Efficacy of elexacaftor/tezacaftor/ivacaftor in patients with cystic fibrosis and advanced lung disease

Kate M. O’Shea1,3, Orla M. O’Carroll2,3, Catherine Carroll2, Brenda Grogan1,2, Anna Connolly2, Lynda O’Shaughnessy2, Trevor T. Nicholson2, Charles G. Gallagher1,2 and Edward F. McKone1,2

Affiliations: 1School of Medicine, University College Dublin, Dublin, Ireland. 2Dept of Respiratory Medicine, St Vincent’s University Hospital, Dublin, Ireland. 3Equal contributions.

Correspondence: Edward F. McKone, Dept of Respiratory Medicine, St. Vincent’s University Hospital, Elm Park, Dublin 4, Ireland. E-mail: emckone@svhg.ie

@ERSpublications
Elexacaftor/tezacaftor/ivacaftor is an effective treatment and is well tolerated in cystic fibrosis and advanced lung disease https://bit.ly/30Q87p3


This single-page version can be shared freely online.

To the Editor:

Cystic fibrosis (CF) is an autosomal recessive disorder which results from mutations in the cystic fibrosis transmembrane regulator (CFTR) gene encoding the CFTR protein. Defects in the production or function of this protein result in multiorgan dysfunction via altered conductance of chloride and bicarbonate across epithelial cell surfaces. Traditionally, the treatment of CF has focused on management of downstream organ dysfunction and symptoms caused by reduced function of this protein. However, with the recent advent of CFTR modulators, this treatment strategy has changed to targeting the primary cause of CF. CFTR modulators are a class of drugs which act to improve production, processing and function of the defective CFTR protein. Since the US Food and Drug Administration (FDA) and European Medicines Agency approved the use of ivacaftor in 2012, followed by the approval of lumacaftor/ivacaftor and tezacaftor/ivacaftor, CFTR protein modulation has revolutionised the management of CF, and has allowed the disease to be treated at a molecular level [1, 2].

Copyright ©ERS 2021

Link to published version: https://doi.org/10.1183/13993003.03079-2020