LONG TERM SINGLE DOSE GENE CORRECTION OF THE BIOELECTRICAL CFTR GENE DEFECT IN CYSTIC FIBROSIS MICE

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Introduction

The success of airway gene tran... bioelectrical defect in cystic fibro... not bee... peri... viral (LV) CF transmembrane conductance regulator (CFTR) gene transfer success via repeated nasal potential difference (PD) measures in individual CF mice over their lifetimes.

Methods

The nasal airway of anaesthetized CF... mice was instilled wit... prior to delivery of a LV-CFTR gene ... vector. A third group re... followed by an empty LV ... for control (LV-MT) ... measurem... & 21 months ... were delivered via the single lumen ... calculated from the low chloride response ...

Results

A continuous partial correction of the chloride ... receiving LPC and LV-CFTR and persisted for at least 12 months ... mean ΔPD after PBS pre-treatment or LV-MT treatment (Fig. 3.) was no different to untreated CF mice (n.s., RM ...). There was no correction of the sodium transport apparent at any time points (Fig 4., n.s. RM ANOVA).

Fig. 1a. Nasal TPD measurement

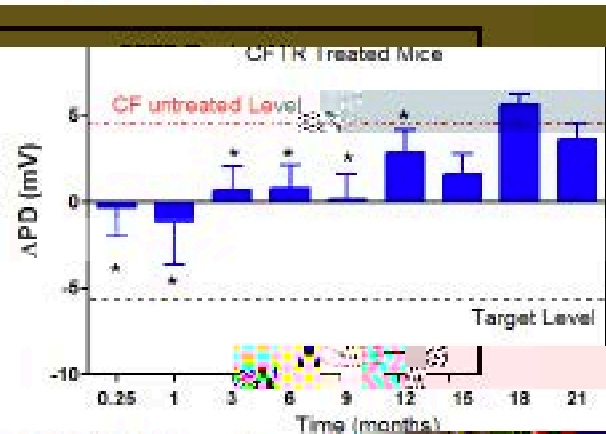

Fig. 2. Partial CFTR correction over ti...

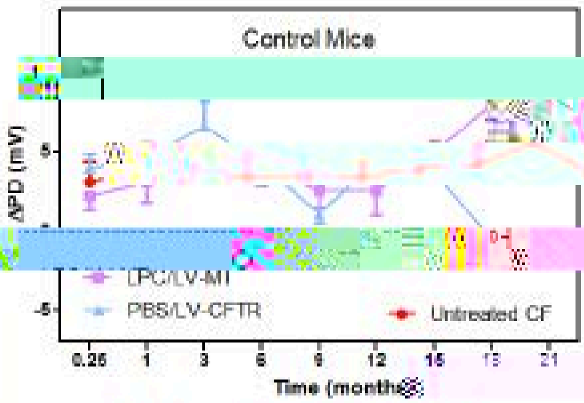

Fig. 3. Control groups over time, n=1-6.

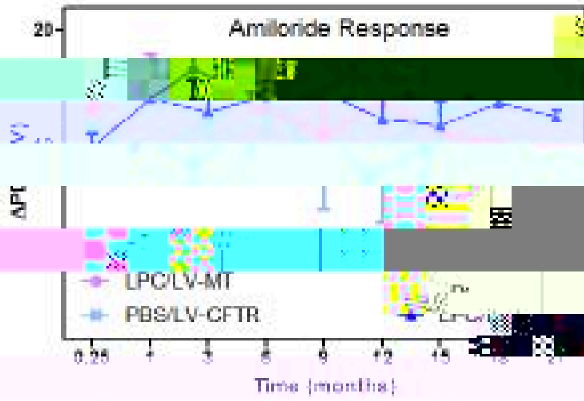

Fig. 4. Sodium Transport Response, n=1-12.

Conclusion

A sustained correction of airway CFTR function ... LPC/LV-CFTR delivery method. These findings ... dose gene transfer therapy for cystic fibr...

Acknowledgements