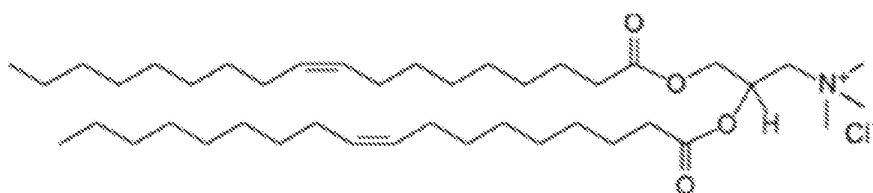
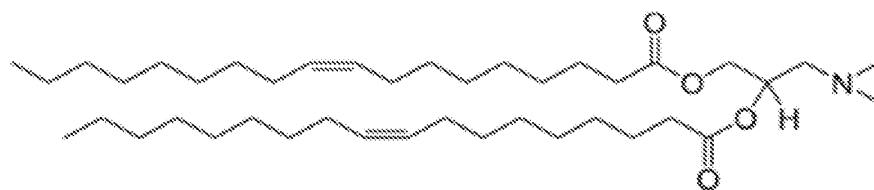


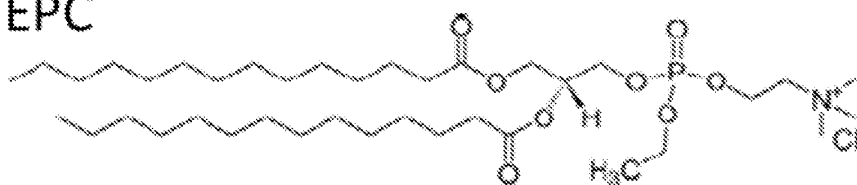
DOTAP

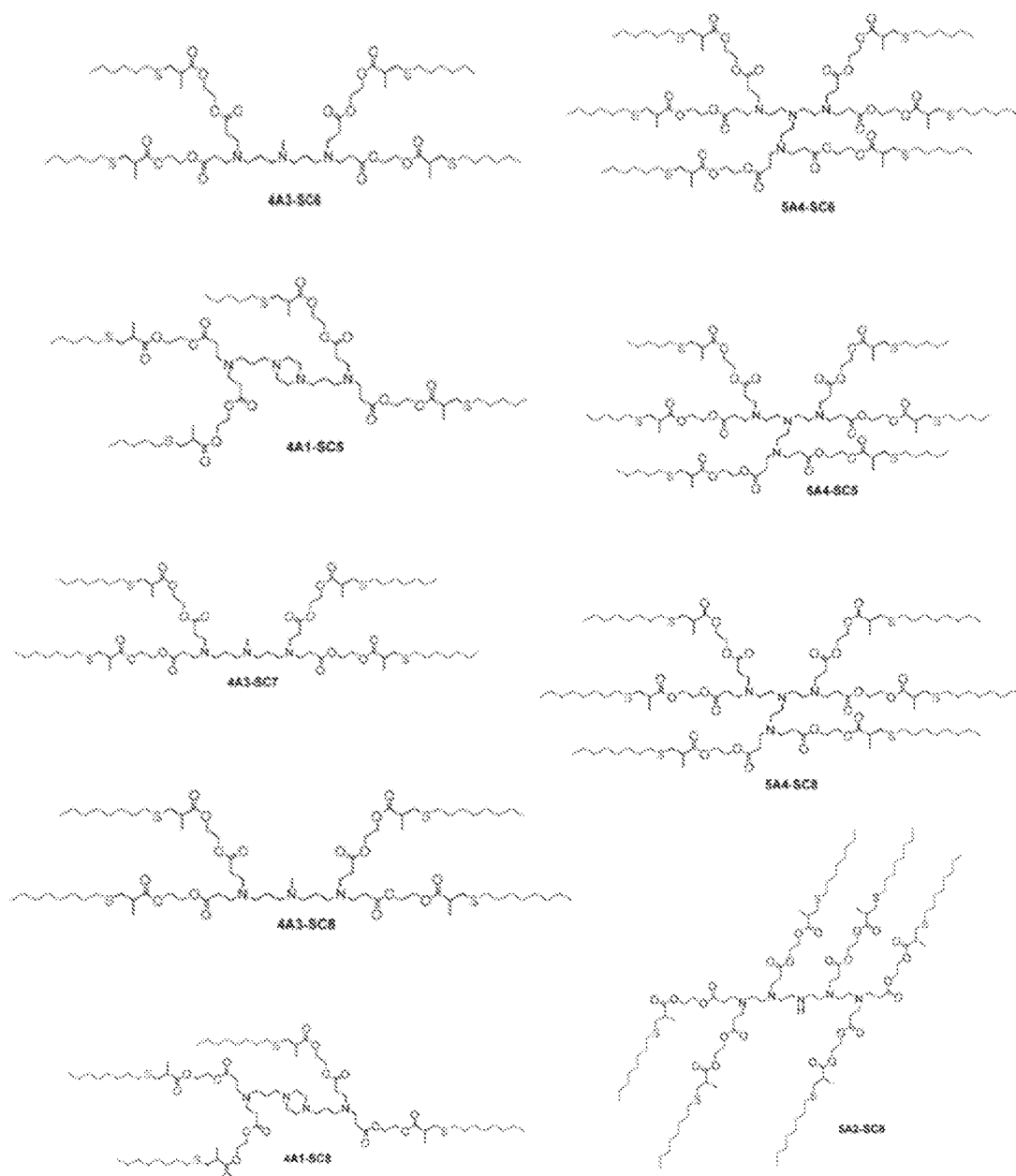


DODAP

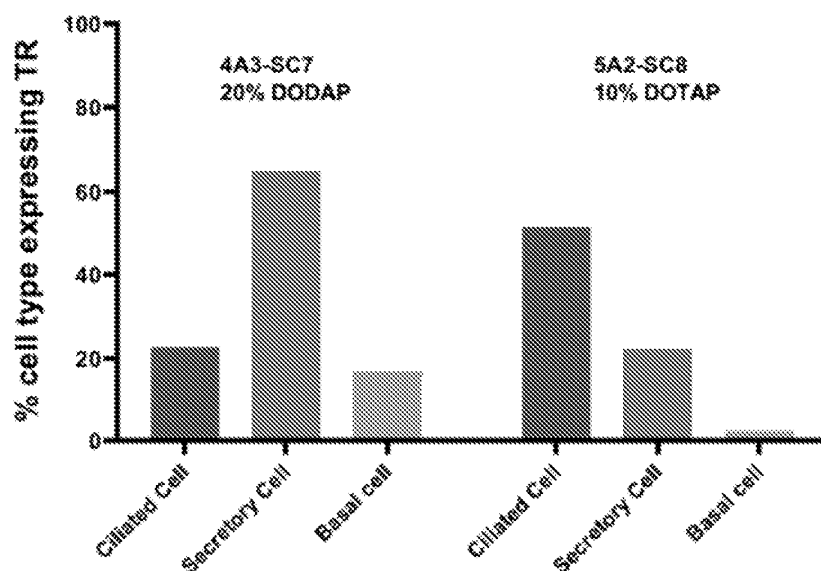


EPC

*Fig. 1*

**Fig. 2**

Apical bolus dosing of well-differentiated human bronchial epithelial cultures with TR mRNA (12 ug)



Aerosol delivery to well-differentiated human bronchial epithelial cultures with TR mRNA (4 ug)

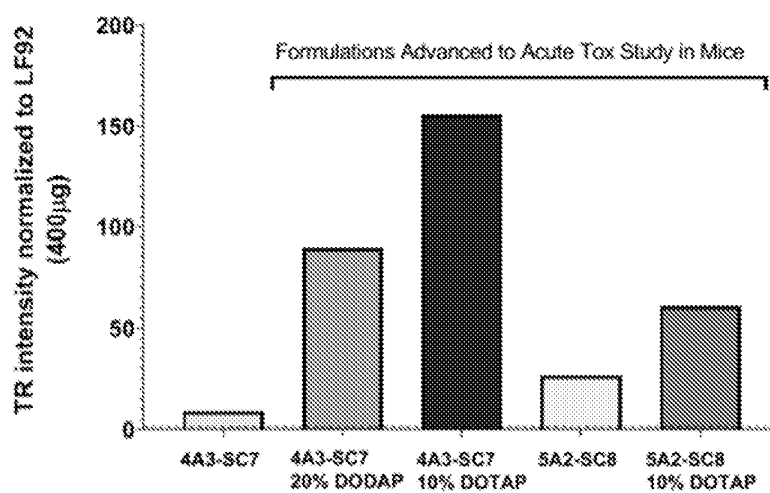
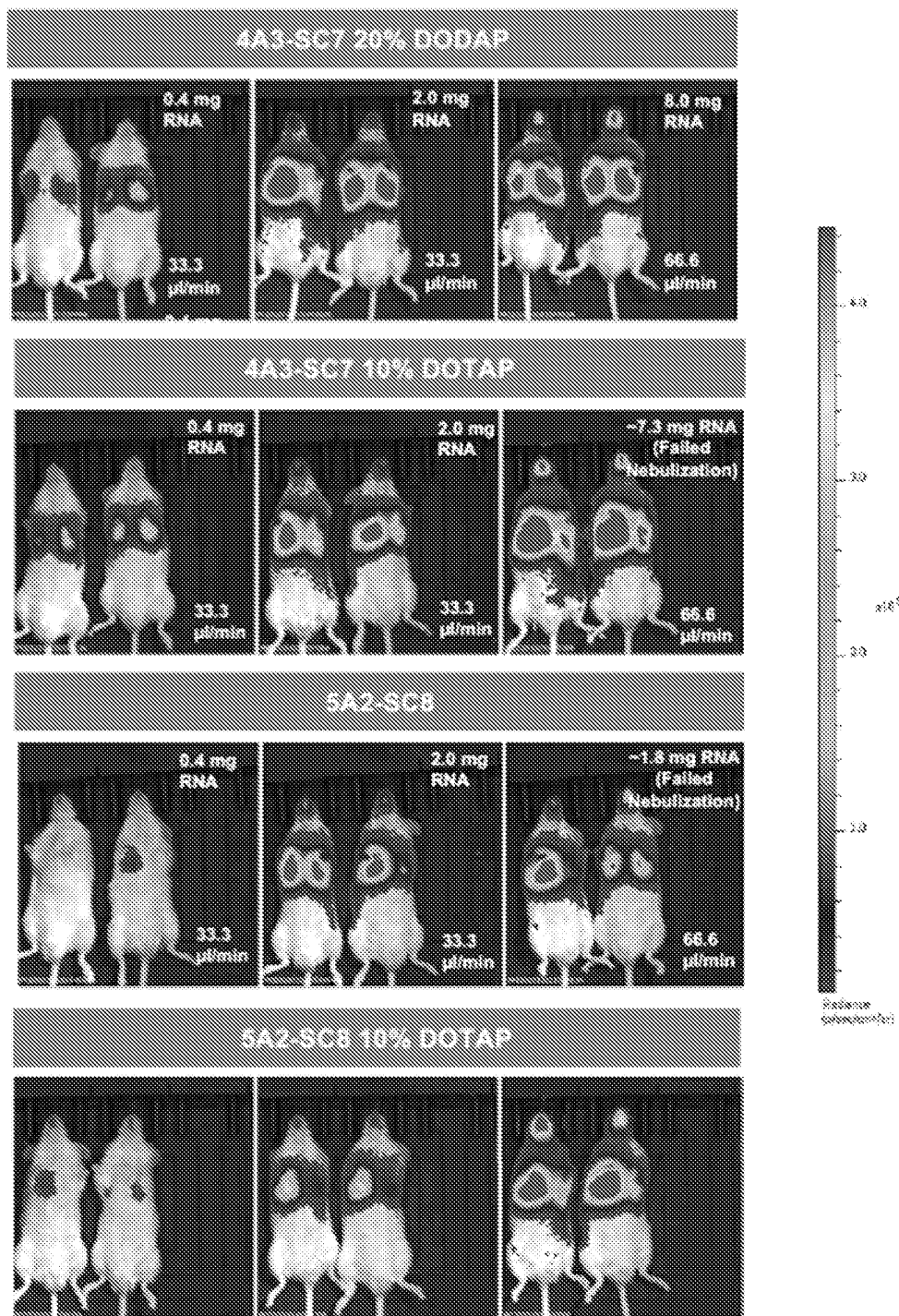


Fig. 3

*Fig. 4*

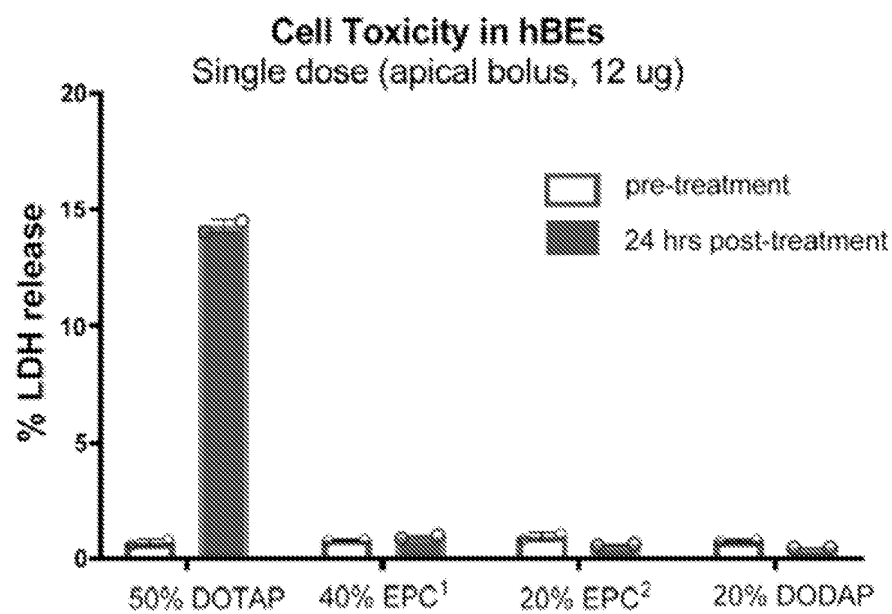
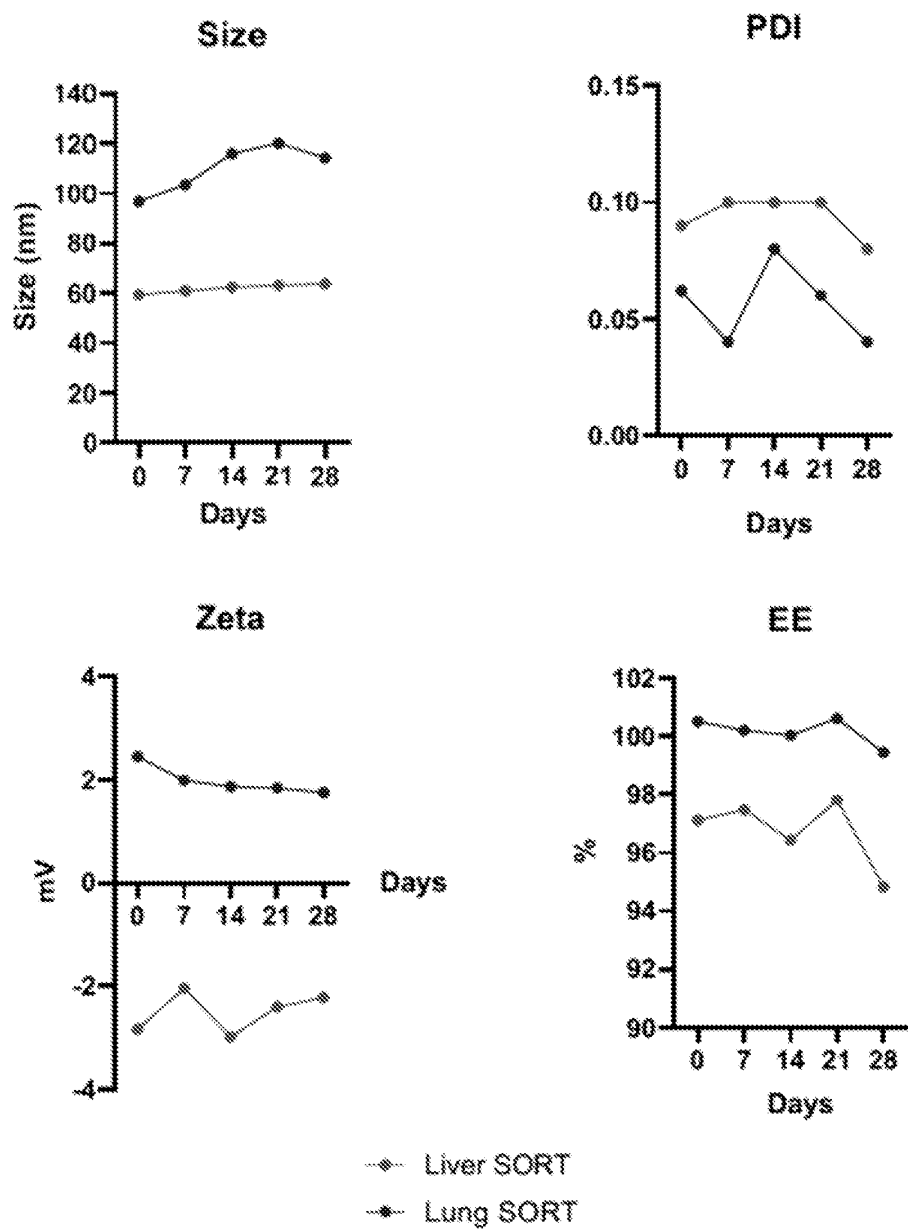
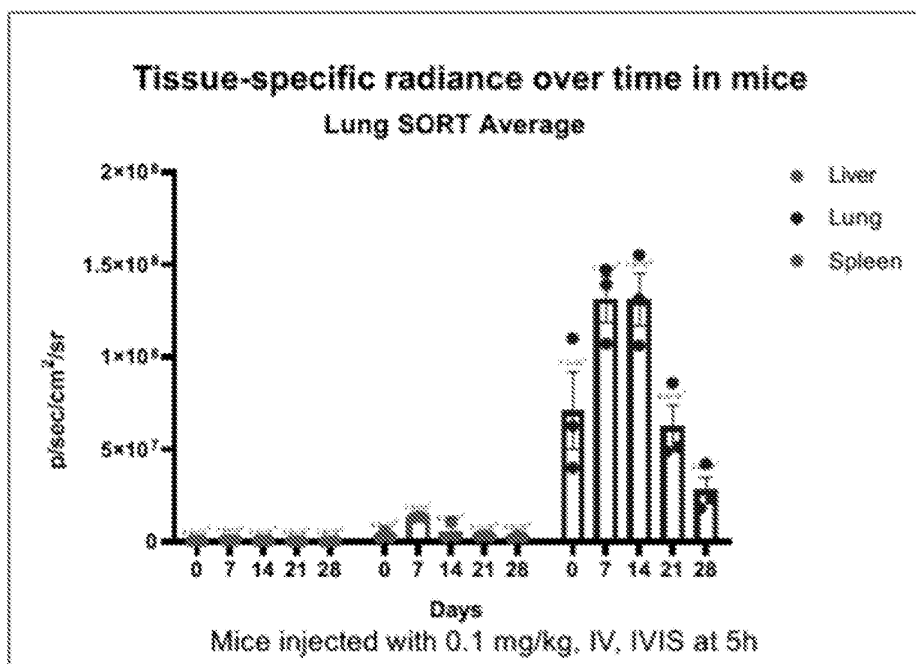


FIG. 5

**FIG. 6**

**FIG. 7**

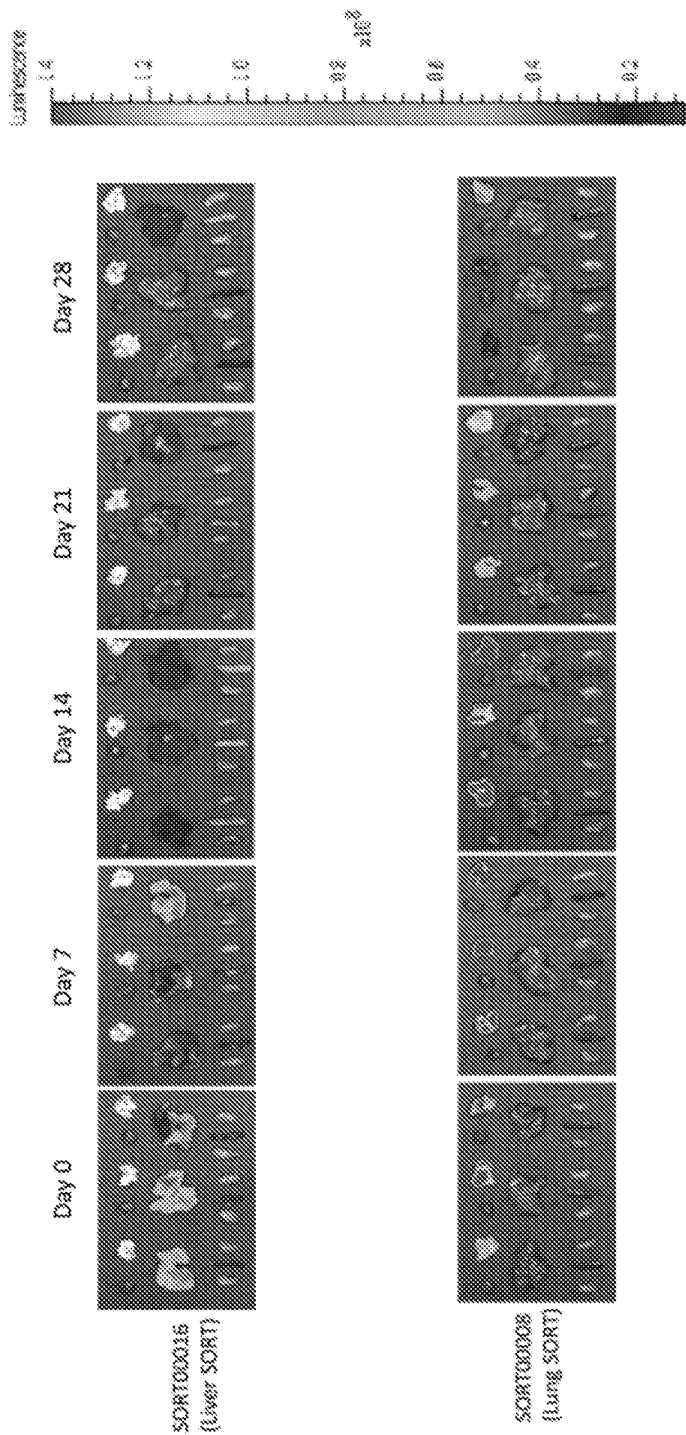
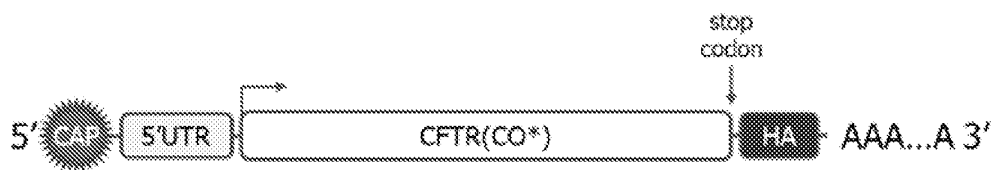
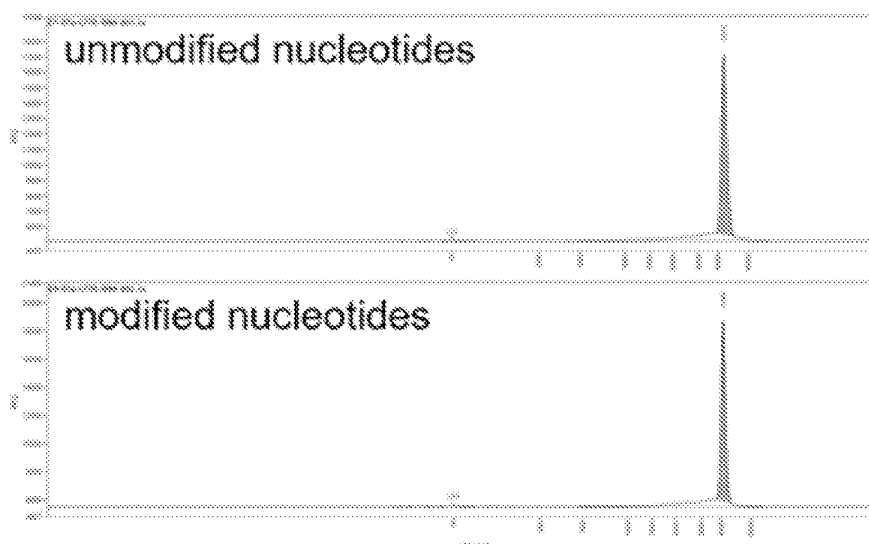


FIG. 8

**FIG. 9A**

CFTR mRNA produced internally by in vitro transcription
using unmodified and modified nucleotides

**FIG. 9B**

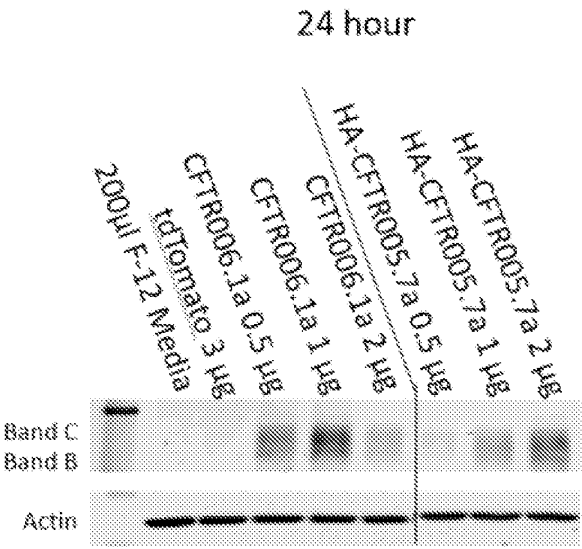


FIG. 10A

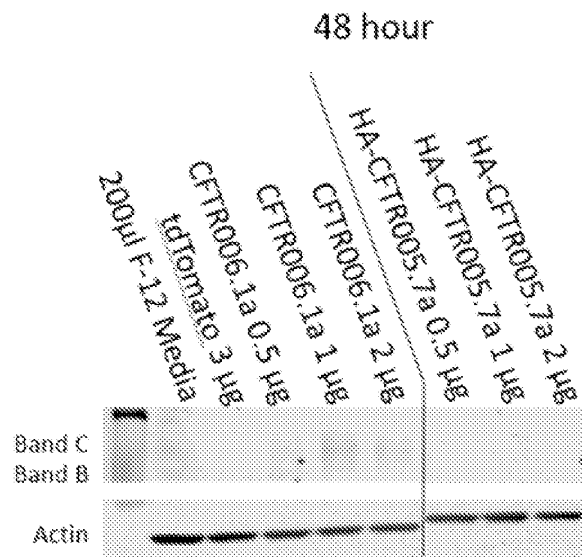


FIG. 10B

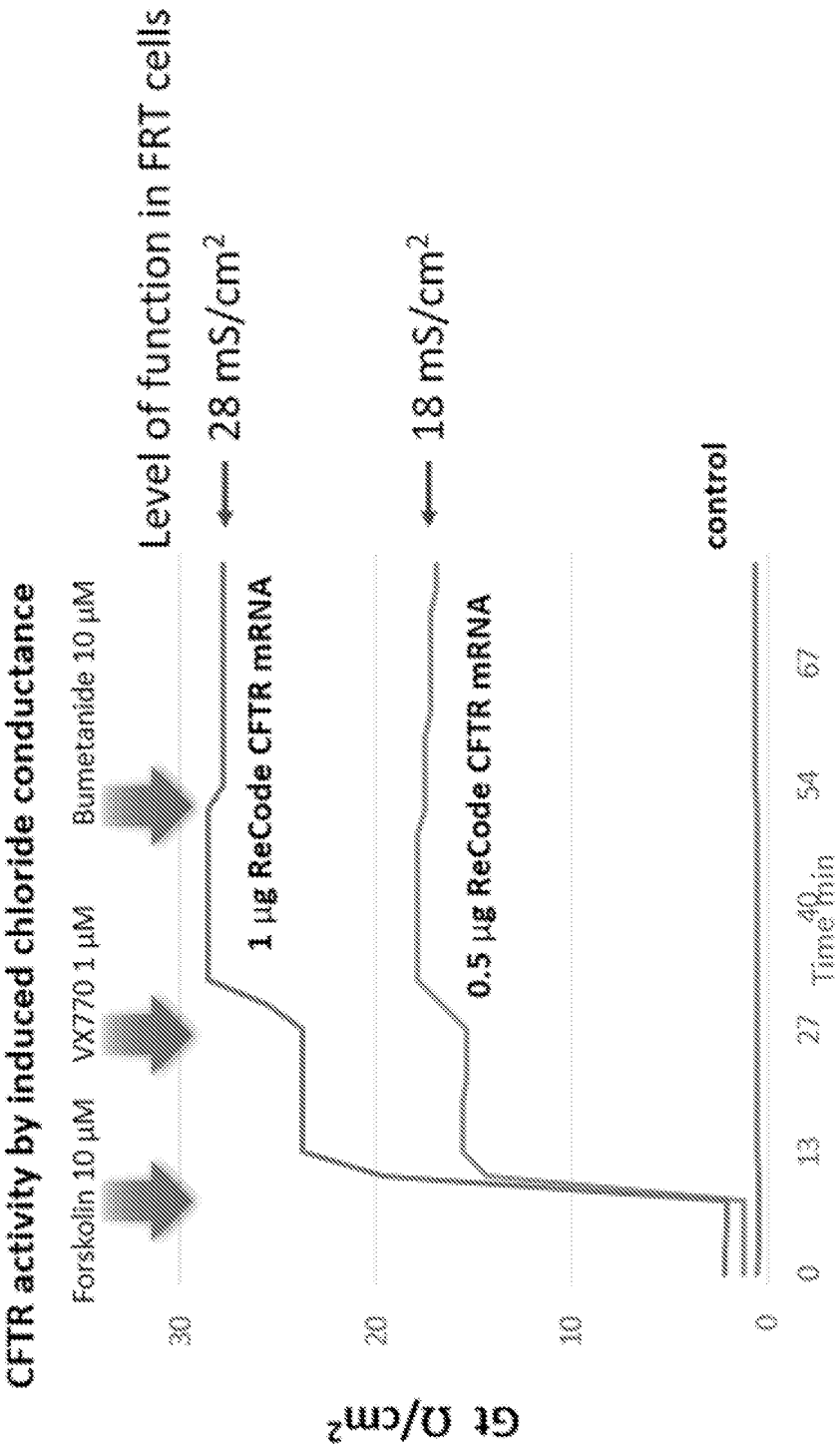


FIG. 11A

Representative conductance kinetic traces in FRT monolayer after addition of CFTR modulators

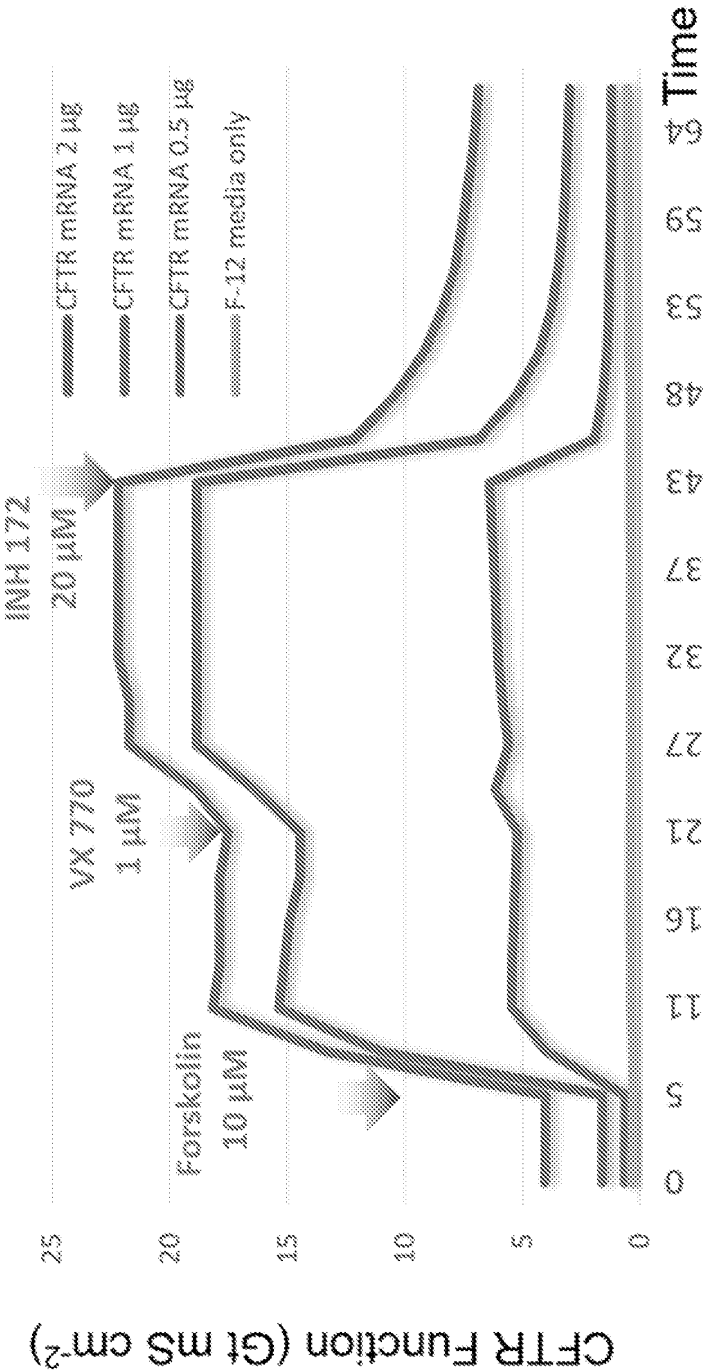


FIG. 11B

CFTR activity by forskolin induced and INH 172-
supressed Cl⁻ conductance

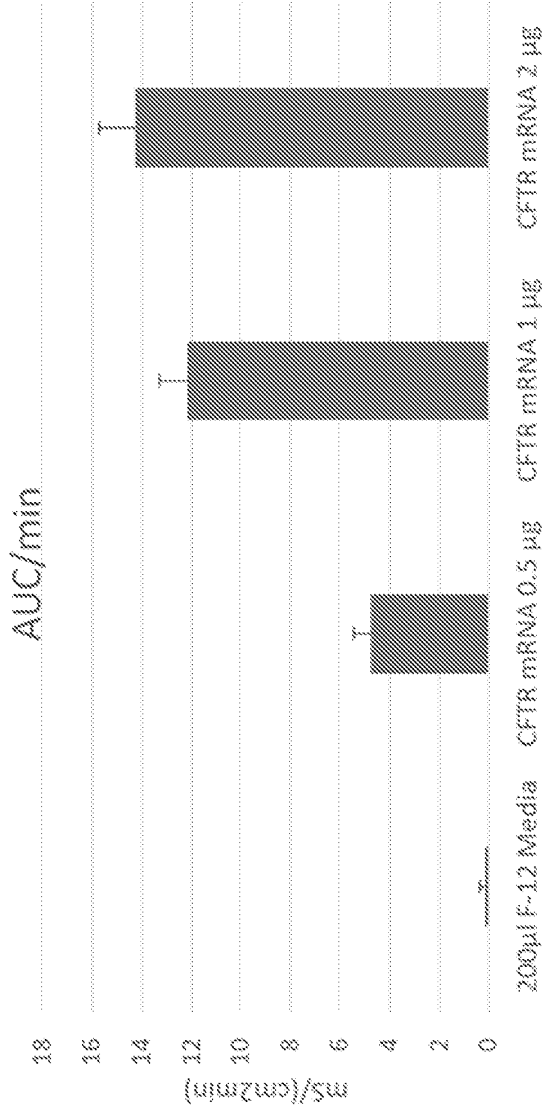


FIG. 11C

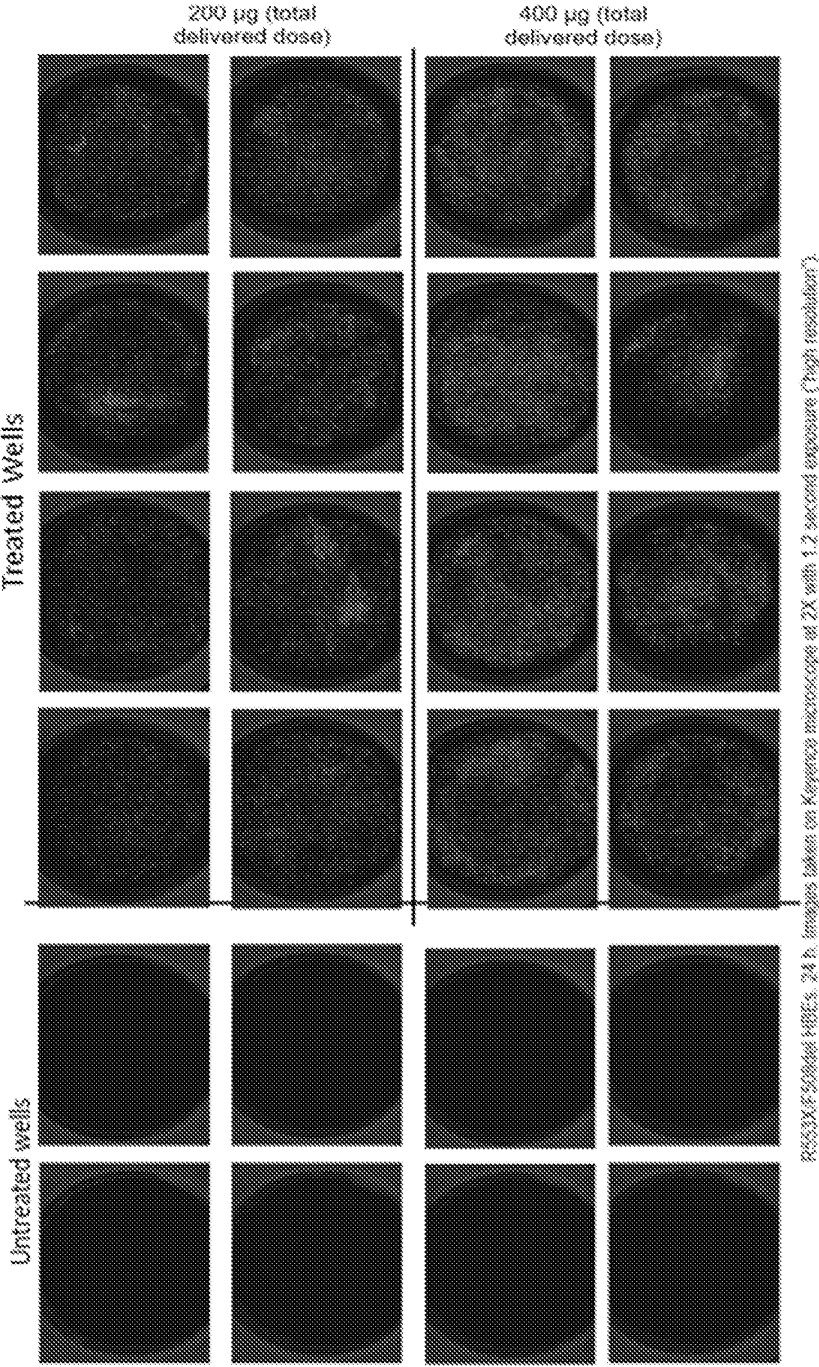


FIG. 12

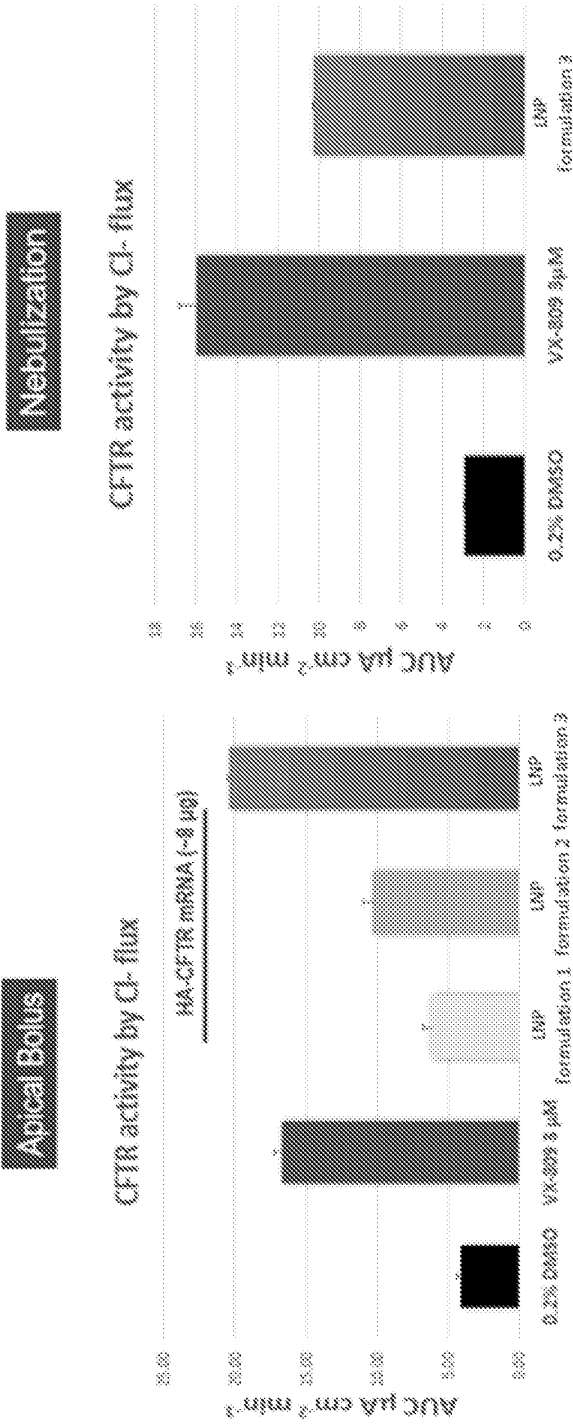


FIG. 14B

FIG. 14A

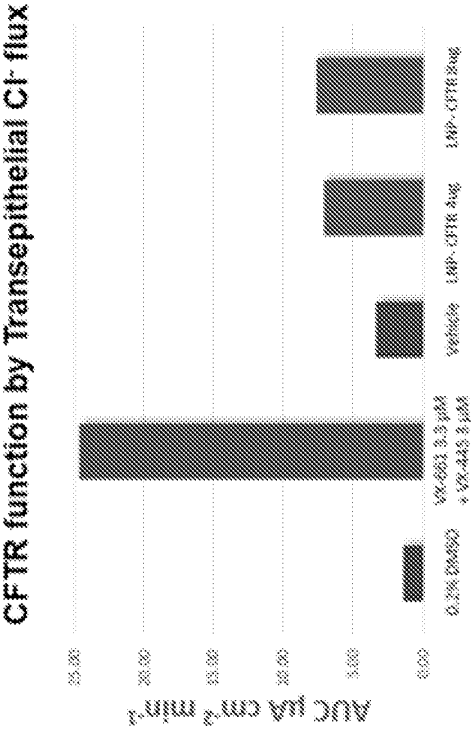


FIG. 15B

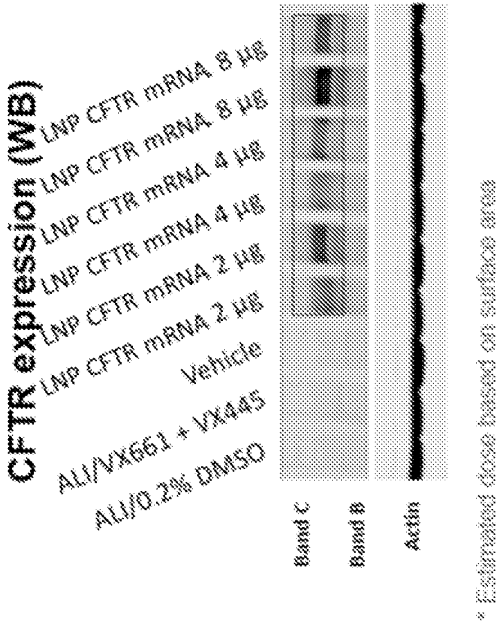


FIG. 15A

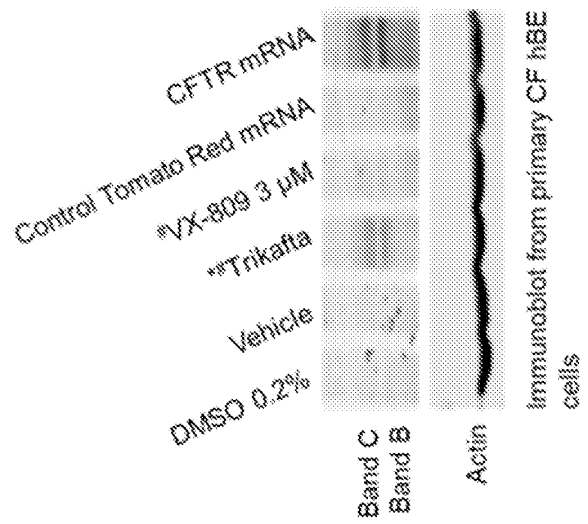


FIG. 16B

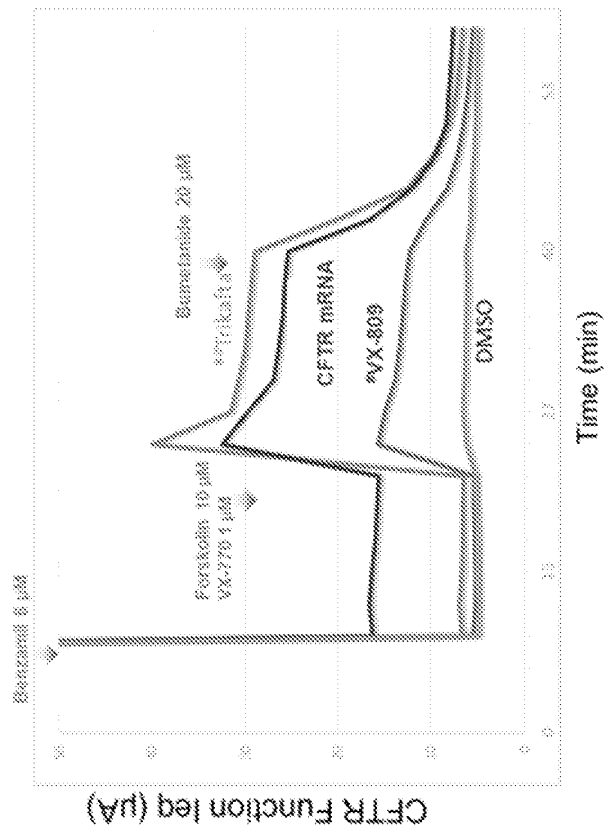


FIG. 16A

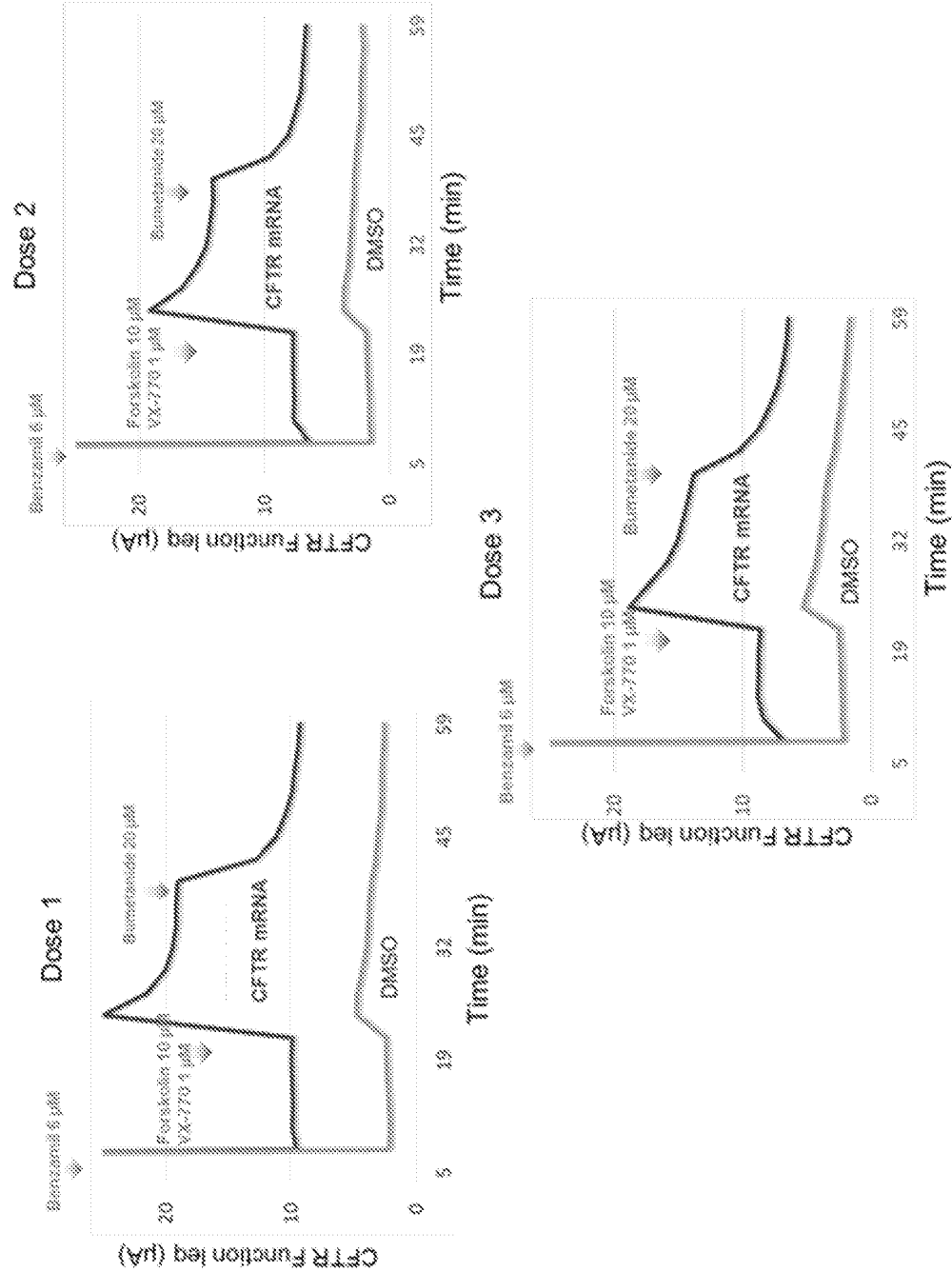


FIG. 17A

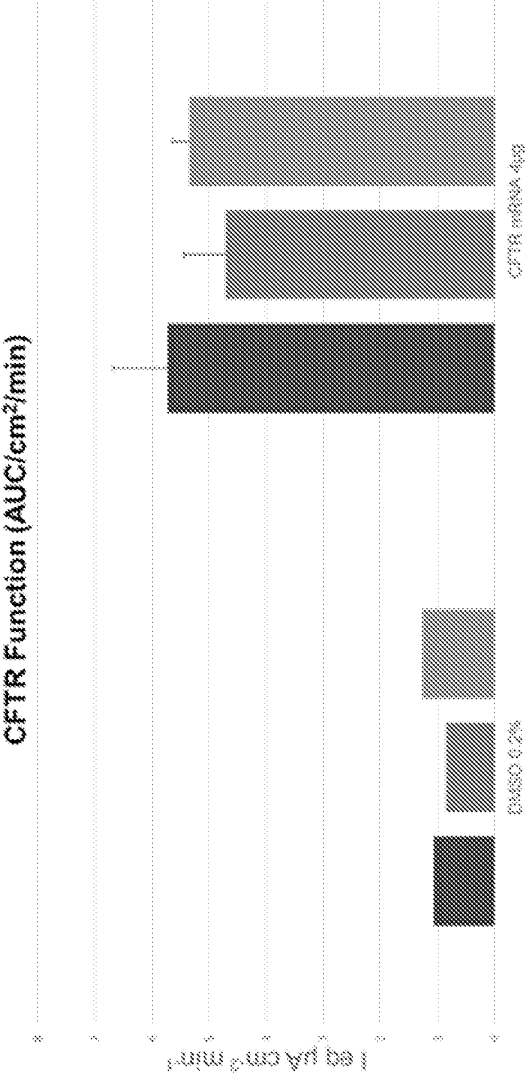


FIG. 17B

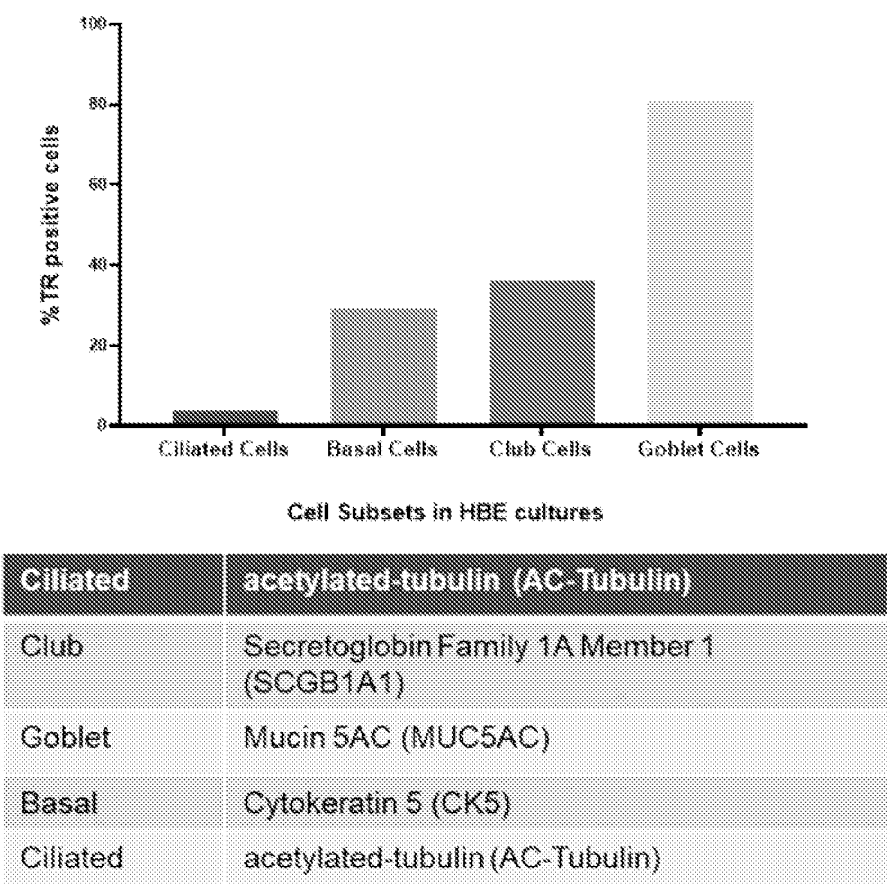


FIG. 18

Examples

Indication / Inclusion criteria	Phase 1/2 Study*	Primary Outcome Measures
CF Lead tRNA Program - Adults - CFTR mutation R553X, R1162X, or G542X - FEV1 between 40% and 90%	SAD ▪ MAD (3 mo.) ▪ OLE (9 mo.) Placebo Low Dose High Dose	- Safety and tolerability - Absolute change in percent predicted FEV1

FIG. 19