Orphan Pharma: pathfinders for an increasingly specialised industry
Foreword

We are continuing our series by profiling the R&D and clinical development leadership of European and North American specialty pharma companies with orphan drug indication.

As companies increasingly specialise around specific diseases or patient groups, there is an increasing profile for specialty pharma. Orphan drugs are part of the market for specialty pharmaceuticals and refer to products used for diagnosis, prevention or treatment of rare diseases that are chronic, debilitating and often life-threatening.

Our expertise in this sector is evidenced by a breadth of talent projects across all functions of orphan & specialty pharma across the world.

Chris Molloy, CEO
The Orphan Market Sector

A new pharma world without parents

In discovery research the orphan approach has broken the traditional mould of blockbuster, broad market treatments and created a model for how to discover, develop and market therapeutics to well-defined patient populations.

Orphan targets were originally thought of as viable drug research targets without a validated link to disease. They were a riskier approach to the traditional ‘1 gene - 1 protein - 1 compound’ drug discovery, still much favoured by the ‘parent generation’ of megapharma. Today this ‘systems biology’ approach, validated through real world patient samples, has become the new frontier of therapeutics research.

So too in clinical development, ‘orphan’ drug designation is given to therapeutic approaches highly targeted to a rare disease or condition. This designation has allowed these nominally ‘parentless’ assets to thrive. Worldwide orphan drug sales are forecast to total $176 billion (CAGR 2014 to 2020:+10.5%); almost double overall prescription market growth (excluding generics). Orphans are also set to be 19.1% of worldwide prescription sales by 2020.

The National Organization for Rare Disorders (NORD) estimates 30 million Americans suffer from 7,000 rare diseases. Prior to the 1983 Act, 38 orphan drugs were approved. Until 2014, 468 indication designations covering 373 drugs had been approved.

Impact of orphan drug companies

Orphan drugs cost less in clinical development than broad market treatments: trial populations are smaller, and can be rapidly recruited because the patient community is highly motivated by lack of focused treatments. These patients and their clinicians also tend to be highly engaged through active patient communities. The good news extends into the market too. In today’s market where proven outcomes influence pricing, orphan stands tall. On average they command higher prices when they launch: their cost per patient is six times that of non-orphan drugs.

The ability to target focused, engaged patient populations and key opinion leaders has another tectonic effect on the industry. Biotech companies who would have traditionally licensed clinical assets to megapharma are now choosing to ‘hold’. They are raising money based on low-risk-to-market assets, then launching and marketing them directly or through outsourced sales channels. It is no wonder that megapharma is adopting the ‘orphan’ approach as it reassesses its viability and business model.
Learning the lessons for the next generation of increasingly specialised pharma

Despite the word orphan we should really be looking at this sector as a driver for the broader group of ‘specialised pharma’, leading the way toward a more targeted, precise future for therapeutics.

We have focused our Talent Equity® research outside the spotlights of megapharma and of oncology and haematology, to examine how mid-size pharma/biotech firms are bringing targeted therapeutics through R&D. We analysed 63 European and North American pharmaceutical and biotech companies, 1002 orphan drug designations and 157 marketing approvals to identify the top 10 performers.

We then looked at the CMOs who have driven focused clinical evaluation and for the earlier stage leaders, the CSOs who have brought clinical candidates through to designation. These people represent the R&D faculty we should look to for education on how this industry will evolve.

What we show is a group of highly focused CSOs with a passion for the diseases they are targeting and a tight grip on the technologies they employ to craft their candidates. Coupled to this are seasoned CMOs, most trained in the schools of megapharma, that demonstrate a passion for the patient and their need to engage with them.

A word on culture

A new generation always develops a youth culture, which becomes the new normal. So it is in the shift from ‘blockbuster’ towards precision therapeutics. Specialised and orphan pharma companies do more than talk about the power of the patient and engagement with them. We find that they truly live it and derive their corporate culture from the one thing they - and we - are all focused on: better people.
Key Talent Equity® Themes

Our leading companies in the sector have CMOs and CSOs with shared skills and characteristics. The leading indicators of success in these companies include:

**Innovation & pioneering**
Due to the small patient populations for orphan drugs, most of the featured CMOs have had to pioneer new approaches to clinical development, regulatory, approval and medical affairs. Most of the CSOs have succeeded in translating science into first-in-class therapies for previously untreatable rare diseases.

**Ability to build teams**
Many of the featured leaders have a remarkable track record of building successful clinical development or research teams to accommodate various product development stages and to advance product towards marketing approval.

**Big pharma experience**
Most of the featured leaders gained their diverse experience at a larger pharma or a non-orphan biotech. This allowed them to rotate through various leadership roles with different stakeholders and objectives.

**Multiple therapeutic area expertise**
Besides their experience in conducting a clinical research development at a large pharma, many of the CMOs have worked across multiple therapeutic areas in different stages of development. This is in contrast with CSOs, who gained their experience at specialised small cap biotech.

**Scientific leadership**
All of the featured CSOs have made remarkable contributions to their field of study. These scientific leaders have published multiple research papers and have numerous patents under their name.

Big pharma background map

![Big pharma background map](image)

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<th>Average No. of Publications</th>
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Objective Methodology

Our analysis identifies the leading scientific and clinical development talent in Europe and in the United States. Through our study of the recent regulatory wins - orphan drug designations and marketing approvals - we have identified 10 Chief Medical Officers and five Chief Scientific Officers who drive their company’s success in the orphan drug field.

We conducted an analysis of all of the pharmaceutical and biotech companies that have received orphan drug marketing approvals and/or orphan drug designations between 2012 and 2014. Further, our focus was on companies that have obtained orphan drug designations in both Europe and the United States, not specialising in the fields of oncology or haematology. We used data from EvaluatePharma and other industry sources and analysed 63 European and North American pharmaceutical and biotech companies, 1002 orphan drug designations and 157 marketing approvals. To identify our top picks we applied objective metrics, our talent expertise and industry knowledge in the selection process.

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<th>COMPANY</th>
<th>Orphan Drug Marketing Approvals USA</th>
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<th>Orphan Drug Designations USA</th>
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<td>Actelion</td>
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<td>uniQure</td>
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<td>Vertex Pharmaceuticals</td>
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Objective Methodology

Unmet need:
There are over 7,000 identified rare diseases and fewer than 500 of them with treatments. These disorders affect 6-7% of the global population, which in comparison to common conditions, such as hypertension, (affecting 40% of population over 25), characterise a very small market opportunity.

In the past these were unattractive to drug developers but increasingly these specific treatments reflect the reality of some of even the widest spread diseases, which are in reality a combination of many discrete patient populations.

Positive impact of Orphan Drug Act

Regulatory incentives:
The paradigm shift for orphan drug development occurred in 1983 with the Orphan Drug Act, which provided incentives such as tax credits, FDA fee waiver, grants and seven-year drug exclusivity for companies tackling the unmet need. Europe, Japan and other regions have since implemented similar policies. These and additional incentives such as premium pricing, shorter timelines and FDA’s/EMA’s flexibility due to the lack of alternative treatments, together with a faster uptake of treatments in the market, have positively impacted the rare diseases space.
Small patient population with premium pricing:

Despite the challenge of patient-centricity, big pharma actively participates in the orphan space to compensate for drying pipelines and increased competition from generics. In addition to the regulatory incentives, this field has a set of positive unique characteristics. From the clinical development perspective, a small patient pool translates into a small clinical trial with potential high return on investment. From the commercial point of view, the severity of an orphan disease and the lack of alternative treatments create price inelasticity. In 1994 one of the pioneers of the orphan drug space, Genzyme, now a Sanofi company, launched Cederase for Gaucher disease with an annual cost per patient of ~ $200,000. Since then, not only have some orphan drug companies kept pricing high, but in many cases have brought it to new heights. The most frequently quoted case is Alexion’s Soliris for paroxysmal nocturnal haemoglobinuria, with a price tag of $400,000 per patient per year. Recently the world’s first approved gene therapy treatment for lipoprotein lipase deficiency, Glybera, developed by uniQure, set a new record with a staggering price tag of over $1 million for a single treatment.
RSA Sector Equity™
Orphan Companies

Our 10 company picks (in alphabetical order)

Actelion
Aegerion
BioMarin
Emergent Biosolutions
Hyperion

PTC Therapeutics
Raptor Pharmaceuticals
Sobi
uniQure
Vertex

With a niche, strong pipeline, Actelion is a pioneer of the biotech industry in Switzerland. Currently the company is focused on organic growth and the boosting of its returns. Actelion’s new treatment for pulmonary arterial hypertension, Opsumit, had a better than expected launch.

USA OD: 12, EU OD: 11, USA OMA: 5, EU OMA: 4

Aegerion diversified its pipeline with the acquisition of an orphan drug from AstraZeneca, Myalept, in November 2014. For its inaugural FDA-approved orphan drug Juxtapid, the company won approvals in Mexico and Canada last year.

USA OD: 3, EU OD: 1, USA OMA: 2

BioMarin expanded its reach into the Duchenne muscular dystrophy market with their $840 million deal to buy Prosensa in November 2014. The company also won marketing approvals for VIMIZIM in the EU, Japan, Brazil, Australia and Canada throughout the year.

USA OD: 10, EU OD: 8, USA OMA: 4, EU OMA: 3

With Cangene’s $222 million acquisition, Emergent BioSolutions added the antitoxin to botulism to its portfolio last year. Most recently the company won an orphan drug designation from the FDA for BioThrax, an anthrax vaccine.

USA OD: 10, EU OD: 4, USA OMA: 4
Hyperion has demonstrated strong commercial execution of its two drugs, Ravicti and Buphenyl in 2014. Its Phase II study of Glycerol Phenylbutyrate (GPB) for the treatment of hepatic encephalopathy has met primary points. The company was acquired by Horizon Pharma for $1.1 billion in July 2015.

USA OD: 3, EU OD: 7, USA OMA: 1

Raptor’s PROCYSBI has won FDA and EMA approval for the Treatment of Nephropathic Cystinosis. The drug is currently being further developed in advanced clinical trials for three additional indications: Huntington’s disease, NAFLD and Leigh syndrome. In August 2015 Raptor expanded their rare disease portfolio with the acquisition of Quinsair, the first inhaled fluoroquinolone, for the management of chronic pulmonary infections due to Pseudomonas aeruginosa in adults with Cystic Fibrosis.

USA OD: 4, EU OD: 2, USA OMA: 1, EU OMA: 2

uniQure, a European gene therapy pioneer, has made a comeback to the public markets by raising $106 million through an IPO last year. Currently, the company is focused on Glybera’s launch and clinical development of its treatment for haemophilia B. uniQure has also expanded into heart disease by acquiring Inocardi last year.

USA OD: 3, EU OD: 5, EU OMA: 1

In August 2014, PTC Therapeutics received conditional marketing approval for its nonsense mutation Duchenne treatment, Translarna. The company is conducting a Phase III study of its lead drug, Ataluren, and is expected to announce the results at the end of Q4 this year.

USA OD: 4, EU OD: 3, EU OMA: 3

With a focus on orphan drugs, Sobi has scored multiple product partnerships with companies such as Biogen and Exelixis (recently-extended). With a EU/US presence, Sobi has a well-diversified pipeline in clinical development. Most recently, the FDA approved its drug for haemophilia B and CHMP provided a positive opinion on its treatment for Peyronie’s disease.

USA OD: 6, EU OD: 1, USA OMA: 2

Since Kalydeco’s approval in December 2012, Vertex has worked to extend its reach to more cystic fibrosis patients. Its unique treatment for the underlying cause of CF has won FDA approval for treating patients with multiple genetic mutations and, most recently, a paediatric designation for children aged 2-5 years.

USA OD: 5, EU OD: 4, USA OMA: 1, EU OMA: 1
Birgitte Volck
CMO - Swedish Orphan Biovitrum

**Background:** Clinical development/scientific research

**Key traits:** Multiple therapeutic area expertise, scientific expertise in translational research, big pharma & biotech experience

**Education:** MD, PhD - University of Copenhagen

Birgitte has been serving as the CMO at Sobi since 2012. She leads the medical organisation, clinical development, regulatory affairs, drug safety, advance life cycle management and late stage pipeline projects.

**Orphan achievement (for the period of 2012-2014):**

- **US:** Orphan Marketing Approval: 21/12/12 – Kineret (CAPS)

**Academic experience:**
No. of publications: 9

Birgitte was engaged in multiple clinical and scientific assignments within rheumatology at the Copenhagen University Hospital

**Patents:** 1

**Biotech experience:**
Birgitte held multiple senior positions within Amgen. She served in several executive roles at Amgen, such as an Executive Development Director of Bone, Neuroscience & Inflammation and Executive Medical Director, Nordic & Baltic Region. Earlier, she served as Nordic Medical Director at Genzyme and VP of Clinical Development and Medical Affairs at Pharmexa.
Bruce Scharschmidt  
CMO - Hyperion Therapeutics (Acquired by Horizon Pharma on 5/7/15)

**Background:** Research/ academic/clinical development  
**Key traits:** Thought leader, big pharma & biotech experience, GI/liver disease therapeutic expertise  
**Education:** MD - Northwestern University

Patents: 15  
**Big Pharma experience:** Bruce served as VP in Novartis Vaccines Division after its acquisition of Chiron and led clinical development of early stage vaccines.  
**Biotech experience:** Bruce headed clinical development of therapeutics and vaccines at Chiron, where he was involved in the management of key research programmes.

As CMO, Bruce led the clinical development of Ravicti to its orphan drug marketing approval in 2013. He was an active member of Hyperion’s road show team leading to its successful 2012 IPO. He is board certified in internal medicine and gastroenterology.

**Orphan achievement (for the period of 2012-2014):**  
US: Orphan Marketing Approval: 1/2/13 – Ravicti (UCD)  

**Academic experience:**  
No. of publications: 200  
Professor of Medicine & Chief of Gastroenterology at UCSF Association, Editor of Gastroenterology Editor-in-Chief of the Journal of Clinical Investigation, President of the American Society for Clinical Investigation, Chair of the Translational Team for the J. David Gladstone Institute

Christian Meyer  
CMO - uniQure

**Background:** Clinical/ regulatory affairs  
**Key traits:** Previous rare disease experience, multiple therapeutic area expertise, big pharma & biotech Experience  
**Education:** MD, PhD in Clinical Cardiology University of Copenhagen

**Biotech experience:**  
Prior to uniQure he served as CMO of Cardoz, holding responsibility for the clinical development of an investigational anti-inflammatory drug. Earlier, he served as the SVP of Clinical Development and Regulatory Affairs at Symphogen, advancing the company’s leading compound into clinical development, which led to a licensing deal with Merck. As Director of Clinical Development at Zymenex, he led a rare genetic disease treatment, Metazym, into clinical development. Zymenex was acquired by Shire in a cash deal.

**Big Pharma experience:**  
Christian held multiple roles with an increasing responsibility during his career at Novo Nordisk. As an International Medical Officer he managed clinical trials in multiple therapeutic areas.

**Orphan achievement (for the period of 2012-2014):**  
EU: Orphan Designation: 3/21/12 AMT-010 (PH1)  
Orphan Marketing Approval: 25/10/12 Glybera (lipoprotein-lipase deficiency)

Since his appointment as the CMO of uniQure, Christian has built an experienced clinical team to advance the company’s pipeline in clinical development. He is leading clinical development and post market support of clinical stage products in multiple therapeutic areas.
Guy Braunstein  
Head of Global Clinical Development - Actelion

**Background:** Clinical development  
**Key traits:** Multiple therapeutic area expertise, multiple large pharma experience, medical affairs  
**Education:** MD and PhD in Life Science from Université de Paris

As the Head of Global Clinical Development, Guy leads Actelion in advancement of its clinical capabilities and drives the development of innovative compounds for the company’s existing and future pipeline. During his time in large pharma he gained experience in multiple therapeutic areas, in particular respiratory, inflammation and autoimmune disorders, multiple sclerosis and oncology.

**Orphan achievement (for the period of 2012-2014):**  
**US:** Orphan Marketing Approval: 18/10/13 – Opsumit (PAH)  
**EU:** Orphan Designation: 21/3/12 – AMT – 010 (PH1)  
Orphan Marketing Approval: 25/10/12 Glybera (lipoprotein-lipase deficiency)

**Academic experience:**  
No. of publications: 19

**Big pharma experience:**  
Prior to joining Actelion, Guy served as the CMO at Merck Serono. Throughout his career at Merck he held a wide range of roles from Medical Director and Global Medical Affairs through to Director of Research and Development. He also held multiple executive positions at Astra, Glaxo-Wellcome, GSK, Fisons, Rhone Poulenc Rorer and Chiron.

Harald Petry  
CSO – uniQure

**Background:** Academic research  
**Key traits:** Drug Discovery, Scientific Expertise in Immunology, Academic  
**Education:** PhD in Biology Justus-Liebig-Universität Giessen

Harald has served as CSO of uniQure since January 2012. uniQure’s Glybera is the first gene therapy approved for treatment in the EU and the first medication approved for patients with Lipoprotein Lipase Deficiency. Harald leads the development of a modular platform to develop gene therapy that is curative through single treatment.

**Orphan achievement (for the period of 2012-2014):**  
**EU:** Orphan Designation: 21/3/12 AMT – 010 (PH1)  
Orphan Marketing Approval: 25/10/12 Glybera (lipoprotein-lipase deficiency)

**Academic experience:**  
No. of publications 95

**His academic background includes a 10-year research career at the German Cancer Center in Heidelberg and the German Primate Centre in Göttingen in the areas of vaccines and gene therapy.**

**Patents:** 23

**Biotech experience:**  
Since 2007 he has been Director of Research at AMT (uniQure’s predecessor company). He led the discovery of AMT’s lead product for Lipoprotein-Lipase Deficiency, its first gene therapy. He served as a senior scientist and a project leader at Berlex Biosciences, where he led the research group in the gene therapy department. Earlier, as a senior scientist and a project leader at Jenapharm, he established a gene therapy working group.
Henry Fuchs  
CMO - BioMarin Pharmaceutical

**Background:** Clinical/operational/executive  
**Key traits:** Operational experience, big pharma & biotech experience, multiple therapeutic area expertise  
**Education:** MD George Washington University; BA Biochemical Sciences Harvard University

Henry was appointed as BioMarin’s CMO in 2009. He is leading BioMarin’s ongoing clinical development efforts to continue laboratory-to-marketplace execution in the orphan drug space. He is a highly capable contributor to company’s business and corporate development efforts, such as Prosensa’s acquisition in 2014.

**Orphan achievement (for the period of 2012-2014):**  
**US:** Orphan Designation: 25/11/14 – BMN 250 (MPS IIIB); 17/1/13 BMN-111 (achondroplasia)  
Orphan Marketing Approval: 14/2/14  
Vimizim (Morquio A syndrome)

**Big pharma experience:**  
During his tenure as Senior Director at Genentech he was responsible for the advanced clinical development for Herceptin and the approval of Pulmozyme.

**Biotech experience:**  
As CMO of Onyx Pharmaceuticals, he led a successful study of Nexavar and expanded its treatment into additional types of cancer. He held multiple operational and clinical roles with increasing responsibility and served on the board of directors at Ardea Biosciences (acquired by AstraZeneca). Starting as VP Clinical he rose to CEO during his eight year career at Intrabiotics.

W. James Jackson  
CSO - Emergent BioSolutions

**Background:** Research/operational  
**Key traits:** Scientific expertise in immunology, biotech expertise, multiple therapeutic area expertise  
**Education:** PhD in Microbiology - University of Georgia  
BA in Microbiology/Biochemistry - University of Tennessee

James has served as CSO of Emergent BioSolutions since February 2008. Prior to that he held multiple senior operational roles within the company. He led research efforts related to antigen identification and characterisation, preclinical evaluation, and initial development of bacterial vaccines in multiple therapeutic areas.

**Orphan achievement (for the period of 2012-2014):**  
**US:** Orphan Designation: 4/11/2014 BioThrax (anti-infective)  
Orphan Marketing Approval: 22/3/13 BAT (botulism); 20/12/12 Varizig (Varicella Zoster treatment)

**Academic experience:**  
No. of publications: 53
Jeffrey Chodakewitz
CMO - Vertex Pharmaceuticals

**Background:** Clinical development

**Key traits:** Multiple therapeutic areas expertise, big pharma experience, academic

**Education:** MD Yale University School of Medicine; BSc in Biochemistry Yale University

Jeffrey joined Vertex as CMO in January 2014. He leads all clinical development efforts and oversees global regulatory affairs, medical affairs, drug safety and other related functions. He oversees company’s research and development efforts in cystic fibrosis and other indications.

**Orphan achievement (for the period of 2012-2014):**

**US:** Orphan Designation: 24/4/14 - VX-661 (investigational CF drug)
Orphan Marketing Approval: 31/1/2012 – Kalydeco (CF treatment)

**EU:** Orphan Designation: 7/4/14 - VX-661 (investigational CF drug);
22/8/14 - Lumacaftor/ivacaftor VX – 809 (investigational combined CF treatment)
Orphan Marketing Approval: 23/7/12 Kalydeco (CF treatment)

**Patents:** 7

**Big Pharma experience:**
Prior to Vertex and during his service of 20 years at Merck & Co, he led clinical pharmacology and oversaw clinical development and regulatory strategy. His multiple positions included Vice President of Clinical Research - Infectious Diseases & Vaccines, Vice President of Clinical Pharmacology/Early Stage Development, Senior Vice President of Late Stage Development and Senior Vice President of Global Scientific Strategy (Infectious Diseases, Respiratory/Immunology).

Mark Sumeray
CMO - Aegerion Pharmaceuticals

**Background:** Clinical development

**Key traits:** Medical affairs, cardiovascular therapeutic expertise, big pharma experience

**Education:** MD - UCL, FRCS Surgery – Royal College of Surgeons

Mark has been CMO at Aegerion since 2011, he is leading the development and commercialisation of novel therapies for patients with debilitating rare diseases worldwide, such as Homozygous Familial Hypercholesterolaemia. He heads medical affairs through the approval and post-approval processes for the company’s pipeline.

**Orphan achievement (for the period of 2012-2014):**

**US:** Orphan Marketing Approval: 21/12/12 – Juxtapid (HoFH)

**EU:** Orphan Marketing Approval: 01/08/13 – Lojuxta (HoFH)

**Academic experience:**
No. of publications: 12

**Big Pharma experience:**
Prior to Aegerion, Mark served as VP Cardiovascular/Metabolics at Bristol-Myers Squibb. He also held various roles at The Medicines Company, such as VP and Business Unit Co-Leader, VP Medical Business Development, VP Clinical Development and Head of Medical Science. Earlier, he served as Worldwide VP of Clinical Development for Ethicon (franchise of Johnson & Johnson).
Martine Clozel
CSO - Actelion

**Background:** Research / clinical development

**Key traits:** Entrepreneur, scientific expertise in receptor biology, big pharma experience

**Education:** MD Nancy University; PhD in Physiology and Pharmacology McGill University, and the University of California, San Francisco.

Martine is the co-founder of Actelion. Since 1997 she served as a SVP and a Head of Drug Discovery Pharmacology and Preclinical Development. Since her appointment as the company’s CSO in 2009, Martine is a scientific expert heading Actelion’s drug discovery projects. She is also a member of the company’s management board.

**Orphan achievement (for the period of 2012-2014):**
- US: Orphan Designation: 11/9/13 - Procysbi (nephropathic cystinosis)
  Orphan Marketing Approval: 30/4/13 – Procysbi (cystinosis)
- EU: Orphan Designation: 27/9/14 – Procysbi (Huntington’s disease)
  Orphan Marketing Approval: 6/9/13 – Procysbi (cystinosis); 6/9/13 – Procysbi (Huntington’s disease)

**Academic experience:** No. of publications: 130

**Patents:** 69

**Big Pharma experience:**
Prior to co-founding Actelion, and during her 11-year career at Hoffmann-La Roche, she initiated the research on endothelin and endothelin receptor antagonists which led to the discovery and clinical development of bosentan and other molecules.

**Patrice Rioux
CMO - Raptor Pharmaceuticals (2009-2014)**

**Background:** Research / clinical

**Key traits:** Previous rare disease experience, broad big pharma experience, pharmacogenetics expertise

**Education:** MD, PhD Mathematical Statistics - Paris VI / Pitié-Salpétrière

Patrice held a Researcher role in Clinical Research and Epidemiology at INSERM

**Patents:** 3

**Big Pharma experience:**
As Medical Director at Biogen he led early to late stage clinical trials in autoimmune diseases.

**Biotech experience:**
Prior to that he served as the CMO of FerroKin Biosciences, an early-stage development company. Earlier, he served as a VP of Medical Research at as Repligen Corp, focusing on pharmacogenetics and pharmacogenomics across multiple therapeutic areas. Earlier, Patrice served as CMO and VP Clinical/Regulatory for Edison Pharmaceuticals, focusing on inherited and acquired energy impairment diseases.

**Academic experience:** No. of publications: 54

**Patents:** 3

**Big Pharma experience:**
As the CMO at Raptor Pharmaceuticals, Patrice led clinical operations, regulatory and medical affairs. He designed and conducted the phase III trial in orphan disease that led to an FDA approval of Procysbi.

**Orphan achievement (for the period of 2012-2014):**
- US: Orphan Designation: 11/9/13 - Procysbi (nephropathic cystinosis)
  Orphan Marketing Approval: 30/4/13 – Procysbi (cystinosis)
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Peter Mueller
CSO - Vertex Pharmaceuticals (July 2003 - October 2014)

Background: Academia
Key traits: Drug Discovery expertise, big pharma experience, development

Education: PhD Chemistry - Albert Einstein University of Ulm; fellowship in Quantum Pharmacology - Oxford University; fellowship in Biophysics - Rochester University

Peter was the CSO at Vertex Pharmaceuticals, where he oversaw global drug discovery and development programs. He led Vertex to the successful approval and launch of Inivek (Hepatitis C), and an approval for Kalydeco (CF) in the US, EU, Canada and Australia.

Orphan achievement (for the period of 2012-2014):
EU: Orphan Designation: 7/4/14 - VX-661 (investigational CF drug); 22/8/14 - Lumacaftor/ivacaftor VX – 809 (investigational combined CF treatment) Orphan Marketing Approval: 23/7/12 Kalydeco (CF treatment)

Academic experience:
No. of publications: 38
Peter holds a Professorship in Theoretical Organic Chemistry at the Albert Einstein University of Ulm

Big Pharma experience:
Previously, as the SVP R&D at Boehringer Ingelheim Pharmaceuticals, Peter was responsible for Research in North America and the development of all drug candidates of the company’s global portfolio in multiple therapeutic areas. During his tenure at Boehringer Ingelheim, he led the discovery of multiple development therapies and served in several research and medicinal chemistry positions.

Robert Kauffman
CMO - Vertex Pharmaceuticals (2009 - 2014)

Background: Clinical development
Key traits: Therapeutic area expertise in immunology, pharma & biotech experience, regulatory

Education: MD, PhD in Virology, Microbiology - University of Pennsylvania

Robert held multiple senior roles in clinical development during his career of over 15 years at Vertex. He had served as the company’s CMO from 2009 until 2014. He led the clinical development and won the approvals of Incivek for treatment of Hepatitis C and Kalydeco for treatment of Cystic Fibrosis.

Orphan achievement (for the period of 2012-2014):
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Academic experience:
No. of publications: 25

Patents: 12

Big Pharma:
Earlier, as a Medical Director of Serono Laboratories, he oversaw the clinical development of metabolic and immune therapies.

Biotech experience:
Prior to Vertex, he served as VP of Clinical Affairs at BioTransplant, where he developed a system for induction of immune tolerance in solid organ transplantation. As Executive Director, Medical Research at Syntex Research (acquired by Roche), he led the international clinical development team for studies for the prevention of rejection of kidney transplants.

Academic experience:
No. of publications: 25

Patents: 12

Big Pharma:
Earlier, as a Medical Director of Serono Laboratories, he oversaw the clinical development of metabolic and immune therapies.

Biotech experience:
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Robert Spiegel  
CMO - PTC Therapeutics  

**Background:** Clinical development  

**Key traits:** Big Pharma & biotech, multiple therapeutic expertise, strategic acumen  

**Education:** MD - University of Pennsylvania  

Since Robert has joined PTC Therapeutics in 2011 as the CMO (part-time April 2011 to January 2014), the company has won EU marketing approval for its inaugural orphan drug candidate.  

**Orphan achievement (for the period of 2012-2014):**  
**US:** Orphan Designation: 10/12/14 – Translarna (BMD)  
**EU:** Orphan Designation: 4/7/14 – Translarna (BMD)  

**Orphan Marketing Approval:**  
Schering-Plough’s multiple executive committees holding responsibility for research projects and licensing activities.  

**Patents:** 4  

**Big pharma experience:**  
Prior to joining PTC, he served as CMO at Schering-Plough, where throughout his career he won over 30 successful new drug applications. He also served on the Schering-Plough’s multiple executive committees holding responsibility for research projects and licensing activities.  

**Biotech experience:**  
Robert serves on the board of directors of Geron Corporation, Sucampo Pharmaceuticals and Edge Therapeutics. His previous board and scientific advisory committee assignments include Clavis Pharma and Capstone Therapeutics.  

**Academic experience:**  
No. of publications: 75  

Robert holds an Associate Professor position at Weill Cornell Medical Center.  

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Todd Zankel  
CSO - Raptor Pharmaceuticals  

**Background:** Research / academic  

**Key traits:** Drug discovery, scientific expertise in receptor biology, entrepreneur  

**Education:** PhD in Chemistry Columbia University  

Todd is co-founded Raptor Pharmaceuticals. As the CSO, he leads discovery of innovative drugs, production methods and drug delivery platforms. He is overseeing research and development of novel drugs and drug-targeting platforms derived from the human receptor-associated protein (RAP), focusing on neurodegenerative diseases, genetic diseases and cancer.  

**Orphan achievement (for the period of 2012-2014):**  
**US:**  
**Orphan Designation:** 11/9/13 - Procysbi (nephropathic cystinosis)  
**Orphan Marketing Approval:** 30/4/13 – Procysbi (cystinosis)  

**EU:**  
**Orphan Designation:** 27/9/14 – Procysbi (Huntington’s disease)  
**Orphan Marketing Approval:** 6/9/13 – Procysbi (cystinosis); 6/9/13 – Procysbi (Huntington’s disease)  

**Academic experience:**  
No. of publications: 5  

National Institutes of Health postdoctoral fellowship at the Plant Gene Expression Center; postdoctoral fellowship at the Swiss Institute of Technology.  

**Patents:** 12  

**Biotech experience:**  
He served as a Senior Director of Research at BioMarin, leading the development of manufacturing cell lines, novel therapeutics, drug delivery technologies and supporting the regulatory group.