PTC124 phase 3 results

The Phase 3 trial (NCT00803205) "Efficacy and safety study of ataluren (PTC 124) as an oral treatment for nonsense-mutation-mediated cystic fibrosis" was conducted in 36 sites across 11 countries (EU, Israel and the United States). Two hundred and thirty-eight subjects (≥ 6 years-old) bearing at least one nonsense CFTR mutation were enrolled to evaluate the safety and efficacy of ataluren. Subjects received either 3 times daily ataluren (10 mg/kg morning and midday and 20 mg/kg evening) or placebo during the 48-week study. The results showed a positive trend in the lung function as measured by FEV_1 (primary endpoint) and in the rate of pulmonary exacerbations (secondary endpoint) favoring ataluren versus placebo. However, none of the endpoints reached statistical significance. A statistical significant effect was seen between treatment and use of inhaled antibiotics at baseline (mainly inhaled aminoglycosides). A post-hoc analysis showed a statistical significant treatment effect in patients not receiving chronic inhaled antibiotics, both on change in %-predicted FEV_1 and on pulmonary exacerbation rate. Safety results indicated that ataluren was generally well tolerated. The overall incidence of adverse events was similar in both groups. Some patients experienced creatinine elevations that occurred in connection with concomitant treatment with systemic aminoglycosides.

6 ECFS-CTN sites in Belgium, France, Germany, Italy, Sweden and The Netherlands participated in this study and enrolled 83 of the 238 patients.

The summary of data provided here is from a sponsor press release (June 8, 2012) and from data presented at the European Cystic Fibrosis Society Conference (2012).