

TMEM16A inhibitors for Cystic Fibrosis Treatment

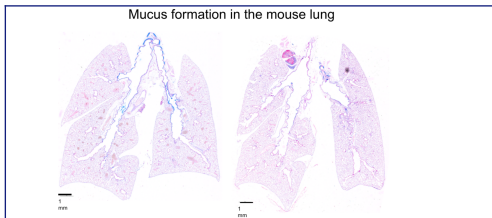
Cystic fibrosis (CF) is a life-threatening orphan disease affecting over 105,000 individuals globally, primarily presenting with severe respiratory complications due to excessive mucus production. Caused by mutations in the CFTR gene, the disease results in airway obstruction and chronic infections. Current therapeutic options, mainly CFTR modulators, show variable efficacy across patient populations, indicating a need for new therapeutic strategies.



Utilization of FDA-Approved Niclosamide for Mucus Regulation

TMEM16A is a calcium-activated chloride channel that is upregulated in cystic fibrosis, leading to excessive mucus production. Niclosamide, an FDA-approved drug, has been identified as a potent TMEM16A inhibitor. Preliminary research shows that niclosamide can reduce mucus secretion and may enhance respiratory function. It is suitable for both inhaled delivery, directly targeting the airways, and oral administration.

- 01 Targeted Mechanism: Modulates excessive mucus production via TMEM16A inhibition.
- 02 Established Safety: Utilizes the proven safety profile of the FDA-approved niclosamide.
- 03 Oral or inhalable Formulation: Designed for direct delivery to the lungs.
- 04 Research-Driven: Focused on addressing the underlying causes of



Asthma mouse lung before (left) and after (right) niclosamide treatment, showing reduced mucus hypersecretion. The asthma model serves as a suitable surrogate for CF, recapitulating TMEM16A-driven airway pathology absent in CFTR-knockout mice.

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CHALLENGE

Despite the success of CFTR modulators roughly 10% of CF patients carry rare or nonsense mutations that are not addressed by these genotype-specific drugs. Existing therapies target CFTR dysfunction but leave downstream pathology—excessive mucus secretion and chronic airway inflammation—only partially controlled. Because CF is caused by more than 2000 different mutations, a therapy from which all patients would benefit does not exist. An causal therapy would reduce these respiratory symptoms.

INNOVATION

Both can be achieved by using compounds that inhibit the calcium-activated chloride channel TMEM16A, which plays a crucial role in both airway smooth muscle contraction and mucus formation. Among such compounds, the FDA-approved and well-tolerated anthelmintic drug niclosamide and related substances have been shown to effectively reduce mucus formation in preclinical animal studies. Its established safety profile and existing regulatory approval could significantly shorten development timelines and reduce costs compared to novel CFTR modulators.

TRL 08

TRL 07

TRL 06

TRL 05

TRL 04

TRL 03

TRL 02

TRL 01

Technology Readiness Level (TRL)

01 Basic principles observed · 02 Technology concept formulated · 03 Experimental proof of concept · 04 Technology validated in lab · 05 Technology validated in relevant environment · 06 Technology demonstrated in relevant environment · 07 System prototype demonstrated in operational environment · 08 System complete and qualified



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