Development, Roche

Hal V. Barron, M.D., F.A.C.C. has served as a member of Roche’s Board since December 2007. In April 2009, Dr. Barron became Executive Vice President of Genentech, Inc. and Chief Medical Officer of F. Hoffman-La Roche, Ltd., a pharmaceutical company, following La Roche, Ltd.'s acquisition of Genentech, Inc. Previously, starting in December 2003, Dr. Barron served as Senior Vice President of Development at Genentech, Inc. and as Chief Medical Officer since March 2004. Dr. Barron joined Genentech, Inc. in 1996 as a clinical scientist and in 2002 he was promoted to Vice President of Medical Affairs. Dr. Barron's academic positions include Associate Adjunct Professor of Epidemiology and Biostatistics and Associate Clinical Professor of Medicine/Cardiology at the University of California, San Francisco. Dr. Barron's experience as chief medical officer, vice president and clinical scientist of a large, publicly-traded pharmaceutical company gives him relevant industry experience, and his tenure with Genentech, Inc. in a variety of positions has provided him with the knowledge of the multiple stages of development of pharmaceutical companies and the challenges Roche will face at each stage.

Dr. Barron received his B.S. in physics from Washington University in St. Louis, his M.D. from Yale University and completed his training in medicine and cardiology at the University of California, San Francisco.

Why we chose Dr. Barron:
Roche is expected to maintain 2nd position in worldwide orphan drug sales in 2018. Dr. Barron’s leadership within Roche-Genentech has allowed the company to avoid relying exclusively on its sales champion, Rituximab, and has enabled it to advance clinical trials for other orphan drug candidates, like RoACTEMRA and Octreolin, which were acquired by Roche from the Israeli company Chiasma.
Robert Beall, President and Chief Executive Officer, Cystic Fibrosis Foundation

Robert J. Beall, has been with the Cystic Fibrosis (CF) Foundation for more than 30 years and currently serves as president and CEO. Beall began his tenure as executive vice president for medical affairs, and for the past 18 years, he has served as president and CEO.

Prior to joining the CF Foundation, Beall was on the medical school faculty of Case Western Reserve University in Cleveland, Ohio, and at NIH, where he managed a large portion of the agency’s cystic fibrosis program.

Beall received his doctorate in biochemistry from the State University of New York at Buffalo.

Why we chose Dr. Beall:
Under Dr. Beall’s leadership, the CF Foundation has become one of the most respected voluntary health organizations in the country, and is recognized for its innovative approaches to bringing new therapies to patients with the disease. As a result of the foundation’s pioneer business model, there are currently nearly 30 potential CF therapeutic products in the development pipeline.
Camille Bedrosian, Senior Vice President and Chief Medical Officer, Alexion

Dr. Camille L. Bedrosian joined Alexion as Chief Medical Officer in May 2008. A medical oncologist and hematologist, she brings more than 25 years of medical practice, research and drug development expertise to this role. At Alexion, she provides leadership for the development of compounds across therapeutic franchises, including expansion of both the PNH franchise as well as additional therapeutic indications for Alexion's marketed product, eculizumab. In addition, she is leading the effort to build the oncology platform initially with the first-in-class anti-CD200 monoclonal antibody. Dr. Bedrosian has global responsibility for aspects of clinical research, such as clinical operations, clinical data management, medical affairs and pharmacovigilance. In her role, she also interacts extensively with global commercial and business development teams.

Why we chose Dr. Bedrosian:
Dr. Bedrosian is the co-author of the successful clinical trials of Soliris, a life-changing treatment for atypical hemolytic uremic syndrome (aHUS), a genetic, life-long, ultra-rare disease. In previous positions she has been recognized for her leadership and implementation of the strategic development for other companies’ lead product candidates, like deforolimus, thru the drug’s clinical development, human trials and its orphan drug designation in both the United States and European Union, to a new dosage form, and to an SPA-approved Phase 3 trial for patients with Sarcoma.
Marc Beer, Chief Executive Officer, Aegerion Pharmaceuticals

Marc Beer is the Chief Executive Officer of Aegerion Pharmaceuticals, Inc. (NASDAQ: AEGR). Aegerion is a biopharmaceutical company dedicated to the development and commercialization of innovative, life-altering therapies for patients with debilitating often fatal, rare diseases. Marc took the company public in October 2010. The Company’s first marketed product, JUXTAPID™ (lomitapide) was approved by the U.S. Food and Drug Administration in December 2012. The company also has a Marketing Authorization Application for lomitapide under review in the EU. Marc brings over 20 years of development and commercialization experience in biotechnology, pharmaceuticals and diagnostics.

In April 2000, Marc became the founding CEO of ViaCell (VIAC), a biotechnology company specializing in the collection, preservation, and development of umbilical cord blood stem cells, and over a period of seven years led the company from its inception to a rapidly growing and robust commercial organization. Under Marc's leadership, the company grew to more than 300 employees, went public in 2005 and was ultimately acquired in 2007 by PerkinElmer (PKI). Prior to founding ViaCell, Marc held various positions within Genzyme (GENZ), most recently as Vice President of Global Marketing where he was responsible for the commercial launch of several products addressing orphan disease populations on a global scale. Prior to Genzyme, he held a variety of sales and marketing roles in the pharmaceutical and diagnostic divisions of Abbott Laboratories (ABT). He also was a former member of the Mass Life Science Board of the Commonwealth of Massachusetts. Marc serves on the Biotechnology Industry Organization (BIO) Emerging Companies Section Governing Board as well as on the board of directors of Good Start Genetics, Inc. where he is Chairman; ERYtech Pharma; Strategic Consultant to OvaScience; and a member of the Graduate Studies and Research Advisory Council of Notre Dame University.

Marc holds a B.S. from Miami University (Ohio).

Why we chose Marc:
Marc has led the company thru its first orphan drug launch, Juxtapid for HoFH, an ultra-orphan disease. Marc is at the helm of Aegerion’s move towards becoming one of the most promising orphan drug biotechs in the industry.
Jean-Jacques Bienaimé, Chief Executive Officer, Biomarin

Mr. Bienaimé joined BioMarin in May 2005 as Chief Executive Officer and member of the Board of Directors, bringing with him over 25 years of experience in the biotechnology and pharmaceutical industries. Under his leadership, the market capitalization of BioMarin went from around $450 million in May 2005 to over $3.5 billion in May 2008. By 2008, under Mr. Bienaimé’s leadership BioMarin was a fully integrated biopharmaceutical company with nearly 700 employees and $300 million in revenue.

He received an M.B.A from the Wharton School at the University of Pennsylvania and an undergraduate degree in economics from the Ecole Superieure de Commerce de Paris.

Why we chose Jean-Jacques:
JJ is well known in the orphan drug industry for leading the first company to successfully launch 3 three orphan drugs. To date there are very few companies with three orphan drugs on the market. There are currently companies with more than 3 orphan drugs for sale. He is actively looking to acquire smaller biotechs with drugs in development in order to maintain Biomarin’s position.
Sherrod Campbell Brown (born November 9, 1952) is the senior United States Senator from Ohio and a member of the Democratic Party. Before his election to the Senate, he was a member of the United States House of Representatives, representing Ohio's 13th congressional district from 1993 to 2007. He previously served as the Ohio Secretary of State (1983–1991) and a member of the Ohio House of Representatives (1974–1982).

An Eagle Scout, Brown is a native of Mansfield, Ohio, where he spent summers working on his family’s farm. He is married to Pulitzer Prize-winning columnist Connie Schultz. They reside in Avon, Ohio, and have three daughters, a son, both a daughter and son-in-law, and two grandsons.

Why we chose Senator Brown:
Senator Brown is well known for advocating in favour of advancing rare disease research and improving orphan drug development regulation. He recently introduced the National Pediatric Research Network Act (HR. 6163) which will ensure that the National Institutes of Health (NIH) increase their attention to pediatric medical research. This effort was applauded by the National Organization for Rare Disorders (NORD) for increasing the focus on devastating rare pediatric diseases.
Robert Casey Jr., US Senator, Pennsylvania

U.S. Senator Bob Casey knows that public service is a privilege and that he was elected to fight for Pennsylvania priorities and Pennsylvania values. He is working to foster financial security for American families, protect our children and invest in their futures and ensure safety at home and respect abroad.

Senator Casey is a nationally recognized advocate for children. He has introduced two landmark bills to increase access to early childhood education and reverse the chronic underfunding of child care. He was a strong supporter of the law that expanded the Children’s Health Insurance Program which was signed into law in February 2009 to provide health care coverage to more than 14 million American children. During debate on the new health care law, Senator Casey helped to include a number of provisions to protect and improve health care for children. Senator Casey has been recognized with numerous awards for his work benefiting children including the Champion for Children Award from First Focus, Children’s Champion Award from the Children’s Hospital of Philadelphia and the Delaware Valley Association for the Education of Young Children (DVAEYC) Champion for Young Children Award.

Casey is the eldest son of the late Governor Robert P. Casey and his wife, Ellen. Throughout his public career, Bob Casey has been guided by the legacy of his father, and the principle that: "All public service is a trust, given in faith and accepted in honor."

Why we chose Senator Casey:
Bob Casey Jr. (D-PA) is one of the US Senators who introduced the *Preserving Access to Orphan Drugs Act of 2011*, a bipartisan legislation to ensure that the development of treatments for rare diseases is not jeopardized. This legislation ensures that unnecessary regulatory hurdles are not placed in front of innovative new therapies for rare diseases and conditions.
Tony Coles, Chairman and Chief Executive Officer, Onyx Pharmaceuticals

Dr. Coles joined Onyx in March 2008 as President and Chief Executive Officer. In 2012, Dr. Coles was appointed as Chairman of the board of directors. Prior to joining Onyx, he held several senior level positions at: NPS Pharmaceuticals, Vertex Pharmaceuticals Inc., Bristol-Myers Squibb and Merck & Co.

Dr. Coles completed his cardiology and internal medicine training at Massachusetts General Hospital and was a research fellow at Harvard Medical School. He earned an M.D. degree from Duke University, a master’s degree in public health from Harvard University, and an undergraduate degree from Johns Hopkins University. Dr. Coles currently serves as a trustee and member of the Executive Committee for the Johns Hopkins University Board of Trustees, as well as a member of the board of trustees for Johns Hopkins Medicine. Dr. Coles is also a member of the board of the Biotechnology Industry Organization (BIO).

Why we chose Dr. Coles:
Two of the expected top 30 selling orphan drugs by 2018 are being developed by Onyx. Since Dr. Coles' leadership, Onyx is now considered a model biotech, demonstrating how a savvy R&D strategy coupled with a strong commercial strategy can command respect from the market and make a company stand out from the crowd.
**Francis Collins, Director, NIH**

Francis S. Collins, M.D., Ph.D. is the Director of the National Institutes of Health (NIH). In that role he oversees the work of the largest supporter of biomedical research in the world, spanning the spectrum from basic to clinical research.

Dr. Collins is a physician-geneticist noted for his landmark discoveries of disease genes and his leadership of the international Human Genome Project, which culminated in April 2003 with the completion of a finished sequence of the human DNA instruction book. He served as director of the National Human Genome Research Institute at the NIH from 1993-2008.

Before coming to the NIH, Dr. Collins was a Howard Hughes Medical Institute investigator at the University of Michigan. He is an elected member of the Institute of Medicine and the National Academy of Sciences, was awarded the Presidential Medal of Freedom in November 2007, and received the National Medal of Science in 2009.

**Why we chose Dr. Collins:**

Dr. Collins led the successful effort to complete the Human Genome Project, a complex multidisciplinary scientific enterprise to map and sequence human DNA. This will undoubtedly help in the investigation and diagnosis of rare diseases.
John Crowley, Chairman and Chief Executive Officer, Amicus Therapeutics

John F. Crowley is the Chairman, President and CEO of Amicus Therapeutics, Inc., a publicly traded biotechnology company focused on the development of novel treatments for human genetic diseases.

John and his family have been profiled on the front page of The Wall Street Journal and are the subjects of a book by Pulitzer prize-winning journalist Geeta Anand, "The Cure: How a Father Raised $100 Million-And Bucked the Medical Establishment-In a Quest to Save His Children." The major motion picture, Extraordinary Measures, starring Brendan Fraser and Harrison Ford, is inspired by the Crowley family journey. John is also the author of a personal memoir, ‘Chasing Miracles: The Crowley Family Journey of Strength, Hope and Joy.’ John is also a commissioned officer in the U.S. Navy Reserve, assigned to the United States Special Operations Command and is a veteran of the global war on terrorism, with service in Afghanistan.

He graduated with a B.S. in Foreign Service from Georgetown University, and earned a J.D. from the University of Notre Dame Law School and an M.B.A. from Harvard. The Crowley family was the recipient of the 2011 Family Exemplar Award from the University of Notre Dame. John is a Henry Crown Fellow at the Aspen Institute. He lives in Princeton, NJ with his wife, Aileen and their three children, John, Megan and Patrick. John serves on the executive committee of the National Board of Directors of the Make A Wish Foundation of America.

Why we chose John:
John is famous for founding biotech companies devoted to curing genetic diseases. For this reason, he is often referred to as the ‘father of biotechs.’ He is best known for founding, Novozyme, which has since merged with Genzyme. Nowadays he continues to develop drugs for rare disease at Amicus another biotech he founded.
Colin Farrell, Actor

Colin Farrell is one of Ireland's best rising stars in Hollywood and abroad today. His film presence has been filled with memorable roles that range from an inwardly tortured hit man, to an adventurous explorer, a determined-but-failing writer, and the greatest military leader in history.

Farrell was born on May 31, 1976 in Castleknock, Dublin, Ireland. His father and uncle were both professional athletes, and for a while, it looked like Farrell would follow in their footsteps. Farrell auditioned for a spot in the Irish Boy Band, Boyzone, but it didn't work out. After dropping out of The Gaiety School of Acting, Farrell was cast in "Ballykissangel" (1996), a BBC television drama. "Ballykissangel" was not his first role on screen. Farrell had previously been in The War Zone (1999), directed by Tim Roth and had appeared in the independent film Drinking Crude (1997). Farrell was soon to move on to bigger things.

Why we chose Colin:
Colin Farrell has been actively advocating for rare disease research since his son, James was diagnosed with Angelman Syndrome, a very rare genetic neurological disorder. His prominence as a globally-recognized Hollywood actor has helped patient organizations like CureAngelman increase awareness about the disease and expedite research for a cure.
Anne Marie Finley, VP, Government Relations & Public Policy, Celgene

Anne Marie Finley joined Celgene in November, 2007 as Vice President, Government Relations. She is based in Washington, DC. Anne Marie joined the company from Biotech Policy Group LLC, a government relations and regulatory policy consulting firm she founded in 2001. Previously, she held the positions at GE Healthcare (formerly Amersham Health); Ketchum Public Relations; U.S. House of Representatives Committee on Government Reform and Oversight and Hershey Foods Corporation. She served in the administration of President George H. W. Bush as Special Assistant for Legislative and Public Affairs in the Office of the Commissioner, Food and Drug Administration.

Apart from her job at Celgene, Anne Marie also serves on the US Department of Health and Human Services Advisory Committee on Blood Safety and Availability and is a trustee of the Hemophilia Association of New Jersey. Anne Marie is a certified Regulatory Affairs Specialist (RAC) and the author of numerous reports and articles on infectious agents, blood safety, food safety, and food additives.

Anne Marie graduated from Georgetown University with a BA in Government and received an MS in Science and Technology Commercialization from the University of Texas at Austin. She also earned a graduate certificate in government relations from George Washington University.

Why we chose Anne Marie:
Anne Marie is actively involved in key discussions dedicated to designing a regulatory framework for orphan drug development. An expert with access to different national regulators, Anne Marie has a unique holistic perspective on the challenges surrounding orphan drug development across multiple markets.
**Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy**

Pat Furlong is the Founding President and CEO of Parent Project Muscular Dystrophy (PPMD), the largest nonprofit organization in the United States solely focused on Duchenne muscular dystrophy (Duchenne). Its mission is to improve the treatment, quality of life, and long-term outlook for all individuals affected by Duchenne through research, advocacy, education, and compassion.

When doctors diagnosed her two sons with Duchenne in 1984, Pat immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment, and the mechanisms for optimal care. In 1994, Pat, together with other parents of young men with Duchenne, founded PPMD to change the course of Duchenne and, ultimately, to find a cure.

Today, Pat continues to lead the organization and is considered one of the foremost authorities on Duchenne in the world. In 2010, Pat was named WebMD’s Health Hero and was featured in The New Yorker as a “World Changer.” She is the recipient of Research!America’s 2008 Gordon and Llura Gund Leadership Award.

**Why we chose Pat:**
Pat is probably the most renowned Duchenne Muscular Dystrophy advocate globally. Her brave and successful efforts in founding and developing the Parent Project Muscular Dystrophy Organization has exponentially increased studies and new therapies for the disease.
Chris Garabedian, Chief Executive Officer, Sarepta Therapeutics

Chris joined AVI, now Sarepta Therapeutics, as President and Chief Executive Officer on January 1, 2011. He has served as a director of the Company since June 2010. Previously he was Vice President of Corporate Strategy for Celgene Corporation from July 2007. From November 2005 to June 2007, Chris served as an independent consultant to early stage biopharmaceutical companies. From 1997 to 1998 and from 1999 to November 2005, he worked at Gilead Sciences, Inc., where he served in a number of global leadership roles, including as Vice President of Corporate Development, Vice President of Marketing, and Vice President of Medical Affairs. Chris also held various commercial roles at COR Therapeutics, Inc. from 1998 to 1999 and at Abbott Laboratories from 1994 to 1997. He started his biopharmaceutical career as a consultant with Migliara/Kaplan Associates from 1991 to 1994. Chris received his BS in marketing from the University of Maryland.

Why we chose Chris:
Chris brings his wide experience in the pharmaceutical and biotechnology sector to lead Sarepta Therapeutics in becoming one of the top RNA-based therapeutics companies globally. His wide experience with portfolio and strategic management, marketing and commercialization were essential to Sarepta in building a strong pipeline for life-threatening rare and infectious diseases, and in accelerating the development of their lead orphan drug candidate for Duchenne Muscular Dystrophy, Eteplirsen, which should be under the NDA submission process by the first half of 2014.
Jordi Llinares Garcia, Head of Orphan Medicines, European Medicines Agency

Dr. Jordi Llinares is the head of orphan medicines at the European Medicines Agency (EMA). Dr. Llinares joined the EMA in 2002. Prior to joining the EMA, Dr Llinares worked as clinical pharmacologist at Hospital de Sant Pau (Barcelona) and collaborated with the Spanish Drugs Agency as clinical assessor. Dr Llinares was a member of the Ethics Committee of the Institut d’Assistencia Sanitaria (Salt).

Dr. Llinares graduated as an MD from the University of Barcelona and specialised (MIR) as clinical pharmacologist at Hospital Vall d’Hebron (Barcelona). Dr Llinares holds a Masters in Science in Epidemiology from the London School of Hygiene and Tropical Medicine (London).

Why we chose Dr. Llinares:
Dr. Llinares heads the group responsible for assessing applications for orphan designation in Europe. He also coordinates the activities necessary for the adoption of orphan designation, including the drafting of summary reports. Dr. Llinares supports the secretariat of the Committee for Orphan Medicinal Products (COMP).
Jim Gerlach, US House of Representatives, Pennsylvania

A lifelong Pennsylvania resident, Jim Gerlach grew up in Ellwood City – a town about an hour north of Pittsburgh. He earned a Bachelor of Arts degree from Dickinson College and a Juris Doctor from the Dickinson School of Law in Carlisle, Pennsylvania. He currently lives in Chester Springs, Chester County with his wife, Karen, and they have three children and three step-children.

Jim has served the citizens of Southeastern Pennsylvania for more than 20 years. His distinguished career began in 1990 with the first of two terms in the Pennsylvania House of Representatives, which was followed by two terms in the Pennsylvania Senate.

Jim is serving his sixth term in the U.S. House of Representatives, representing portions of Berks, Chester, Lebanon and Montgomery counties. In December 2010, he earned a spot on the influential House Ways and Means Committee. Jim serves on the Ways and Means Subcommittees on Health and Select Revenue. One of his biggest legislative accomplishments was creating a much-needed veterans cemetery in Southeastern Pennsylvania. The bill Jim authored was signed into law by the President on Veterans Day in 2003, and the cemetery opened in Bucks County in 2009.

Why we chose Representative Gerlach:
Representative Jim Gerlach (R-PA) introduced the Preserving Access to Orphan Drugs Acts with lead co-sponsor Rep. Richard Neal (D-MA). He has been applauded by multiple patient and industry organizations for demonstrating exceptional leadership and commitment to the rare disease community with this legislation.
Kathy Giusti, Founder and CEO, Multiple Myeloma Research Foundation

Kathy Giusti is the Founder and Chief Executive Officer of the Multiple Myeloma Research Foundation (MMRF) and the Multiple Myeloma Research Consortium (MMRC). Prior to founding the MMRF, Giusti held senior level positions in pharma at G.D. Searle & Co. and Merck & Co., and also worked at the Gillette Company.

Giusti, a multiple myeloma patient, was most recently named to the 2011 TIME 100 List of the world’s most influential people and has also received a number of other awards for her leadership, including the American Association for Cancer Research (AACR) Centennial Medal for Distinguished Public Service, the Harvard Business School Alumni Achievement Award, and the Healthcare Businesswomen's Association's Woman of the Year Award.

Giusti currently serves on the Executive Management Committee to Stand Up to Cancer and the Harvard Business School Advisory Board. She has previously served on several other boards, including the President’s National Cancer Advisory Board, the Board of Directors for IMS Health, and the Institute of Medicine's National Cancer Policy Board.

Giusti received her MBA in general management from Harvard Business School and graduated from the University of Vermont magna cum laude with a Bachelor of Science in Biological Sciences.

Why we chose Kathy:
Kathy has been recognized and awarded by multiple organizations for the progress she has made in accelerating the way research and drug development are conducted, ultimately improving the outlook for patients with multiple myeloma and other cancers.
**Stephen Groft**, Director, Office of Rare Disease Research, NIH

Steve is the Director of the Office of Rare Diseases (ORD) at the National Institutes of Health (NIH). His major focus is on stimulating research with rare diseases and developing information about rare diseases and conditions.

Current activities include developing genetic tests for rare diseases, developing an educational module on rare diseases for school children, establishing a public information center on genetic and rare diseases, maintaining the Rare Diseases Clinical Research Network, and providing a special emphasis clinic with senior clinical staff for patients with undiagnosed diseases at NIH’s Clinical Research Center Hospital.

Steve received the B.S. degree in Pharmacy in 1968 and the Doctor of Pharmacy degree from Duquesne in 1979.

**Why we chose Dr. Groft:**
Dr. Groft is well known across the global rare disease research community for his impressive experience and leadership as the Director of the NIH’s Office of Rare Disease Research. As the division’s Head, Dr. Groft identifies new research opportunities, establishes research priorities and co-sponsors over 1,000 rare diseases-related scientific conferences.
Margaret Hamburg, Commissioner, U.S. Food and Drug Administration

Margaret A. Hamburg became the 21st commissioner of food and drugs on May 18, 2009. The second woman to be nominated for this position, she is an experienced medical doctor, scientist, and public health executive.

As the top official of the Food and Drug Administration (FDA), Dr. Hamburg is committed to strengthening programs and policies that enable the agency to carry out its mission to protect and promote the public health. "Strengthening FDA’s programs and policies will help us protect the safety of the food supply, give the public access to safe and effective medical products, find novel ways to prevent illness and promote health, and be transparent in explaining our decision-making," says Dr. Hamburg. "A strong FDA is an agency that the American public can count on."

Why we chose Dr. Hamburg:
As head of the world’s most powerful agency within the pharmaceutical industry, Dr. Hamburg has been pushing industry, government, patient groups and other orphan drug stakeholders to work on resolving current obstacles that have been impeding a faster delivery of new therapies for rare disease patients. She has been overseeing the implementation of FDASIA and the new breakthrough therapy designation to address those challenges and by promoting the enhanced use of science and technology in the FDA, she has increased the agency’s predictability, benefiting rare disease research further.
Tateo Ito, Chief Executive Officer, Japan Patient Association

Afflicted by Myasthenia Gravis, Tateo Ito went on to establish the Hokkaido Conference for People with Intractable Diseases in 1973 and remained its executive director until 1982, when he established the Hokkaido Intractable Disease Center, where he served as its director until 2006.

Tateo established the Japan Patient and Family Council Society in 1986 and served as its chairman until 2005. In 2003 he established the National Intractable Disease Center Research Group,. In 2005 he established the Japan Patient Association (JPA).

In 2007 he served as a representative for the Intractable Diseases Support Network in Hokkaido. In 2008 he served as a representative for the National Myasthenia Fellowship.

He has also served as the chairman for several nonprofit organizations, offering job assistance for people with disabilities, and as a member of the Council of Health Sciences Committee and a member of the Investigative Commission for Institutional Framework in Regenerative Medicine.

For the past 12 years he has been independently administering care to his wife who suffers from a juvenile form of Alzheimer’s disease. Tateo is a part-time lecturer at the Hokkaido Prefectural School of Hygiene.

Why we chose Tateo:

Tateo heads Japan’s biggest rare disease organization, and is responsible for several achievements in expanding Japan’s access to orphan drugs and in accelerating rare disease research through collaborations. He oversaw the recent partnerships with NORD and EURORDIS, establishing a global collaboration to drive progress toward new treatments, improved public policies and better lives for people with rare diseases.
Andrew Jablonski, Founder and CEO, Short Bowel Syndrome Foundation

Born in 1986, in Columbia, Missouri, Jablonski himself has lived with Short Bowel Syndrome his entire life. At birth, it was discovered that he had only four inches of living small intestine, putting him at extreme risk for chronic medical complications. Jablonski defied the odds and his case is still used internationally by gastroenterologists who continue to make advances by comparing diagnostic studies in similar cases.

Jablonski is certified as a nursing assistant, and has had extensive training in emergency medicine and surgical technology. He recently graduated from Southeast Community College with an Associates of Applied Science Degree in Business Administration, with a focus in Entrepreneurship.

Today at age twenty-five, Jablonski resides in Lincoln, Nebraska and manages his condition with oral medication and follow up care with long trusted professionals who are committed to his success. This personal commitment has developed into a partnership expressed through participation on the SBS Foundation's, Board of Directors.

Why we chose Andrew:
Andrew is an amazing inspiration for the entire rare disease community. He’s been extremely successful in supervising the day-to-day operations for the Short Bowel Syndrome Foundation and advocating for accelerated research for the disease.
Emil Kakkis, President and Founder, EveryLife Foundation for Rare Diseases

Dr. Kakkis is best known for his work over the last 18 years to develop novel treatments for neglected rare disorders.

He began his work in a research bungalow at Harbor-UCLA working with minimal funding and support to develop an enzyme replacement therapy (Aldurazyme®) for the rare disorder MPS I. The struggle to get the therapy translated from a successful canine model to patients succeeded due to the critical financial support of a new patient organization formed by Mark and Jeanne Dant for their son Ryan, called the Ryan Foundation. Aldurazyme development was later supported by BioMarin™ and eventually their partner Genzyme™ leading to FDA approval in 2003.

Dr. Kakkis graduated from Pomona College, magna cum laude, and received a combined MD and PhD degree from the UCLA Medical Scientist Program in 1989. He is board certified in both Pediatrics and Medical Genetics. He joined BioMarin in 1998 and held various positions including Chief Medical Officer from 2006 to 2009.

He received the Lifetime Achievement Award from the National MPS Society for his work on Aldurazyme. He has authored numerous scientific articles on MPS I, immune tolerance during enzyme therapy, intrathecal enzyme therapy and studies on treatments for MPS VI and PKU.

Why we chose Dr. Kakkis:
Dr. Kakkis has working to develop and advance rare disease treatments for over 18 years. He has guided the development and approval of multiple treatments for rare disorders, including drugs for MPS VI and PKU. Since Dr. Kakkis left his position as Chief Medical Officer of BioMarin, he’s been pursuing changes in the drug development and regulatory system, both through the EveryLife Foundation for Rare Diseases and Ultragenyx, a rare disease biotech with an impressive orphan drug pipeline. His focus has been on improving the diagnosis and treatment of rare disorders; and specifically the process by which treatments for rare disorders are tested and approved.
Yann Le Cam, Chief Executive Officer, EURORDIS

Mr Le Cam has 25 years of professional experience, and personal commitment, in health and medical research non-governmental organizations in France, Europe and the United States in the fields of cancer, hiv/aids, genetic disorders and rare diseases.

He is the CEO of EURORDIS, an International Non Governmental Organization gathering over 500 member patient advocacy and support associations in 45 countries, therefore representing 30 million people living with rare diseases in Europe. From 2000-2006, he served as Vice-Chair of the European Medicines Agency’s Committee on Orphan Medicinal Products and is one of the founding members of the International Alliance of Patients Organizations (IAPO). Yann has also served on the Management Board and Executive Committee of the French HTA agency ANAES now called HAS for 5 years, on the DIA Advisory Committee Europe for 3 years, on the EPPOSI Board for 6 years.

Why we chose Yann:
As the head and one of the founders of Europe’s largest rare disease focused patient organization, EURORDIS, as well as the co-founder of the European alliance of rare disease patient organizations, Yann is at the center of the decisions that support the advances in rare disease research in Europe. He is an expert in the European orphan drug marketplace and his engagement with multiple committees and organizations makes him one of the executives with the most holistic knowledge of the rare disease and orphan drug community.
Dave Lemus, Chief Executive Officer, Sigma-Tau Pharmaceuticals

Dave Lemus was recently promoted to the newly created position of Chief Executive Officer of Sigma-Tau Pharmaceuticals. Mr. Lemus served as Chief Operating Officer since March 2012, and joined the company as Vice President of Finance in July 2011.

Prior to joining the company, Mr. Lemus was Executive Vice President and Chief Financial Officer of MorphoSys AG, where he launched Germany's first biotech IPO in 1999. Before MorphoSys, Mr. Lemus held a variety of senior management positions at Hoffmann-La Roche and Lindt Chocolate, in Switzerland.

Mr. Lemus presently serves as board chairman of Proteros Gmbh in Munich, and as a non-executive board member of Axela Inc. in Toronto.

Why we chose Dave:
Dave has led Sigma-Tau to the breakthrough development of Cystaran, the first and only approved therapy for the treatment of corneal cystine crystal accumulation in patients with cystinosis. This is a multi-stakeholder collaboration with the National Institutes of Health (NIH), in cooperation with the Cystinosis Foundation, the Cystinosis Research Foundation, and the Cystinosis Research Network.
Freda Lewis Hall, Chief Medical Officer, Pfizer

Freda Lewis-Hall is the Chief Medical Officer of Pfizer Inc. Trained as a psychiatrist, she has held leadership roles in academia, medical research, front-line patient care, and at global biopharmaceutical companies including Vertex, Bristol-Myers Squibb and Eli Lilly.

Prior to her work in industry, she led research projects for the National Institutes of Health and was vice chairperson of the Department of Psychiatry at Howard University College of Medicine.

In 2010, Dr. Lewis-Hall was appointed by the Obama Administration to the inaugural Board of Governors for the Patient-Centered Outcomes Research Institute (PCORI), and in 2012 she was appointed chair of the Cures Acceleration Network Review Board and a member of the National Center for Advancing Translational Sciences (NCATS) Advisory Council of the National Institutes of Health.

She also serves on the Executive Committee of the Clinical Trials Transformation Initiative and on numerous other boards, including those of Harvard Medical School, The Institute of Medicine’s Forum on Drug Discovery, Development, and Translation, and Save the Children.

Dr. Lewis-Hall has been named as one of Savoy’s Top Influential Women in Corporate America in 2012 and was selected as the Healthcare Businesswomen’s Association 2011 “Woman of the Year.”

Why we chose Dr. Lewis-Hall:
Dr. Lewis-Hall leads the Pfizer Medical Division responsible for providing doctors and patients with the medical information they need to make informed choices. With a strong emphasis on leveraging the next generation of orphan drugs, her group makes sure Pfizer is a leader in the safe, effective and appropriate use of medications, from the first clinical trials in humans onward.
Jimmy Lin, Founder and President, Rare Genomics Institute

Jimmy Cheng-Ho Lin, MD, PhD, MHS, is a 2012 TED Fellow and Founder/CEO of Rare Genomics Institute, the world's first platform to enable any community to leverage cutting-edge biotechnology to advance understanding of any rare disease. Partnering with 18 of the top medical institutions, such as Harvard, Yale, Johns Hopkins, and UCSF, RGI helps custom design personalized research projects for diseases so rare that no organization exists to help.

Dr. Lin is also a medical school faculty member at the Washington University in St. Louis and led the computational analysis of the first ever exome sequencing studies for any human disease at Johns Hopkins.


Why we chose Dr. Lin:
Under Dr. Lin’s leadership, the Rare Genomics Institute has been empowering rare disease patients by connecting them with researchers, doctors and community support through a crowd-sourced funding platform. This effort has offered patients and researchers the necessary resources to democratize research for rare diseases.
Geoffrey McDonough, President & CEO, Swedish Orphan Biovitrum AB


Dr McDonough is Chief Executive Officer of Swedish Orphan Biovitrum AB (Sobi). Prior to Sobi Dr McDonough held various senior positions within Genzyme Corporation since 2002, latest as President of Europe, Middle East and Africa. Before Genzyme he worked as Paediatrician and Internist at Massachusetts General Hospital in Boston and was the President and Co-founder of Catalyst Medical Solutions Inc.

Why we chose Dr. McDonough:
Dr. McDonough has impressive experience of the development and commercialization of orphan drugs, having led Catalyst Medical Solutions, Genzyme and SOBI. He is renowned not only for his broad experience in genetic disorders but also for his passion in identifying solutions for patients with unmet medical needs.
David Meeker, President and Chief Executive Officer, Genzyme

David Meeker was appointed CEO of Genzyme in October 2011. In his career with Genzyme, he has held key positions of increasing responsibility, most recently as Chief Operating Officer. In this role, he was responsible for Genzyme’s commercial organization, overseeing its business units, country management organization, and global market access functions. As Chief Operating Officer, he played an important role in the integration with Sanofi. Dr. Meeker joined Genzyme in 1994 as Medical Director to work on the Cystic Fibrosis Gene Therapy program. Subsequently, as Vice President, Medical Affairs, he was responsible for the development of therapeutic products, including treatments in the current rare disease portfolio.

Prior to joining Genzyme, Dr. Meeker held positions at the Cleveland Clinic and the Ohio State University. He has authored more than 40 articles and multiple book chapters.

Dr. Meeker is a Board member of BIO, Prize4Life, the California Institute of Healthcare and Savient Pharmaceuticals. Dr. Meeker received his M.D. from the University of Vermont Medical School. He completed an Internal Medicine residency at Beth Israel Hospital in Boston and a Pulmonary/Critical Care fellowship at Boston University. He completed the Advanced Management Program at Harvard Business School in 2000.

Why we chose Dr. Meeker:
As President of Genzyme’s Global Rare Disease Business, Dr. Meeker oversaw the global launches of Aldurazyme®, Fabrazyme®, and Myozyme®, life-changing therapies for patients with MPS I, Fabry disease and Pompe disease. He’s currently advocating for more NIH funding, education and support to patient organizations, as well as striving for more collaboration between the NIH and the FDA. Dr. Meeker also believes that patients should be consulted about the risk/benefit ratios of being administered an orphan drug.
Vincent Milano, President, CEO and Chairman of the Board, ViroPharma

Vincent Milano is ViroPharma’s President and Chief Executive Officer, and Chairman of the Board of Directors as of March 2008. He joined the company in 1996 and served as Vice President, Chief Financial Officer, and Treasurer from 1997 to 2006. In 2006, he assumed the role of Vice President, Chief Financial Officer, and Chief Operating Officer.

Mr. Milano has been instrumental in building ViroPharma, including leading efforts in raising nearly $900 million in capital and the acquisition of Vancocin(R) from Eli Lilly and Company. He has played a critical role in all business development and investor relations activities of the company, and has contributed significantly to establishing the strategic direction of the company.

Prior to joining ViroPharma, he was with KPMG LLP, independent certified public accountants, where he served as senior manager.

Mr. Milano received his bachelor of science degree in accounting from Rider College.

Why we chose Vincent:
Under Vincent’s leadership, Viropharma has become one of the most prominent orphan drug biotechs in the market, recognized for its appetite for acquisitions, and its amazing fundraising capability. This is a result of the company’s internal committment to advancing and translating science, and accelerating rare disease research.
Richard Moscicki, Deputy Director for Science Operations, CDER, U.S. Food and Drug Administration

Richard A. Moscicki, M.D. had been recently appointed for the newly created position of Deputy Director for Science Operations at the Center for Drug Evaluation and Research. He will help lead CDER in its role to regulate over-the-counter and prescription-only drugs available in the US.

Dr. Moscicki has previously served as Head of Genzyme Clinical Development after the recent integration with Sanofi. In his previous role as Chief Medical Officer and Senior Vice President, Clinical Development and Medical Affairs, he had worldwide responsibility for all aspects of clinical research and medical affairs for Genzyme. Prior to that, he was also responsible for worldwide regulatory and pharmacovigilance at Genzyme. He joined the company in 1992 as Medical Director. In his role at Genzyme, he was involved in the development and approval of nine products. Prior to joining Genzyme, Dr. Moscicki served as a staff physician at Massachusetts General Hospital and was Director of the training program in Allergy and Clinical Immunology for several years. At MGH, his research has been supported by several NIH awards. Dr. Moscicki has had a faculty appointment at Harvard Medical School since 1979.

Dr. Moscicki received his medical degree from Northwestern University in 1976. He served his residency in internal medicine (1976-79) at the Medical Center Hospital of Vermont. From 1979 to 1983, he held clinical and research fellowships at MGH and Harvard Medical School in clinical immunology and immunopathology. His is board certified in Internal Medicine; Allergy and Immunology; and Diagnostic Laboratory Immunology. Dr. Moscicki is a member of numerous professional societies and is an active member of committees of those societies and has served as an advisor to USP. He has more than 60 publications, and has provided editorial support for several professional journals.

Why we chose Dr. Moscicki:
Dr. Moscicki has more than 20 years experience developing orphan drugs. He is an out spoken advocate of patient registry optimization and collaboration to expedite clincial trials.
Dr. Mueller joined Vertex in July 2003. As Executive Vice President Global Research and Development & Chief Scientific Officer, he provides strategic oversight for Vertex’s worldwide drug discovery research programs, Pharmaceutical Development, Quality Assurance and Control, Pharmaceutical Operations as well as Clinical and Non-Clinical Development, Regulatory and Medical Affairs. Key areas of Vertex’ R&D are Hepatitis C, Cystic Fibrosis, IMID, Cancer, and Neurological Diseases, which in 2011 led to the successful approval and launch of INCIVEK (HepC), a NDA/MAA submission for KALYDECO (CF).

Prior to coming to Vertex, Dr. Mueller served as Senior Vice President, Research and Development, for Boehringer Ingelheim Pharmaceuticals, Inc. where he was responsible for the development of all drug candidates of the company’s worldwide portfolio in North and South America, Canada and Japan, beginning in 1997. He also led research programs in the areas of immunology, inflammation, cardiovascular disease and gene therapy on a global basis. During his time with Boehringer Ingelheim, Dr. Mueller oversaw the discovery of numerous development candidates, held several positions in basic research, medicinal chemistry and management in different centers of BI worldwide.

Dr. Mueller received both an undergraduate degree and a Ph.D. in Chemistry at the Albert Einstein University of Ulm, Germany, where he also holds a Professorship in Theoretical Organic Chemistry. He completed fellowships in Quantum Pharmacology at Oxford University and in Biophysics at Rochester University.

Why we chose Dr. Mueller:
Dr. Mueller oversaw the entire development process of the recently approved orphan drug for Cystic Fibrosis, Kalydeco. His leadership in strengthening the scientific collaborations with the Cystic Fibrosis Foundation and other stakeholders was essential for the successful launch of the drug in 2012. He has also been leading the studies to potentially expand Kalydeco’s label to include another group of cystic fibrosis.
Francois Nader, MD, has been president, chief executive officer and a member of the Board of Directors of NPS Pharmaceuticals since 2008. Dr. Nader is a 30-year veteran of the healthcare industry. He joined NPS in 2006 as chief medical and commercial officer and was promoted to chief operating officer in 2007. Previously, he was a venture partner at Care Capital. Dr. Nader served on the North America Leadership Team of Aventis and its predecessor companies and held a number of executive positions including senior vice-president, integrated healthcare markets and North America medical and regulatory affairs. Prior, Dr. Nader led the global commercial operations at the Pasteur Vaccines division of Rhone-Poulenc.

Dr. Nader currently serves as chairman of the Board of Trustees for BioNJ, New Jersey’s trade organization representing the biotechnology industry, and Board member of the New Jersey Chamber of Commerce. He formerly served on the Board of the Healthcare Institute of New Jersey and Noven Pharmaceuticals.

Dr. Nader earned his French Doctorate in Medicine from St. Joseph University (Lebanon) and his Physician Executive MBA from the University of Tennessee.

Why we chose Dr. Nader:
Dr. Nader drove the development and approval for NPS’S lead product, Gattex for Short Bowel Syndrome in Europe and US; which ultimately transformed the company into a leading global biotech focused on treatments for patients with rare disorders. Under Dr. Nader’s leadership NPS now plans to file its second product, Natpara for Hypoparathyroidism, in the US in 2013.
Richard Neal, US House of Representatives, Massachusetts

Richard E. Neal was born in Worcester, Massachusetts on February 14, 1949 and was raised and educated in the City of Springfield. He is a 1972 graduate of American International College, where he received his Bachelor's Degree in Political Science and was a member of the National Honor Society. He received his Masters Degree in Public Administration from the Barney School of Business and Public Administration at the University of Hartford in 1976.

Richard E. Neal was first elected to the United States House of Representatives in 1988. He represents the First Congressional District of Massachusetts. He is a senior member of the powerful Ways and Means Committee and the Ranking Member of the Subcommittee on Select Revenue Measures. He is also the dean of both the Massachusetts delegation and the New England Delegation in the House.

Congressman Neal has been a lead sponsor of legislation to prevent American companies from moving offshore to avoid paying U.S. taxes. He has sponsored legislation that would increase the national savings rate by encouraging the use of individual retirement accounts, and has worked to make health care and tuition expenses tax deductible for middle class people. He is also a member of the Ways and Means Trade Subcommittee.

Congressman Neal is an At-Large Whip for the House Democrats. He is a co-chairman of the New England Congressional Caucus, where he continues to advocate for the unique regional interests of the six New England States.

Why we chose Congressman Neal:
Congressman Neal co-sponsored the Preserving Access to Orphan Drugs Acts, bipartisan legislation that makes an important policy clarification and will remove a barrier to research and develop rare disease therapies.
Flemming Ornskov, Chief Executive Officer, Shire

Flemming Ornskov MD, MBA, MPH became the CEO of Shire in April, 2013.

Prior to this, Flemming was at Bayer where, as Chief Marketing Officer and Global Head, Strategic Marketing for General and Specialty Medicine he oversaw the full pharmaceutical product portfolio, with sales in excess of 10 billion Euros from global marketing units in Europe, China and the US.

Flemming qualified as a Doctor of Medicine at the University of Copenhagen Medical School and gained an MPH at Harvard University School of Public Health, Boston. His early medical and academic career included roles in internal medicine, emergency medicine and pediatrics, as well as clinical decision sciences at medical schools and hospitals in Denmark and France. He gained an MBA from the INSEAD business school in Fontainebleau, France.

He currently serves as non executive Chairman of the Board of Directors for biotechnology companies Santaris Pharma A/S and Evotec AG and is on the Board of Directors for PCI Biotech Holding ASA.

Why we chose Dr. Ornskov:
Dr. Ornskov is the CEO of Shire, a global leader in orphan drug development. While relatively new to the role, he is already starting to affect change at Shire, by combining R&D and business development with pre-clinical research, and BD focused on late-stage and in-licensing products.
Anne Pariser is the Associate Director for Rare Diseases in the Office of New Drugs at the FDA’s Center for Drug Evaluation and Research. Dr. Pariser is also actively involved in numerous collaborations within FDA and with drug developers, other governmental agencies, advocacy groups and other stakeholders to further the development of treatments for rare diseases. Dr. Pariser has worked at FDA since 2000. Prior to founding the Rare Diseases Program, she was a Medical Officer and Team Leader in OND where she worked almost exclusively on the review and regulation of products for rare genetic disorders.

Why we chose Dr. Pariser:
Dr. Pariser established the Rare Diseases Program in the Office of New Drugs in 2010, where she is currently working to support, facilitate and accelerate the development of therapeutics for rare diseases. The Rare Diseases Program concentrates on the development of biomedical and regulatory science; rare disease-specific training and education; and, policy and guidance generation for rare disease product review and regulation.
Gayatri Rao, Director, Office of Orphan Product Development, CDER, U.S. FDA

Gayatri Rao, MD, JD is the Director, Office of Orphan Products Development (OOPD) at FDA, working to advance the development and evaluation of medical products that demonstrate promise for prevention, diagnosis, or treatment of rare diseases.

The provisions of the Orphan Drug Act of 1983 and other amendments to the Federal Food, Drug, and Cosmetic Act established several major programs that the OOPD administers, which Dr. Rao oversees including the Orphan Grants Program, Orphan Drug Designation Program, Humanitarian Use Device Program, and Pediatric Device Consortia Program.

Dr. Rao comes to OOPD from FDA’s Office of the Chief Counsel where she provided advice on a wide range of issues related to medical devices, combination products, clinical trials, and human subject protection.

Dr. Rao graduated from the University of Medicine and Dentistry of New Jersey, New Jersey Medical School and earned both her law degree and bioethics masters degree from the University of Pennsylvania, where she concentrated on healthcare and FDA related issues. Following law school, she worked for a private law firm in Washington, D.C., focusing primarily on food and drug and other healthcare related matters, including matters related to orphan products.

Why we chose Dr. Rao:
Dr. Rao is the Director of the Office of Orphan Product Development at the FDA. She has led the efforts to launch and implement FDASIA, and oversees the office’s grants program to nurture the development of new and breakthrough orphan drugs.
Sir Michael Rawlins, President and CEO, Royal Society of Medicine

Sir Michael Rawlins was chairman of the National Institute of Health & Clinical Excellence (NICE) since its formation in 1999. He is also an Honorary Professor at the London School of Hygiene and Tropical Medicine, University of London, and Emeritus Professor at the University of Newcastle upon Tyne.

He was the Ruth and Lionel Jacobson Professor of Clinical Pharmacology at the University of Newcastle upon Tyne from 1973 to 2006. At the same time he held the position of consultant physician and clinical pharmacologist to the Newcastle Hospitals NHS Trust.


Why we chose Sir Michael:
Sir Michael is a leading global voice in the application of health economics to improve patient access and reimbursement for medicinal products. His contributions have helped advance the understanding of how to reconcile patients’ need for the continued development of new therapeutics for orphan conditions with society’s need to manage the costs of healthcare sustainably, whilst preserving incentives for companies to invest in research and development.
Peter Saltonstall, President and Chief Executive Officer, NORD

Peter L. Saltonstall is the President and CEO of the National Organization for Rare Disorders (NORD). He joined NORD in 2008 after having served for more than 30 years as a senior official in both for-profit and not-for-profit healthcare environments.

Peter is also committed to globalization of the rare disease patient community, as diseases do not recognize geographical boundaries and research can be expedited when patients from many counties are involved. He has established collaborative programs with patient communities in Europe and Japan.

Under Peter’s leadership, NORD also has updated and expanded its Patient Assistance Programs, which include assistance to patients in need of medications that they cannot afford; and has recommitted to facilitate research into new therapies and assure access by patients.

Why we chose Peter:
Under his leadership, NORD has forged new relationships between the patient groups, physicians, government agencies, drug and medical device companies, academia and investment communities. His efforts to build collaborations stems from his view that advances for the rare disease patient can be achieved best through joint efforts.
Hans Schikan is CEO of Prosensa, an innovative Dutch biopharmaceutical company focusing on the discovery, development and commercialization of novel treatments for rare diseases like Duchenne muscular dystrophy, myotonic dystrophy and Huntington’s disease, using its RNA modulation platform.

Before joining Prosensa, Hans worked at Genzyme for five years in various executive roles, including Vice President for Global Marketing and Strategic Development of Genzyme’s portfolio of products for rare genetic diseases. Prior to Genzyme, he spent 17 years at Organon, both at corporate level and in country operations which included assignments in Asia and Europe.

Aside from his role at Prosensa, Hans is currently Executive Board Member of the Dutch Top Institute Pharma and Non-executive Director of Swedish Orphan Biovitrum. He is also past Chairman of Nefarma, the Dutch Association of Research Based Pharmaceutical Industry.

He has a PharmD from Utrecht University. Hans has given numerous presentations on orphan drugs, rare diseases and innovation.

Why we chose Hans:
Under Hans’ leadership, Prosensa has become one of the industry’s most promising biotechs, largely due to the success of its life-changing therapy for Duchenne Muscular Dystrophy. Additionally Hans’ led the firms efforts to establish a key agreement with GlaxoSmithKline for part of its Duchenne compounds at a value of nearly USD 700 million.
Kathleen Sebelius, Secretary, US Department of Health and Human Services

Kathleen Sebelius was sworn in as the 21st Secretary of the Department of Health and Human Services (HHS) on April 28, 2009.

As part of the historic Affordable Care Act, she is implementing reforms that have ended many of the insurance industry’s worst abuses and will help 34 million uninsured Americans get health coverage. She is also working with doctors, nurses, hospital leaders, employers, and patients to slow the growth in health care costs through better care and better health.

Under Secretary Sebelius’s leadership, HHS is committed to innovation, from promoting public-private collaboration to bring life-saving medicines to market, to building a 21st century food safety system that prevents outbreaks before they occur, to collaborating with the Department of Education, to help states increase the quality of early childhood education programs, and give parents more information to make the best choices for their children.

Secretary Sebelius served as Governor of Kansas from 2003 until her Cabinet appointment in April, 2009, and was named one of America’s Top Five Governors by Time Magazine.

Why we chose Secretary Sebelius:
Since taking office, Secretary Sebelius has led ambitious efforts to improve America’s health and enhance the delivery of human services to some of the nation’s most vulnerable populations including young children, those with disabilities, the elderly, and the rare disease population.
In September 2000, Dr. Shrotriya joined Spectrum Pharmaceuticals as President and Chief Operating Officer and in August 2002, he was appointed Chief Executive Officer. In this capacity he has spearheaded major changes in business strategy and coordinated structural reorganization culminating in the formation of Spectrum Pharmaceuticals, Inc.

Previously, Dr. Shrotriya was Executive Vice President and Chief Scientific Officer for SuperGen, Inc. and Vice President, Medical Affairs and Vice President, Chief Medical Officer at MGI Pharma, Inc.

For 18 years he held various positions at Bristol-Myers Squibb Company, the most recent being Executive Director Worldwide CNS Clinical Research.

Dr. Shrotriya is a 2011 Ernst & Young Orange County Region Entrepreneur of the Year® award winner. Dr. Shrotriya was specifically cited for his turn-around of Spectrum. The Ernst & Young LLP award program recognizes entrepreneurs who demonstrate excellence and extraordinary success in such areas as innovation, financial performance and personal commitment to their businesses and communities. Dr. Shrotriya was chosen from 15 finalists by a panel of independent judges.

**Why we chose Dr. Shrotriya:**

Dr. Shrotriya led Spectrum to launch three drugs with emphasis on Belinostat, which is targeted to treat peripheral T-cell lymphoma (PTCL) and other solid tumors. The drug has been granted Orphan Drug and Fast Track designations by the FDA, and Spectrum expects to make a New Drug Application (NDA) filing by mid-2013, with an FDA decision anticipated in 2014.
Eduardo Suplicy, Senator, Brazil

Suplicy was the first elected senator in the history of the Brazilian Labour Party (PT). He has been a public servant for more than 20 years. He was first elected to serve as State Deputy from 1979-1983.

He is one of the founders of the Workers Party of Brazil (PT) and member of the Executive and the National Directory of the Party.

Suplicy was elected Senator of the Republic from 1991-1999, with 4,229,706 votes. He has occupied the position of leader of the PT in the Federal Senate for three terms. Since 1991, Suplicy has served as a Senator for the state of São Paulo. In 1991, he became the first member of the PT to take office as Senator. In the 1998 elections for the Federal Senate, Eduardo Suplicy conquered the biggest poll for this position in the Country and the second greatest of the history of São Paulo, with 6,718,463 votes.

Why we chose Senator Suplicy:
Senator Suplicy has been leading Brazil’s Senate in creating a policy framework dedicated to serving more than 13 million rare disease patients in the country. In 2011, he introduced the National Policy for Protection of Rare Disease Patients Rights, and is also working on an initiative to establish the National Fund for Rare Diseases which will support research for rare and neglected diseases in Brazil.
Marilyn Tavenner, Administrator, Centers for Medicare and Medicaid Services

Marilyn Tavenner is currently the Administrator for the Centers for Medicare & Medicaid Services. Previously, Ms. Tavenner was Principal Deputy Administrator for the Centers for Medicare & Medicaid Services (CMS). As the Principal Deputy Administrator, Ms. Tavenner served as the agency’s second-ranking official overseeing policy development and implementation as well as management and operations.

Prior to assuming her CMS leadership role, Ms. Tavenner served for four years as the Commonwealth of Virginia’s Secretary of Health and Human Resources in the administration of former Governor Tim Kaine. In this top cabinet position, she was charged with overseeing 18,000 employees and a $9 billion annual budget to administer Medicaid, mental health, social services, public health, aging, disabilities agencies, and children’s services.

Before entering government service, Ms. Tavenner spent 25 years working for the Hospital Corporation of America (HCA). She began working as a nurse at the Johnson-Willis Hospital in Richmond, Va., in 1981 and steadily rose through the company. By 1993, she began working as the hospital’s Chief Executive Officer and, by 2001, had assumed responsibility for 20 hospitals as President of the company’s Central Atlantic Division. She finished her service to HCA in 2005 as Group President of Outpatient Services, where she spearheaded the development of a national strategy for freestanding outpatient services, including physician recruitment and real estate development.

Ms. Tavenner holds a bachelor’s of science degree in nursing and a master’s degree in health administration, both from the Virginia Commonwealth University.

Why we chose Marilyn:
The recent structural changes in the US healthcare system, and the move for payers – both private and public – to become central in the discussion of orphan drug access and reimbursement will impact the industry considerably. As head of Medicare/Medicaid, Ms. Tavenner has a key role in addressing these challenges and helping patients improve access orphan drugs.
Henri Termeer, Founder and Former President, Genzyme

Prior to Genzyme Corporation’s acquisition in April 2011 by Sanofi-Aventis, Mr. Termeer was the company’s President, and a director of Genzyme Corporation since 1983, its Chief Executive Officer since 1985 and its Chairman since 1988.

Mr. Termeer is a member of the Board of Directors of the Massachusetts Institute of Technology and Partners HealthCare, the Chairman of the Federal Reserve Bank of Boston and is a member of the Board of Fellows of Harvard Medical School. He also serves on the Board of Directors of Pharmaceutical Research and Manufacturers of America.

Mr. Termeer studied economics at the Economische Hogeschool at Erasmus University in The Netherlands and received his Master of Business Administration from the Darden School of the University of Virginia.

Why we chose Henri:
Henri was a biotech pioneer long before anyone knew what biotechs were. He founded Genzyme which is often said to have kick started today’s orphan drug biotech M&A frenzy. Henri is definitely a mover/shaker in the biotech world and in the orphan drug space. He will always be known as the guy who figured out how to build a great business by making drugs for rare diseases. An inspiration and pioneer, many of his protégés have since moved on to lead other successful companies in the rare disease and biotech space thanks to his influence.
Senator Pat Toomey is a leader on economic, financial services, and budget issues. He is known as a champion of fiscal responsibility. The Philadelphia Inquirer wrote that he has emerged as "a leading voice on money matters."

He helped write and enact the bipartisan JOBS Act, which cuts regulatory burdens on small and medium-sized businesses making it easier for them to raise capital and create jobs. The senator serves on the Finance; Banking; Budget; and Joint Economic committees. Sen. Toomey is the chairman of the Senate Steering Committee - the coalition of Republican senators that advocates for innovative, conservative policies.

Sen. Toomey was previously elected to the House of Representatives, and fulfilled his three-term pledge. In addition to his public service, the senator was also the president of the Club for Growth, owned and operated a small restaurant chain in the Lehigh Valley, and worked in the financial services industry.

Pat and Kris Toomey live in Zionsville with their three children Bridget, Patrick, and Duncan.

**Why we chose Senator Toomey:**

US Senator Patrick Toomey, along with two other Senators introduced the *Preserving Access to Orphan Drugs Act of 2011*, a bipartisan legislation to ensure that the development of treatments for rare diseases is not jeopardized. This legislation ensures that unnecessary regulatory hurdles are not placed in front of innovative new therapies for rare diseases and conditions.
Patrick Vallance, President, Pharmaceuticals R&D, GlaxoSmithKline

Patrick was appointed President, Pharmaceuticals R&D, in January 2012. Prior to his appointment he was Senior Vice President, Medicines Discovery and Development. He is a member of the Corporate Executive Team. As president of R&D he is responsible for ensuring that GSK maintains a flow of potential new medicines through the R&D pipeline from early discovery through to approval.

Patrick joined the company in May 2006 as Head of Drug Discovery. He transformed GSK’s discovery engine to focus on therapy areas that are underpinned by the most promising and mature science, and which offer fresh insights into diseases. Patrick has also re-personalized Drug Discovery by setting up small, empowered teams, called Discovery Performance Units, to drive success of potential new medicines in the pipeline. This new approach has led to a number of potential new medicines progressing into late-stage development.

Why we chose Patrick:
Patrick was involved in the launching of GSK’s Rare Disease Unit in 2010, and was instrumental in structuring many of GSK’s key partnerships in the rare disease space including Angiochem and Prosensa. Patrick also oversees GSK’s innovative approaches to expedite and optimize clinical trials through patient registries.
Rogerio Vivaldi, Senior Vice President and Head of Rare Diseases, Genzyme

Rogerio Vivaldi, MD, MBA is SVP and Head of Rare Diseases at Genzyme. Dr. Vivaldi brings a mission-driven, patient-centric approach to his leadership, and has built critical bridges on behalf of Genzyme with top government, regulatory and patient organizations.

Prior to leading Rare Diseases, he was head of Genzyme’s Renal and Endocrinology unit. Earlier, he established Genzyme’s presence in Brazil and secured access to life-altering therapies for thousands of patients. Later he was appointed President of Genzyme in Latin America.

Dr. Vivaldi had his own private practice in the specialty area of Diabetes and Endocrinology for 18 years. He was the first doctor to treat a patient in Brazil with enzyme replacement therapy for Gaucher in 1992, and has authored several publications on Gaucher disease.

Dr. Vivaldi received his medical degree from Universidade do Rio de Janeiro Medical School, and his MBA from Copead – Universidade Federal do Rio de Janeiro.

Why we chose Dr. Vivaldi:
Dr. Vivaldi heads one of the most successful rare disease units in pharma. EURORDIS even celebrated Genzyme’s accomplishments and dedication to rare diseases with an award. The company was honored for pioneering the development and delivery of therapies for rare diseases, the main thrust of its business. Dr. Vivaldi’s unit was also recognized for its longstanding support of patient organizations - including EURORDIS - as well as initiatives to increase patient access to Genzyme treatments.
Durhane Wong-Rieger, President, Canadian Organization for Rare Disorders

Durhane Wong-Rieger, PHD is President and CEO of the Institute for Optimizing Health Outcomes. She is also president of the Canadian Organization for Rare Disorders and head of Consumer Advocare Network, a national net-work to promote patient engagement in healthcare policy and advocacy. Internationally, she serves as Chair of the Board of the International Alliance of Patient Organizations and is Co-Vice-Chair of the Steering Committee of Health Technology Assessment International Interest Group on Patient/Citizen Involvement. She is a licensed T-Trainer with the Stanford-based Living A Healthy Life with Chronic Conditions.

She has served on numerous health policy advisory committees and panels, including Project Coordinator for the Policy Dialogues for the Commission on the Future of Healthcare in Canada and consultant to the Ontario Premier’s Advisory Board on Organ Donation. She is a member of Health Canada’s Expert Advisory Committee on Vigilance of Health Products and Expert Advisory Panel on Special Access Programme.

From 1984 to 1999, Durhane was professor of psychology at the University of Windsor in Ontario, Canada. Durhane has a BA in psychology from Barnard College in New York City and an MA and PhD in social psychology from McGill University in Montreal. She is author of two books and many articles and a frequent lecturer and workshop leader.

Why we chose Dr. Wong-Rieger:
Dr. Wong-Rieger has conducted extensive training, workshops, and evaluation on all aspects of patient engagement and advocacy with the purpose of enhancing multi-stakeholder collaboration in Canada. These efforts had a great impact in enhancing patient engagement with other stakeholder to accelerate rare disease research and to advocate for more cohesive policies to expedite orphan drug development.
Janet Woodcock, Director, CDER, U.S. Food and Drug Administration

Dr. Woodcock has led many of FDA’s drug initiatives. She introduced the concept of risk management in 2000 as a new approach to drug safety. Since 2002, she has led the “Pharmaceutical Quality for the 21st Century Initiative,” FDA’s highly successful effort to modernize drug manufacturing and its regulation. In 2004, she introduced FDA’s “Critical Path” Initiative, which is designed to move medical discoveries from the laboratory to consumers more efficiently.

Dr. Woodcock previously served as FDA’s Deputy Commissioner and Chief Medical Officer. She also led CDER as director from 1994–2005.

Dr. Woodcock received her medical degree from Northwestern University Medical School, and her undergraduate degree from Bucknell University. She has held teaching appointments at Pennsylvania State University and the University of California at San Francisco. She joined FDA in 1986.

Why we chose Dr. Woodcock:
Dr. Woodcock launched the “Safety First” and “Safe Use” initiatives designed to improve drug safety management within and outside the FDA. Prior to joining CDER, she oversaw approval of the first biotechnology-based treatments for multiple sclerosis and cystic fibrosis in her position as director of the Office of Therapeutics Research and Review in FDA’s Center for Biologics Evaluation and Research (CBER).
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