

## Cystic Fibrosis [2017]: Bulletin #2

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### Abstracts

This update bulletin edition presents US and EU key opinion leader (KOL) views on recent developments in the cystic fibrosis (CF) space. Topics covered include; Anthera Pharmaceuticals announcing that it has commenced screening in Europe in the Phase III non-inferiority RESULT study comparing the pancreatic enzyme replacement therapy (PERT) Sollpura to Pancreaze for exocrine pancreatic insufficiency (EPI) due to cystic fibrosis (CF); Protalix BioTherapeutics' announcing new positive Phase II trial results for Protalix BioTherapeutics' AIR DNase (alidornase alfa; PRX-110) which were presented at the 40th European Cystic Fibrosis Society Conference in Seville, Spain in June 2017; and Verona Pharma initiating a Phase II pharmacokinetic trial in the US for RPL554, the company's a first-in-class, inhaled, long-acting, mixed phosphodiesterase (PDE) 3/4 inhibitor, following the acceptance of an Investigational New Drug application (IND) by the US FDA.

#### Business Questions:

How do KOLs rate the potential for an additional pancreatic enzyme replacement therapy in CF?

What are KOLs' opinions on the upcoming Phase III result study of Sollpura?

If approved, what are the chances that Sollpura will be successful at gaining market share?

What will AIR DNase need to show in order to be considered as a competitor to Pulmozyme?

When could AIR DNase make it to market and what will it need to show in order to get there?

What are KOLs' thoughts on the development of RPL554 and to what extent do they consider it to be a viable product in CF?

What safety concerns do KOLs have about RPL554?

If approved, how would RPL554 be incorporated into the treatment paradigm for CF?

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