Gene Therapy in Alzheimer's Disease: Novel Therapies and Ethical Aspects of Somatic Gene Editing



Human Development

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Goal: Investigate the ethical and social implications of developing a DMT for LOAD, including the impact it will have on future AD patients, researchers, clinicians, and the U.S. healthcare system

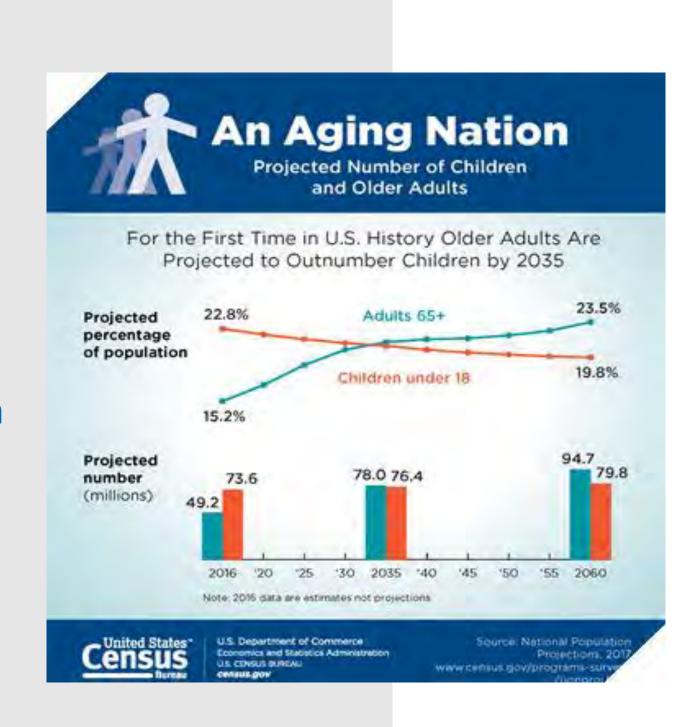
Why is Alzheimer's Disease (AD) unique?

The aging U.S. population is making AD an increasingly common problem

- Currently 5.8 million individuals in the US with AD
- Estimated 13.8 million individuals by 2050

o By 2035, U.S. is expected to have more adults 65+ than children under 18

https://www.alz.org/media/Documen ts/alzheimer s-facts-and-figures-2019r.pdf



Challenges with a successful DMT for AD

- 1. Size of AD population & lack of infrastructure
- 2. Diagnosis and stratification of patients into clinical trials
- 3. Expenses and alternative payment models

Lack of infrastructure

- Current healthcare system lacks appropriate diagnostic infrastructure for large patient pop.
- Limited supply of specialists + physicians



- o The demand for geriatricians is already greater than supply
- o Need an estimated 25,000 full time geriatricians in clinical practice by 2050 to meet demand

Diagnosis and stratification

Methods of AD diagnosis (ex. biomarkers, PET scans, and cerebrospinal fluid) are inaccurate:

- Low sensitivity to pre-symptomatic patients
- Older age of patients complicate the diagnosis

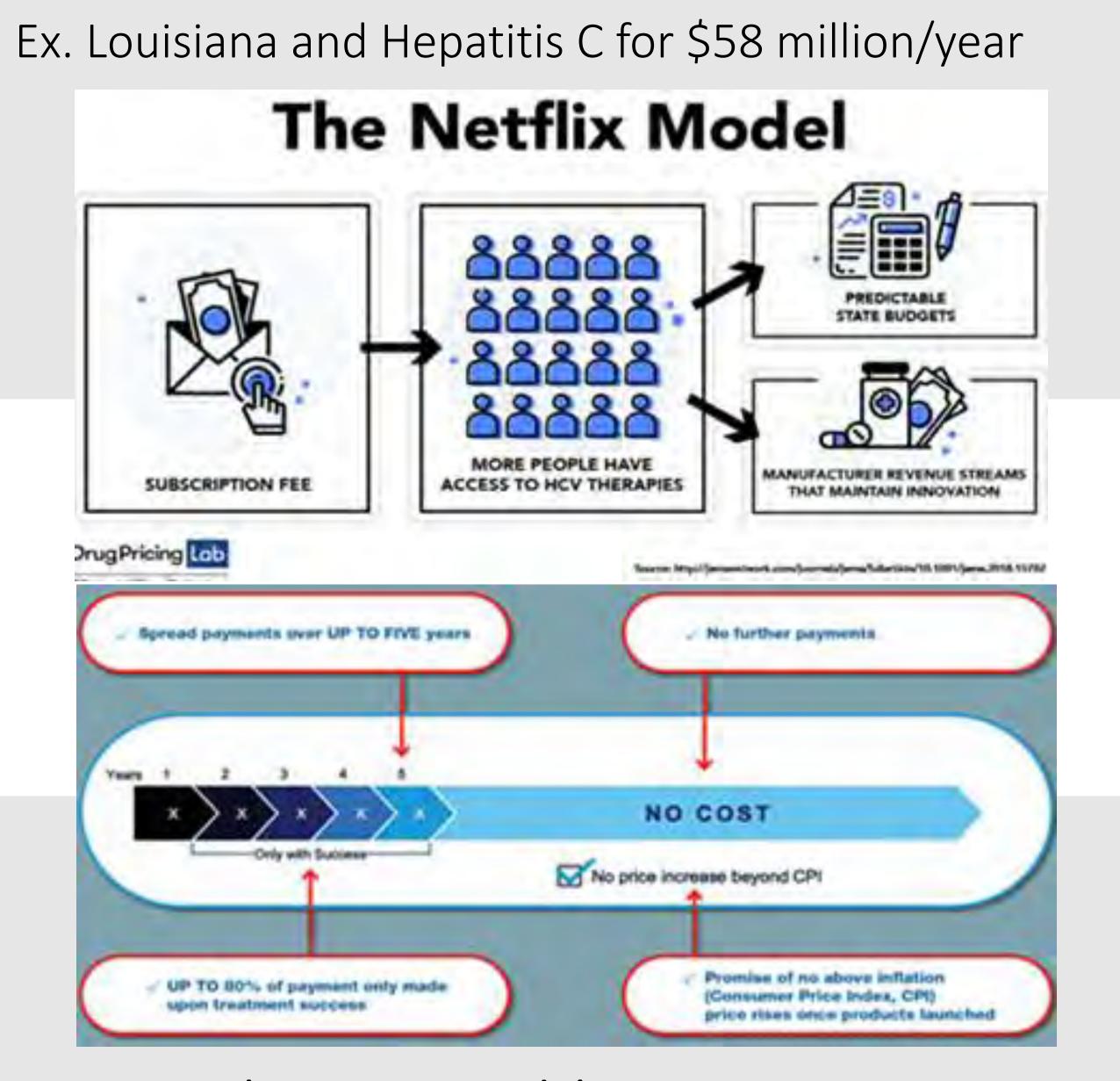
Expenses and alternative payment models

Lifetime healthcare cost of a patient with AD is \$350,174 (calculated for a patient with the average age of 83)

How do we pay for an expensive DMT?

Netflix model

Similar to a monthly subscription plan with a flat fee and unlimited access to the drug



Sequential Payment Model

Patients (or more likely insurers) pay a one time, non-negotiable sum up front; remaining cost is split into annual payments

Ex. Bluebird Bio and \$1.8 million Beta Thalassemia

Conclusion: We must begin to address challenges for an AD DMT now in order to reimagine the current system for better delivery in the future