

## Cystic Fibrosis: Update Bulletin #3

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### Abstracts

This edition presents world leading key opinion leader (KOL) views on recent developments in the cystic fibrosis (CF) space. Topics covered include; expert opinions on Vertex Pharmaceuticals' regulatory submission to the FDA and EMA for tezacaftor/ivacaftor, a second-in-class cystic fibrosis transmembrane conductance regulator (CFTR), which has been granted Priority Review status in the US; ProQR Therapeutics' publication of positive preliminary results from a Phase 1b study assessing QR-010, a novel investigational RNA therapeutic with Orphan Drug status in the EU and US and Fast Track review status granted by the FDA; and Savara's initiation of a Phase III study with AeroVanc (inhaled vancomycin powder) for the treatment of persistent methicillin-resistant *Staphylococcus aureus* (MRSA), which is an increasing problem amongst CF patients.

#### Business Questions:

How do KOLs perceive Boehringer Ingelheim's adalimumab biosimilar, Cyltezo?

With three adalimumab biosimilars now approved in Europe, what concerns do prescribers have with regard to multiple biosimilar versions becoming available?

In August 2017, Vertex Pharmaceuticals reported that the FDA and the EMA had accepted the regulatory submission for tezacaftor/ivacaftor, but how do KOLs perceive the company's next generation cystic fibrosis transmembrane conductance regulator (CFTR)?

How does tezacaftor/ivacaftor compare with its earlier launched competitor Orkambi (lumacaftor/ivacaftor)?

What impact will tezacaftor/ivacaftor have on the current treatment paradigm? Which subgroups of CF patients will be considered eligible candidates for this premium priced targeted therapy?

What barriers to market entry exist for Vertex's tezacaftor/ivacaftor?

How promising is ProQR's first-in-class investigational RNA therapeutic QR-010?

What is the potential patient population for this highly targeted therapy? How will it compete with the CFTR therapies?

Do KOL's consider QR-010's inhaled route of administration to be a potential issue in the treatment of CF?

Savara announced progression of its inhaled vancomycin powder, AeroVanc, to Phase III development for the treatment of persistent methicillin-resistant *Staphylococcus aureus* (MRSA), but how positive are KOLs with regard to this product given that it's available generically in other formulations?

How significant is the issue of MRSA amongst CF patients? How will AeroVanc sit within the current treatment paradigm and what products, if any, will it displace?

What factors, if any, will impact AeroVanc's uptake for the intended indication?

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