

## **Elenco degli studi in corso al 7/12/2023**

- ELX/TEZ/IVA Managed Access Program for Patients 2 Through 5 Years of Age With Cystic Fibrosis Who Have At Least One F508del Mutation in the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Gene (VX22-445-908)
- Compassionate use for ataluren therapy in patients 6 years of age and older with Shwachman-Diamond Syndrome (SDS) diagnosis carrying the c183-184ta>ct mutation in at least one allele (CRCFC-SDSAUT48)
- Studio osservazionale sulla valutazione di pazienti affetti da fibrosi cistica con prescrizione off-label di Kaftrio + Kalydeco in mutazioni non approvate da AIFA
- A Phase 3, Open-label Study Evaluating the Longterm Safety and Efficacy of VX-121 Combination Therapy in Subjects With Cystic Fibrosis (VX20-121-104)   
abel Study Evaluating the Longterm Safety and Efficacy of VX-121 Combination Therapy in Subjects With Cystic Fibrosis (VX20-121-104)
- A Phase 3 Open-label Study Evaluating the Longterm Safety and Efficacy of Elexacaftor/Tezacaftor/Ivacaftor in Cystic Fibrosis Subjects With Non-F508del CFTR Genotypes (VX21-445-125)
- Real World Evidence of Health Care Resource Utilization in patients with CF with at least one F508del mutation treated with ivacaftor/tezacaftor/elexacaftor + ivacaftor in Italy: a non-interventional, retrospective, observational study (HEOR-22-101-051)
- A Longitudinal Study on the Impact of ELX/TEZ/IVA Treatment in Patients With Cystic Fibrosis in the Real World (TRAJECTORY)
- La tripla combinazione di modulatori della proteina CFTR in eterozigoti F508del con una mutazione a funzione minima nel secondo allele: studio retrospettivo multicentrico in fibrosi cistica e malattia polmonare severa (SITTMA)
- Clinical follow-up of CF patients carrying a residual function mutation: a five years cohort study