

The Unique Challenges and Opportunities in Rare Disease R&D

Webinar | Nov 14, 2014 | 10AM PST, 1PM EST, 6PM GMT

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Invited Speakers



Philip Reilly MD, JD
Venture Partner
Third Rock Ventures



**Alexandra
Glucksmann, PhD**
Chief Operating Officer
Editas Medicine



Phil Vickers, PhD
Global Head of Research
and Development, Shire



Gerald Cox, MD, PhD
Vice President of Clinical
Development, Genzyme,
a Sanofi company



Richard Soll, PhD
SVP and Head of
International Discovery
Service Unit, WuXi AppTec

Overview

One in every ten Americans has a rare disease, a health condition by definition that afflicts fewer than 200,000 people per year in the United States and collectively affects an estimated 25 million people. Treatment options are few as only 400 FDA-approved drugs are available to treat the diverse spectra of 7,000 rare diseases. In this complimentary live webinar, WuXi brings together key thought leaders and players to explore the unique challenges and opportunities in the search for effective therapies to address this large unmet medical need.

Speaker Biography

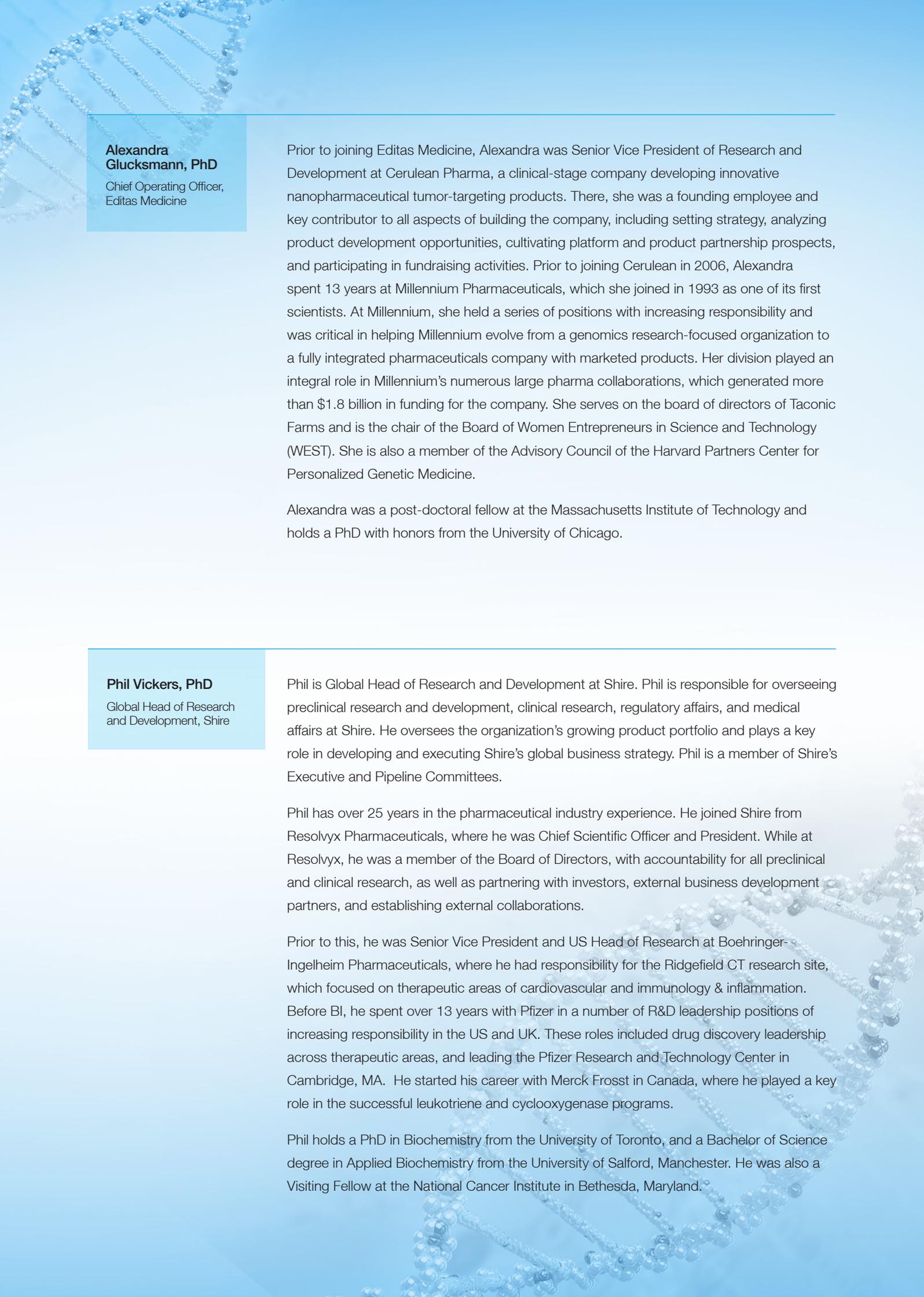
Philip Reilly, MD, JD

Venture Partner
Third Rock Ventures

Phil joined Third Rock Ventures in 2009 to discover, launch and build companies pursuing breakthrough treatments for rare genetic diseases. He is a highly respected clinical geneticist with an extensive track record of launching and building companies in the rare disease space.

In addition to his role at Third Rock, Phil is currently a trustee of Cornell University and is on the board of overseers of Weill Cornell Medical College. Prior to joining Third Rock, he was the chief executive officer and chairman of Interleukin Genetics, and before that, he was the executive director of the Eunice Kennedy Shriver Center for Mental Retardation. He served twice (2000 and 2003) as president of the American Society of Law, Medicine and Ethics. He is a founding fellow of the American College of Medical Genetics. He has authored or co-authored more than 100 articles in scholarly journals as well as several books about clinical genetics. Phil has held teaching positions at Tufts University School of Medicine and Harvard Medical School. Phil received his BA from Cornell University and holds an MD from Yale University, a JD from Columbia University, is a member of the Massachusetts Bar and is board certified in internal medicine and clinical genetics.

When he was much younger, Phil spent a year bartending in the Caribbean.



**Alexandra
Glucksmann, PhD**

Chief Operating Officer,
Editas Medicine

Prior to joining Editas Medicine, Alexandra was Senior Vice President of Research and Development at Cerulean Pharma, a clinical-stage company developing innovative nanopharmaceutical tumor-targeting products. There, she was a founding employee and key contributor to all aspects of building the company, including setting strategy, analyzing product development opportunities, cultivating platform and product partnership prospects, and participating in fundraising activities. Prior to joining Cerulean in 2006, Alexandra spent 13 years at Millennium Pharmaceuticals, which she joined in 1993 as one of its first scientists. At Millennium, she held a series of positions with increasing responsibility and was critical in helping Millennium evolve from a genomics research-focused organization to a fully integrated pharmaceuticals company with marketed products. Her division played an integral role in Millennium's numerous large pharma collaborations, which generated more than \$1.8 billion in funding for the company. She serves on the board of directors of Taconic Farms and is the chair of the Board of Women Entrepreneurs in Science and Technology (WEST). She is also a member of the Advisory Council of the Harvard Partners Center for Personalized Genetic Medicine.

Alexandra was a post-doctoral fellow at the Massachusetts Institute of Technology and holds a PhD with honors from the University of Chicago.

Phil Vickers, PhD

Global Head of Research
and Development, Shire

Phil is Global Head of Research and Development at Shire. Phil is responsible for overseeing preclinical research and development, clinical research, regulatory affairs, and medical affairs at Shire. He oversees the organization's growing product portfolio and plays a key role in developing and executing Shire's global business strategy. Phil is a member of Shire's Executive and Pipeline Committees.

Phil has over 25 years in the pharmaceutical industry experience. He joined Shire from Resolvix Pharmaceuticals, where he was Chief Scientific Officer and President. While at Resolvix, he was a member of the Board of Directors, with accountability for all preclinical and clinical research, as well as partnering with investors, external business development partners, and establishing external collaborations.

Prior to this, he was Senior Vice President and US Head of Research at Boehringer-Ingelheim Pharmaceuticals, where he had responsibility for the Ridgefield CT research site, which focused on therapeutic areas of cardiovascular and immunology & inflammation. Before BI, he spent over 13 years with Pfizer in a number of R&D leadership positions of increasing responsibility in the US and UK. These roles included drug discovery leadership across therapeutic areas, and leading the Pfizer Research and Technology Center in Cambridge, MA. He started his career with Merck Frosst in Canada, where he played a key role in the successful leukotriene and cyclooxygenase programs.

Phil holds a PhD in Biochemistry from the University of Toronto, and a Bachelor of Science degree in Applied Biochemistry from the University of Salford, Manchester. He was also a Visiting Fellow at the National Cancer Institute in Bethesda, Maryland.



Gerald Cox, MD, PhD

Vice President of Clinical Development, Genzyme, a Sanofi company

Gerry is Vice President of Clinical Development in the Rare Diseases Group at Genzyme, a Sanofi company. He also is a practicing clinical geneticist at Boston Children's Hospital and an Instructor in Pediatrics at Harvard Medical School. Since joining Genzyme in 2000, Gerry has been involved in the development of several enzyme replacement therapies (ERT) and substrate reduction therapies (SRT) for lysosomal storage disorders. He played a key role in the worldwide approval of Aldurazyme (laronidase) for Mucopolysaccharidosis type I in 2003, Japan approval of Elapraxe (idursulfase) for Mucopolysaccharidosis type II in 2007, and US approval of Cerdelga (eliglustat) for Gaucher disease type 1 in August 2014. His group recently completed a successful phase 1b study of ERT for Niemann-Pick disease type B and is planning phase 2 studies of a CNS-accessible SRT for neurodegenerative glycosphingolipidoses.

Gerry graduated magna cum laude with a BA in biology from Harvard College in 1980 and obtained an MD and a PhD in biology from UC San Diego in 1989. Following a pediatrics residency and clinical and research fellowships in genetics at Boston Children's Hospital, he became a staff physician and directed the Human Genomic Mapping Facility. Gerry is board-certified in Clinical, Biochemical, and Molecular Genetics and formerly in Pediatrics. He served on the scientific advisory boards of the Barth Syndrome Foundation and the Pediatric Cardiomyopathy Registry, and he has authored more than 60 peer-reviewed publications. In addition to lysosomal storage disorders, Gerry's clinical interests include genetic causes of cardiomyopathy and eye disease.

Richard Soll, PhD

SVP and Head of International Discovery Service Unit, WuXi AppTec

Richard Soll, PhD is SVP and Head of International Discovery Service Unit at WuXi AppTec, a Shanghai headquartered and New York Stock Exchange listed (NYSE: WX) premium provider of pharmaceutical R&D services across the pharmaceutical R&D value chain. In this capacity, Rich has advanced more than 40 integrated programs across major target classes and therapeutic indications from hits to lead optimization campaigns, giving rise to clinical candidates including those designated by the FDA as breakthrough therapies. Previously, Rich was CSO and VP of R&D at TargeGen where he led innovative clinical-stage R&D programs including a highly selective JAK2 inhibitor fedratinib (SAR302503; TG101348) for the treatment of myeloproliferative disorders, leading to the acquisition of TargeGen by Sanofi. Before TargeGen, Rich founded the chemistry department at 3-Dimensional Pharmaceuticals as VP of Chemistry and spent 10 years at Wyeth Pharmaceuticals. Rich has been an SAB member to biotechs, advisor to entrepreneurs, and has produced more than 100 patents and papers.