

# Cystic Fibrosis: KOL Insight [2017]

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## Abstracts

How will the novel CFTR-function restoring therapies impact the CF landscape?

The arrival of Vertex's Kalydeco and Orkambi was a major advance in the disease-modifying CF therapy. However, despite their comparative success, patients' progress and demand persists for improved modalities that can restore the CFTR function in all CF genotypes. Vertex's tezacaftor + ivacaftor and Galapagos NV/AbbVie's GLPG 1837 form part of a rich pipeline, but which candidates stand out to key opinion leaders (KOLs)? Which products and combinations are likely to succeed? And will they improve survival and help patients live beyond their current life expectancy of ~40 years?

Learn how KOLs see the CF market evolving in KOL Insight: Cystic Fibrosis (CF).

12 US and European KOLs provide candid insights on 4 marketed and 12 emerging therapies targeting CFTR function and symptomatic aspects of the CF disease.

Take a tour of the report now:

The table of contents

The key business questions answered

The key KOL quotes

See the 15 therapies covered

Find out who the 6 EU & 6 US KOLs are

Review an extract from the report - 1 drug profile

## Top Takeaways

Can Vertex' Kalydeco (ivacaftor) maintain its leading position in the CF market? Galapagos NV and AbbVie are developing GLPG 1837, a potentiator which also targets F508del and G551D? Find out how GLPG 1837 and other competitors stack up to ivacaftor?

Will Orkambi (ivacaftor/lumacaftor) gain wider acceptance in the market? There is considerable pushback from payers and insurance programmes on the pricing of CF therapies. What do KOLs think about the drug's prospects and what factors can its uptake in CF patients?

Is there a future for PTC Therapeutics' Translarna? The drug is already approved for to treat patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) but will the drug reach the CF market?

Will Vertex's follow-up small molecule tezacaftor replace lumacaftor in Orkambi in the near future? How do KOLs compare its safety and efficacy and will patients have adequate access to the tezacaftor + ivacaftor combination?

Triple combination therapy with CFTR modulators is on the horizon. What impact are they likely to have and which ones will succeed? Vertex's is leading the race with tezacaftor and its next-generation (VX-152 and VX-440) corrector molecules with Galapagos/AbbVie's hot on its heels with its CFTR modulators (GLPG1837 and GLPG2222). Find out how KOLs weigh up these new drug options.

Will Genentech/Roche's Pulmozyme remain ingrained in the CF treatment pathway? Or is it likely to be superseded by emerging candidates such as AIR DNase (alidornase alfa; Protalix BioTherapeutics), inhaled OligoG (AlgiPharma) and Parion/Vertex's VX-371.

How do KOLs view the development of anti-inflammatory drugs for CF? There are no approved drugs for CF in this class but what concerns do these experts have about drugs targeting leukotriene A4 hydrolase (LTA4H) and cannabinoid receptor CB2 pathways? How do they see their long-term potential?

## Quotes

“It is very rare that you see a treatment in any condition, particularly in respiratory medicine, where there is a very clear ‘treatment makes you better, stopping it makes you worse’. You know, utterly transformative in the way that ivacaftor was for those patients.”

## EU Key Opinion Leader

## Sample of therapies covered

## Marketed Therapies

Kalydeco (ivacaftor; Vertex)

Orkambi (lumacaftor/ivacaftor; Vertex)

Pipeline Therapies

Riociguat (Bayer)

GLPG1837 (Galapagos/AbbVie)

Translarna (ataluren; PTC Therapeutics)

Tezacaftor (VX-661) + ivacaftor (Vertex)

VX-371 (formerly P-1037; Parion Sciences/Vertex)

Acebilustat (CTX-4430; Celtaxsys/CFF Therapeutics)

Plus 5 more - download the full list now

## Sample of KOLs interviewed

## KOLs from North America

Dr Patrick Flume, MD, Powers-Huggins Endowed Chair for Cystic Fibrosis,

Professor of Medicine and Pediatrics, Medical University of South Carolina, Charleston, SC, USA.

Dr Michael W. Konstan, MD, Vice Dean for Translational Research, Professor of Pediatrics at Case Western Reserve University School of Medicine, Cleveland, OH, USA.

Dr Richard B. Moss, MD, Emeritus Professor of Pediatrics and former chief of the Pediatric Pulmonary and Allergy Divisions, Stanford University, Palo Alto, CA, USA.

#### KOLs from Europe

Dr Carla Colombo, MD, Professor of Paediatrics, Department of Paediatrics, University of Milan, Italy.

Professor Stuart Elborn, MD, FRCP, CBE, Former president of the European Cystic Fibrosis Society. Professor of Respiratory Medicine and Dean of the School of Medicine, Dentistry and Biomedical Sciences at Queen's University, Belfast, UK.

Dr Harry G.M. Heijerman, MD, Director of the Adult CF Centre, Hague Teaching Hospital, The Hague, Netherlands.

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