R&D Trends: Cystic Fibrosis - New drugs tackle both cause and effect

Description: Two decades after the identification of the cystic fibrosis transmembrane conductance regulator protein, approved drugs capable of correcting dysfunctions in this protein are nearing reality. This report captures the thoughts of cystic fibrosis specialists on this exciting development, and also highlights potentially life-extending innovations among inhaled antibiotics.

Features and benefits

- Features a multi-parameter assessment of the cystic fibrosis pipeline from preclinical to preregistration.
- Identifies the most promising innovative new candidates within the pipeline.
- Analyzes the changes to the cystic fibrosis research portfolio over the past 2.5 years.
- Discusses the key learnings from candidates recently discontinued from late-stage cystic fibrosis clinical trials.
- In-depth interviews with four key opinion leaders reveal strong views on unmet needs and target product profiles.

Highlights

- There remain significant unmet needs among cystic fibrosis (CF) patients, but commitment to the discovery of new therapies is growing. The pharmaceutical industry and CF organizations have increased their efforts significantly. Over the past 2.5 years the CF pipeline has grown by 11%, and the number of clinical trials active/recruiting has doubled.
- Key opinion leaders interviewed by Datamonitor were unanimous in their praise for the great strides made by protein repair treatments. Courtesy of work done by Cystic Fibrosis Foundation Therapeutics, PTC Therapeutics and Vertex, there is now precedent for small molecule-mediated protein repair across the most common three classes of CF mutation.
- An arms race is emerging between increasingly resilient lung bacterial infections and the next generation of inhaled antibiotics. CF specialists are looking to powerful broad-spectrum agents and/or biofilm-penetrating liposomal formulations to counter the threat posed by the rising incidence of aminoglycoside-resistant pathogens.

Your key questions answered

- What are the major unmet needs among cystic fibrosis patients, and how is the development pipeline evolving to address these?
- Which pipeline candidates are expected to have the biggest impact on tackling the underlying cause of cystic fibrosis?
- How will the next generation of inhaled antibiotics counter the threat posed by the rising incidence of aminoglycoside-resistant pathogens?
- Which areas of research are being championed by leading cystic fibrosis specialists?

Contents:

Executive Summary
- Strategic scoping and focus
- Datamonitor key findings

OVERVIEW
- Catalyst
- Summary

CLINICAL PIPELINE OVERVIEW
- Despite increased life expectancy there remains an urgent need for improved cystic fibrosis treatments
- Pipeline is growing but is still dominated by early-stage candidates
- Scant options currently exist for treating cystic fibrosis
- Azithromycin
- Cayston
- Pulmozyme
- TOBI
Full pipeline demonstrates high project turnover and mechanistic diversity
Few companies have more than one cystic fibrosis therapy in their portfolio
Cystic fibrosis research is narrowing its focus onto preferred therapeutic classes
  More emphasis is on symptomatic therapies
  Protein repair treatment leads the way among the disease-modifying approaches
Late-stage candidates promise wider treatment options in the near future
  TOBI Podhaler (tobramycin inhalation powder; Novartis)
  Bronchitol (mannitol; Pharmaxis)
  Colobreathe (colistin; Forest Laboratories)
  Kalydeco (ivacaftor, VX-770; Vertex/CFFT)
  Solpura (Trizytek, liprotamase; Eli Lilly/CFFT)
  Aeroquin (levofloxacin, MP-376; Aptalis Pharma/PARI)
  Arikace (liposomal amikacin; Insmed/CFFT/PARI)
  Ataluren (PTC124; PTC Therapeutics/CFFT)
  Spiriva (tiotropium bromide; Boehringer Ingelheim)
  T100 (tobramycin in eFlow nebulizer; PARI)
Late-stage development compounds recently discontinued
  Denufosol tetrasodium
  AZD-
  Cobiprostone
  Exinalda
  GS

TARGET PRODUCT PROFILE
  TOBI continues to form the basis of gold-standard combination regimens
  Target product profile versus current level of attainment with TOBI-based combinations reveals significant unmet needs
  Efficacy
  Safety
  Formulation
  Cost

CLINICAL TRIAL DESIGN IN CYSTIC FIBROSIS
  Recent trends among cystic fibrosis clinical trials
    Trials routinely use extra endpoints to supplement FEV
    Clinical testing of new candidates is getting progressively harder
    Trials need to be large and are taking longer
    Modified endpoints are emerging for pediatric studies
  Design of a typical cystic fibrosis clinical trial
  Future developments in cystic fibrosis clinical trials
    More pooling of resources between countries
    Wider use of alternative endpoints

INNOVATIVE EARLY-STAGE APPROACHES
  Ranking of key innovative therapeutic approaches in cystic fibrosis
    Class II CFTR mutation correctors
    Complementary antibiotics combined in a single product
    DPI formulation of a broad-spectrum synthetic antibiotic
    Liposomal antibiotics

THE FUTURE OF TREATMENT IN CYSTIC FIBROSIS
  Key findings
    More clinical trials on the horizon to identify best drug combinations
    Options for protein repair treatments are likely to increase
    Animal models of cystic fibrosis will accelerate research
    New mechanisms for treating/correcting cystic fibrosis will continue to emerge

BIBLIOGRAPHY
  Journal papers
  Websites
  Datamonitor reports

APPENDIX
  Contributing experts
  Report methodology

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