

PERCEPTIONS OF AIRWAY GENE THERAPY FOR CYSTIC FIBROSIS

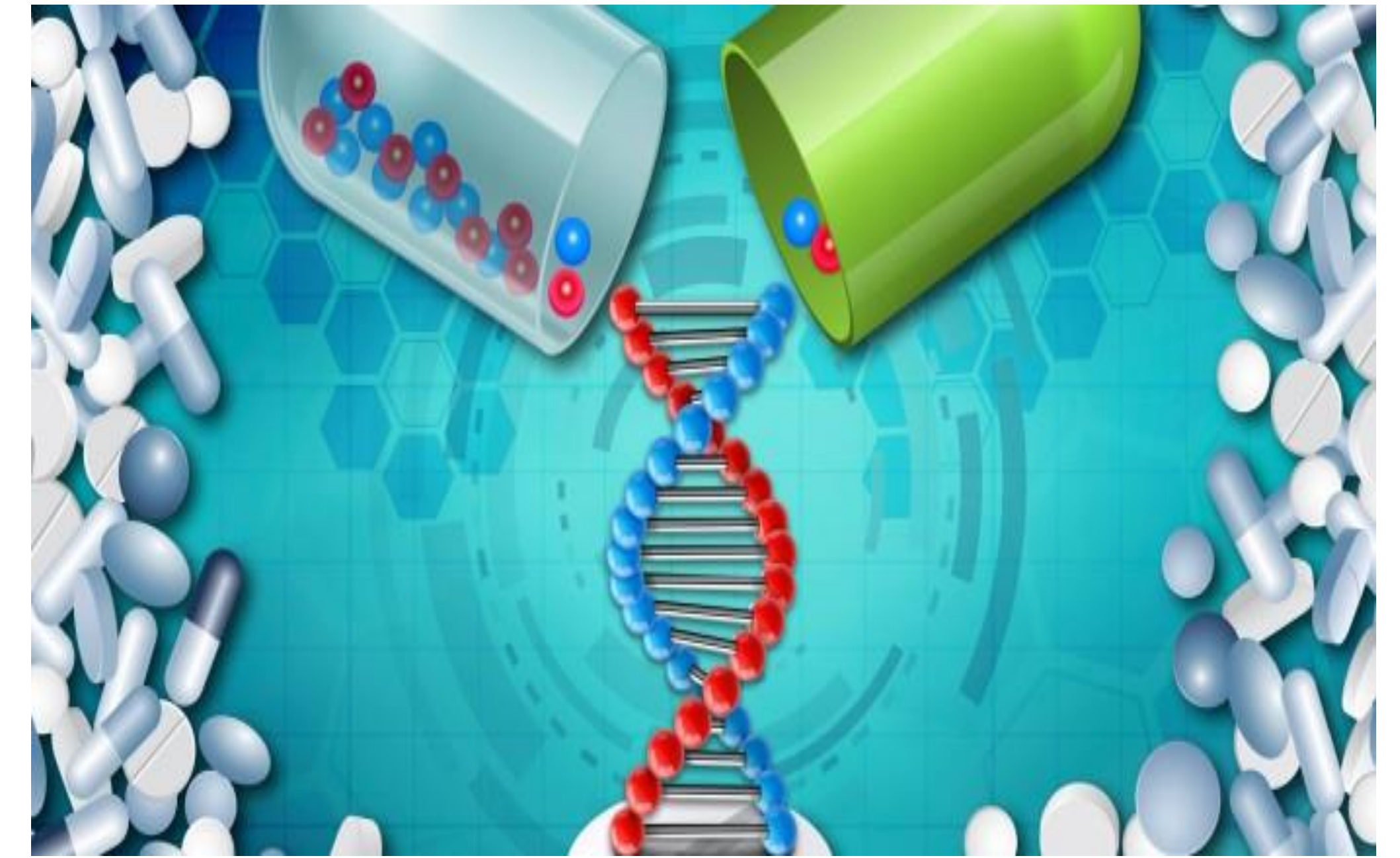


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Background

- ❖ Airway gene therapy has the potential to produce a lasting therapeutic benefit for cystic fibrosis (CF) lung disease
- ❖ Both patient and public support is critical for the successful adoption of this new technology.
- ❖ **Aim: to examine the perceptions towards airway gene therapy for CF among people with CF, their families, and people not affected by CF.**



Picture from <http://blogs.plos.org/dnascience>

Methods

- ❖ Quantitative online survey.
- ❖ Measures of: experience with CF, perceptions of health, knowledge of current treatments, knowledge of gene vectors, potential benefits and harms of gene therapy, and the acceptability of different treatment regimens.
- ❖ Preliminary data has been collected from 128 participants (107 female, 21 male).
 - ❖ 18 participants have a diagnosis of CF ($M_{age} = 32.89$, $SD = 10.92$, range 14-48).
 - ❖ 73 participants have a family member with CF (i.e., child, sibling).
 - ❖ 24 participants know someone with CF.



Picture from <http://theconversation.com>

Current Findings

- ❖ 65% of the sample believe they have 'quite a bit of knowledge' about treatments for CF in general and 71% of had heard about gene therapy.
- ❖ Close to 60% of participants hope that gene therapy will be able to provide a cure for the disease.
- ❖ 64% would be willing to consider consenting to take part in a gene therapy trial.

- ❖ 95% agreed that it is "ethically correct for young children to be given the opportunity to be involved in gene therapy trials, if their families agree and if the trials are run very carefully".
- ❖ Airway bronchoscopy was considered an acceptable form of delivery by 74% of participants, and a further 24% were unsure, stating that it would be dependent on risk, severity, and the frequency of doses. A dosing frequency of 2 or more years was considered extremely acceptable by more than 60% of the sample.
- ❖ When asked whether they had any other comments related to gene therapy for CF, some raised concerns about not knowing the potential longer term impacts, the time it may take to develop, and the potential cost of a new treatment. Others say it is their "only hope".

Discussion

- ❖ Overall people have positive opinions about airway gene therapy for CF.
- ❖ Understanding the views of the CF community regarding gene therapy will provide researchers with important insights in to how to best target potential treatment.
- ❖ Further education for the community about gene therapy is vital.



Scan to participate!

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**INSPIRING
ACHIEVEMENT**