

# Gene therapy: cross roads or revolution?

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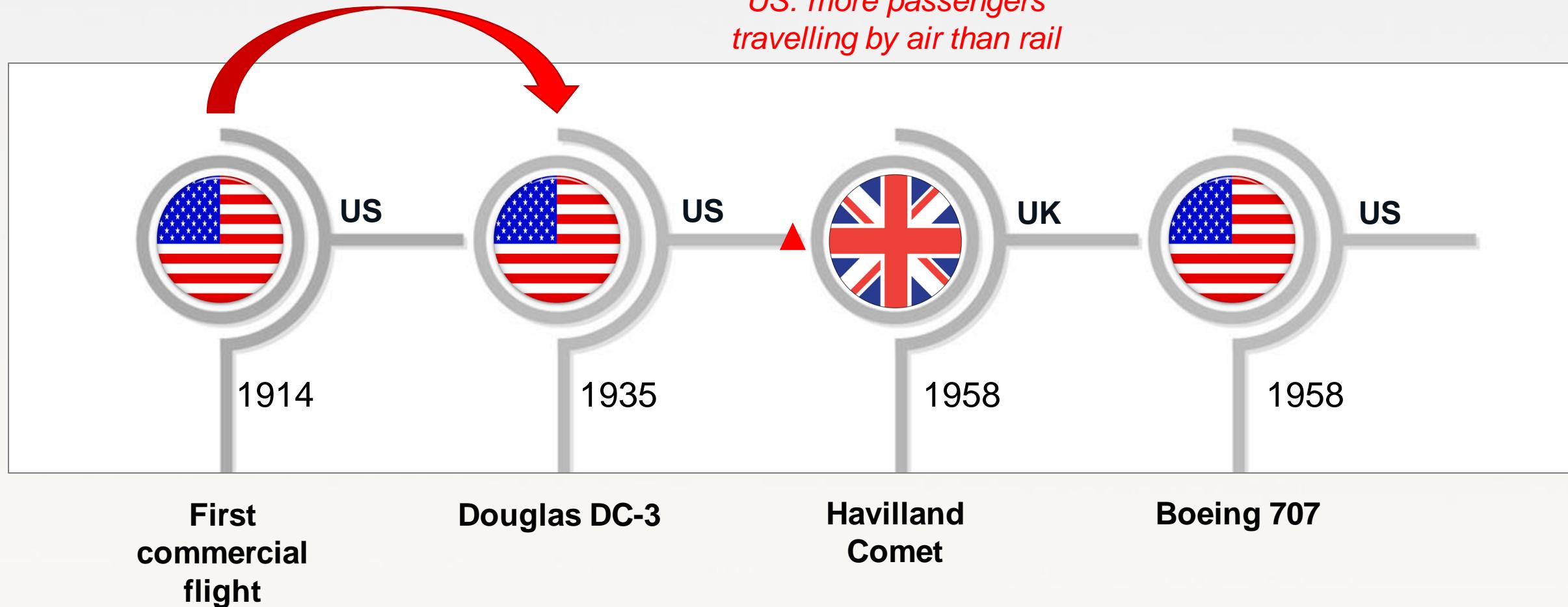


# Agenda

- **Introduction**
- Parallels with another revolutionising industry
- Reimbursement Considerations



# A brief history of commercial aviation



# ...but it wasn't all 'plane' sailing



## Safety

- Landing 'blind'
- Weather challenges
  - Leap of faith

## Perception

- Negative public perception
  - Fears



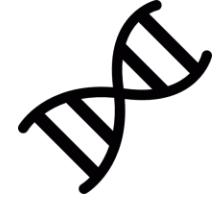
## Affordability

- First flight fare across Florida \$400
- Only for the rich

## Economics

- Low profits
- Non-viable economics

# This could be gene therapy!



## Safety

- Initial failures
- Long term impact
  - Suitability

## Perception

- Negative public perception
- Unmanageable costs, misunderstanding



## Affordability

- First gene therapy \$1m
- Only for the 'privileged'

## Economics

- Low profits
- Non-viable business model

# Lessons learned



## Group Vision

Making passenger air travel a sustainable reality '*the spirit of boldness to make it happen*'



## Blueprint

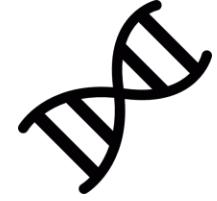
Guggenheim fund to develop a successful blueprint and demonstrate effectiveness



## Collaborate

Competing companies created partnerships and shared learnings, innovations  
Public investments

# Applying that to gene therapy



## Safety

- Evidence generation
  - Dealing with data uncertainty



## Perception

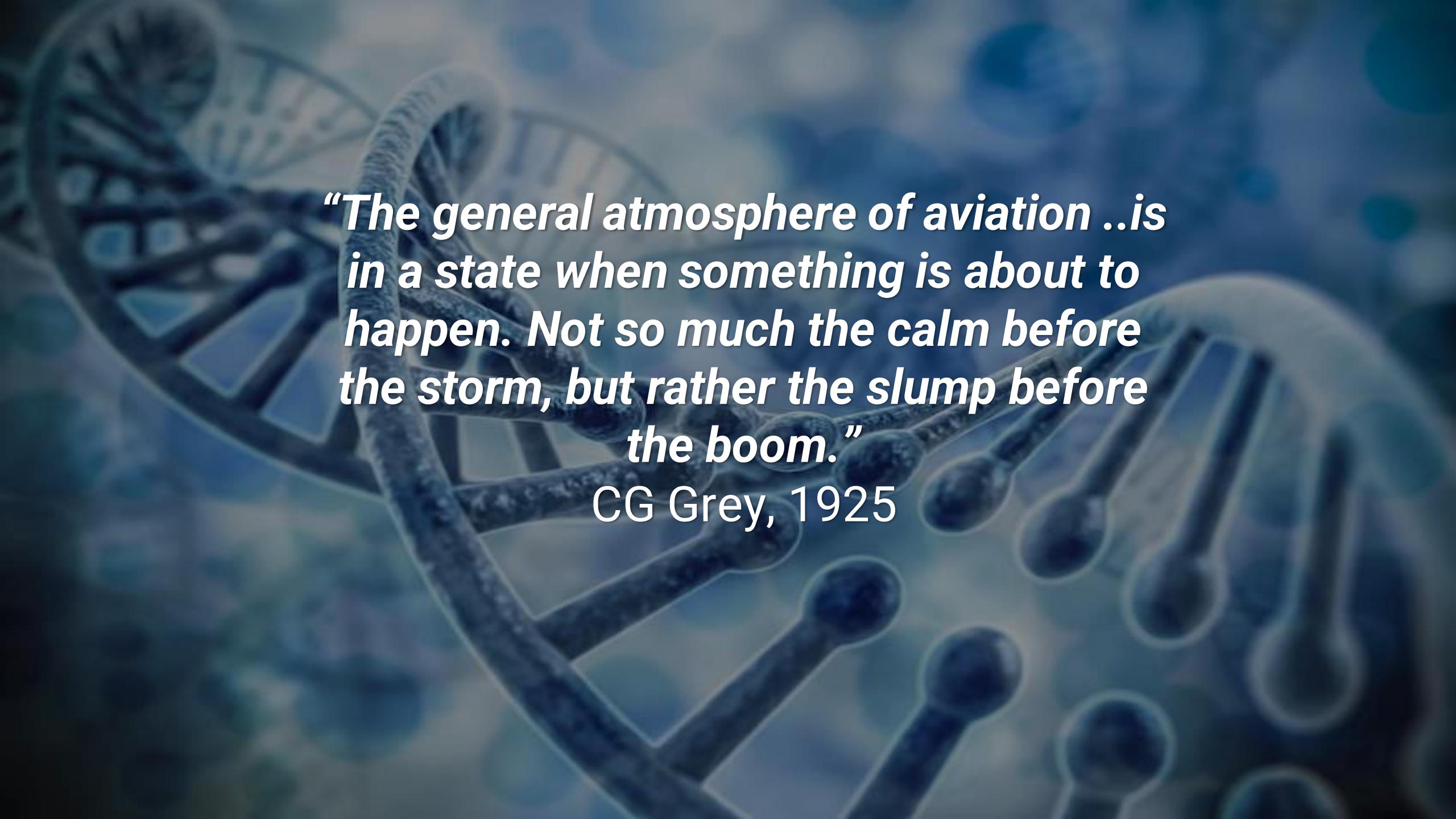
- Proactively managing stakeholders expectations
  - Cross-company partnerships, collaboration, ARM

## Affordability

- MEAs and funding annuity models to minimise budget impact

## Economics

- Government funding?
- Sharing innovations e.g. portable gene editing units and Bio e-printing



*"The general atmosphere of aviation ..is  
in a state when something is about to  
happen. Not so much the calm before  
the storm, but rather the slump before  
the boom."*

CG Grey, 1925

# Join the gene therapy debate

## Webinar 28<sup>th</sup> November 1800 CET



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