Title	***12. Open-Label, Phase 3 Trial to Evaluate the Safety of Aztreonam 75 mg Powder and Solvent for Nebuliser Solution/Aztreonam for Inhalation Solution (AZLI) in Children with Cystic Fibrosis (CF) and Chronic Pseudomonas aeruginosa (PA) in the Lower Airways (PALS)***
Project Coordinator	C. Braggion, MD (c.braggion@meyer.it)
Internal Collaborators	R. Pasotto, study coordinator
Study design	Phase III, multicentric study, which involve different countries in Europe and include 5 Centres in Italy (Eudract no. 2011-001362-18).
Grant by	Gilead Sciences Inc.
Background and aims	Gilead Sciences, Inc. (Gilead) has developed aztreonam 75 mg powder and solvent for nebuliser solution/aztreonam for inhalation solution (AZLI), a lyophilized formulation of the monobactam antibiotic aztreonam, for the treatment of CF patients with PA infection. The EU approved indication is for the suppressive therapy of chronic pulmonary infections due to Pseudomonas aeruginosa (PA) in patients with CF aged 18 years and older. The aim is to evaluate safety of treatment with AZLI 75 mg 3 times daily (TID) for 3 courses of therapy (28 days on/ 28 days off) in female and male children less than 13 years of age with CF and chronic PA infection/colonization.
Inclusion criteria	Male or female patients with CF, less than 13 years of age, documented positive lower respiratory tract culture for PA at the screening visit plus 2 documented positive lower respiratory tract cultures for PA within 12 months prior to study entry (start of treatment); clinical stability with no evidence of significant respiratory symptoms or, if obtained for clinical evaluation, no chest radiograph findings at screening that would require administration of IV antipseudomonal antibiotics, oxygen supplementation, or hospitalization.
Exclusion criteria	Known hypersensitivity to adverse reaction to aztreonam, or beta-agonists; use of IV or inhaled antipseudomonal antibiotics within 14 days of study entry (start of treatment); use of any investigational drug within 30 days of study entry (start of treatment; presence of a condition or abnormality that would compromise the patient's safety or the quality of study data, in the opinion of the investigator.
Methods	This is an open-label, multi-center study in pediatric patients age less than 13 years with CF and chronic PA pulmonary colonization/infection. Eligible subjects will receive study treatment as 3 intermittent 28-day courses of AZLI 75 mg TID administered via the Investigational eFlow Nebulizer System, each followed by 28 days off AZLI. The study schedule will consist of 8 visits: Screening, Baseline (Day 1), Day 28, Day 56, Day 84, Day 112, Day 140 and Day 168. The total study period will be 28 weeks.
Expected results and anticipated output	To evaluate safety of treatment with AZLI 75 mg 3 times daily (TID) for 3 courses of therapy (28 days on/28 days off) in female and male children less than 13 years of age with CF and chronic PA infection/colonization.
Start of recruitment	For our Centre at March-April 2012.
End of experimental plan	For our Centre at March 2013.
Pubblication on medical Journal	2-3 paediatric patients (age less than 13 years) will be enrolled.