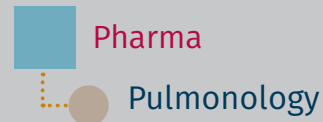


# Exploring a novel therapeutic target in cystic fibrosis



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## SUMMARY

Cystic Fibrosis (CF) is a life-limiting disease caused by mutations in the *CFTR* gene. Although highly effective *CFTR* modulators are emerging, ~10-15% of patients will not benefit from these therapies, while the high price of these drugs prevents access in many countries.

Hence, there is an unmet need to develop new therapies that can be applied to patients, independently of the underlying *CFTR* mutation. A strategy to achieve this, relies on restoring epithelial ion transport, bypassing *CFTR* dysfunction.

This project aims to validate drug candidates previously identified in a drug screen as potential modulators of an alternative ion transporter. These potential hits may path the way for a novel and inclusive therapy for CF.

## PROJECT ACHIEVEMENTS DURING & AFTER SPARK

- *in vitro* functional studies with selected hits
- Further analysis of mechanism of action

## LONG-TERM GOALS

- Show *in vivo* PoC
- Perform clinical testing
- License to Pharma