Cincinnati Children's | Innovation Ventures

Pulmonology

Method of Treating Cystic Fibrosis with Src Kinase Inhibitors

Brief Description of Technology

Scientists have shown that src kinase inhibitors are effective at treating CF patients with a frame deletion of F508 in the CFTR gene.

TECHNOLOGY ID

2017-0203

BUSINESS OPPORTUNITY

Exclusive License or Sponsored Research

TECHNOLOGY TYPE

Therapeutic Target

PATENT INFORMATION

US Non-Provisional Filing

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Technology Overview

The most common mutation in CF patients is a frame deletion of phenylalanine 508 in the CFTR gene. Cincinnati Children's researchers have shown that src kinase inhibitiors, alone or in combination with existing treatments, are effective at treating CF patients with this deletion. In particular, PP2, a known src kinase inhibitor, resulted in better restoration of ion channel function in bronchial epithelia of CF patients than Orkambi. Further the combination of PP2 and Orkambi was synergistic. It is hypothesized that src kinase inhibitors produce anti-inflammatory and anti-infective properties giving them a dual-action benefit to patients. Significantly lower doses of the src kinase inhibitors were given in vivo to show efficacy indicating the possibility of identifying a therapeutic window below the MTC.

Applications

- CF therapy
- Repurposing
- Combination therapy

Advantages

- Better efficacy than current treatments
- Small molecule strategy
- Therapeutic and anti-infective dual action

Market Overview

There are nearly 2000 mutations in the CFTR gene that can cause CF, and the F508del mutation is found in ~70% of CF patients. The high occurrence of the F508del mutation in the CF population makes it an ideal drug target.

Investigator Overview

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