TMEM16A inhibitors as a treatment option for cystic fibrosis

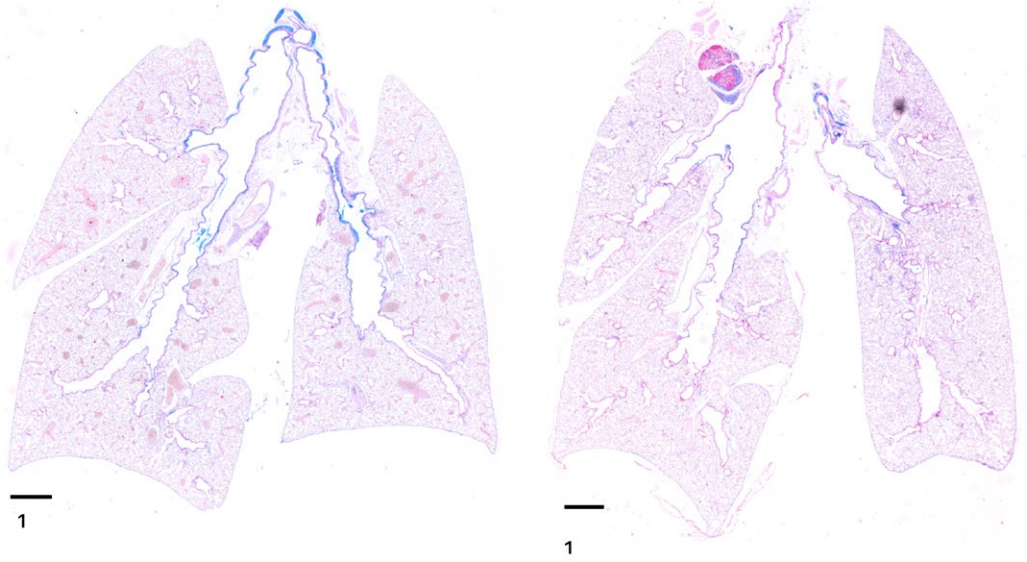
Reference No: B78025

CHALLENGE

It is estimated that more than 70 000 patients worldwide are suffering from cystic fibrosis (CF), a lethal genetic disorder characterized by a severe pulmonary disease caused by production a highly viscous and adherent mucus. Because CF is caused by more than 2000 different mutations, a therapy from which all patients would benefit does not exist. An ideal causal therapy for CF would reduce the excessive mucus formation as well as the airway contraction.

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 drug niclosamide and related substances have been shown to effectively reduce mucus formation in preclinical animal studies. The drugs may ideally be formulated for inhaled delivery with a spray or nebulizer.



Left side: Mucus formation (blue) in the asthmatic mouse lung

Right side: Niclosamide reduces mucus formation (blue) in the asthmatic mouse lung

COMMERCIAL OPPORTUNITIES

- TMEM16A inhibitors reduce axcessive mucus production as well as airway contraction
- Possible repurposing of FDA-proven and well-tolerated drugs
- Development of a drug formulation for inhalation (in form of a spray or nebulizer)

DEVELOPMENT STATUS

Efficacy proven *in-vivo* (mouse asthma model). Seeking partners for further development and licensing.

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