

Collaborating with Academia

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Rare Bootcamp

Ultragenyx Pharmaceutical

April 17, 2025

Disclaimer



My opinions are based on my experiences and perspective at a large public university.

I use a cynical tactic to illustrate the nuances — albeit factual — of collaborating with academic researchers.

Every investigator, department, and institution is different.

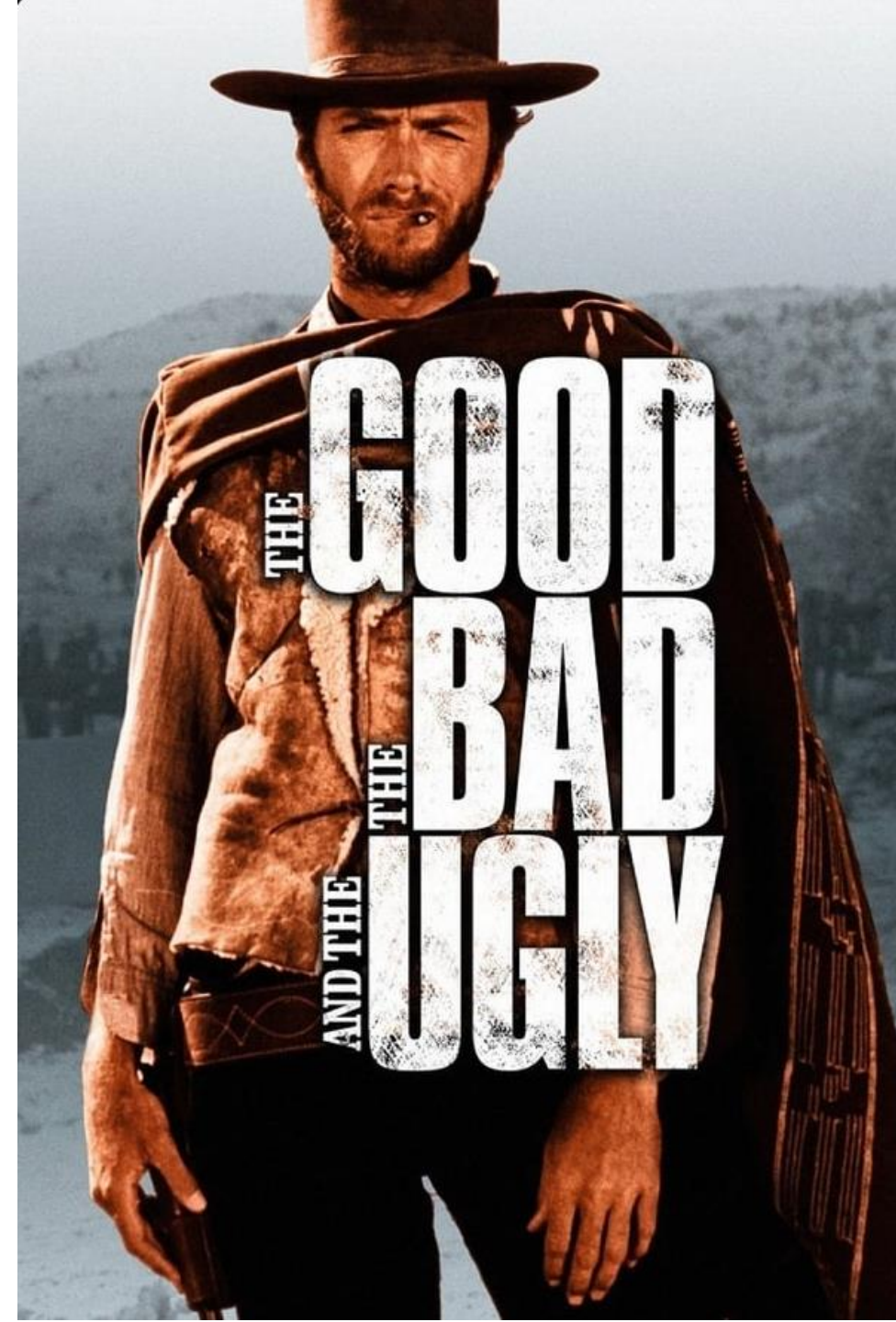
My Background

- Tenured Professor, Texas A&M University (2008-present)
- Chief Scientific Officer, GeneTx Biotherapeutics (2017-2020)
- Executive Director Molecular Genetics, Ultragenyx Pharmaceutical (2020-present)

My Background

- Continuous funding from federal agencies, foundations, companies, and licensing agreements.
- 15 years collaborating with rare disease foundations.

Collaborating
with
Academia



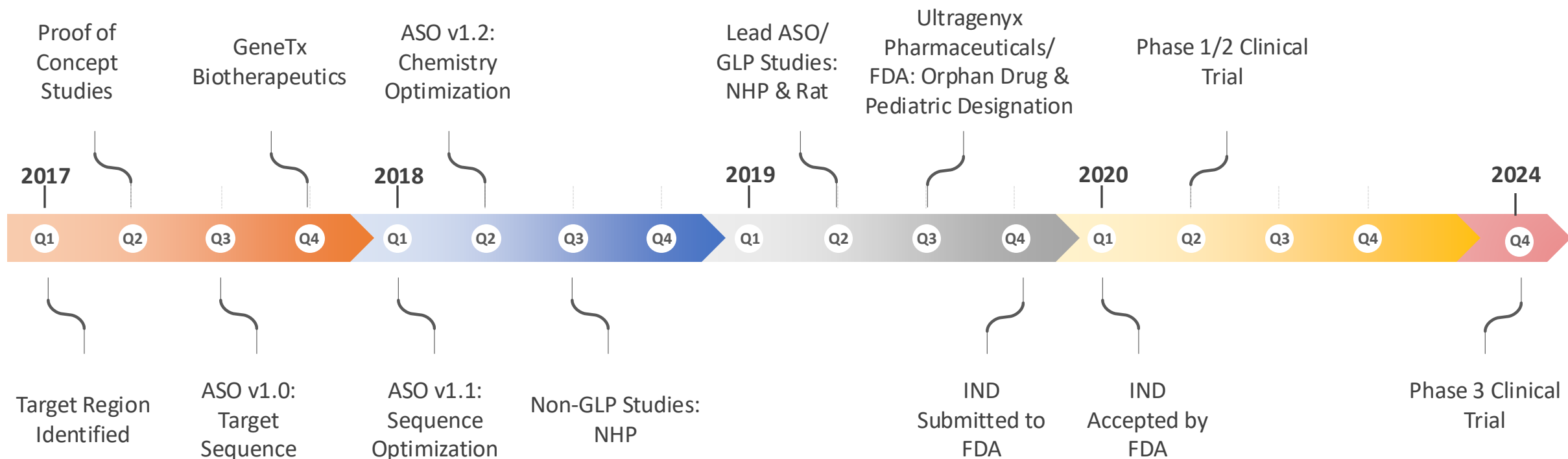
The GOOD

Academic research

The engine that drives innovation



The First Molecular Therapy for Angelman Syndrome to Advance into Clinical Development



the BAD

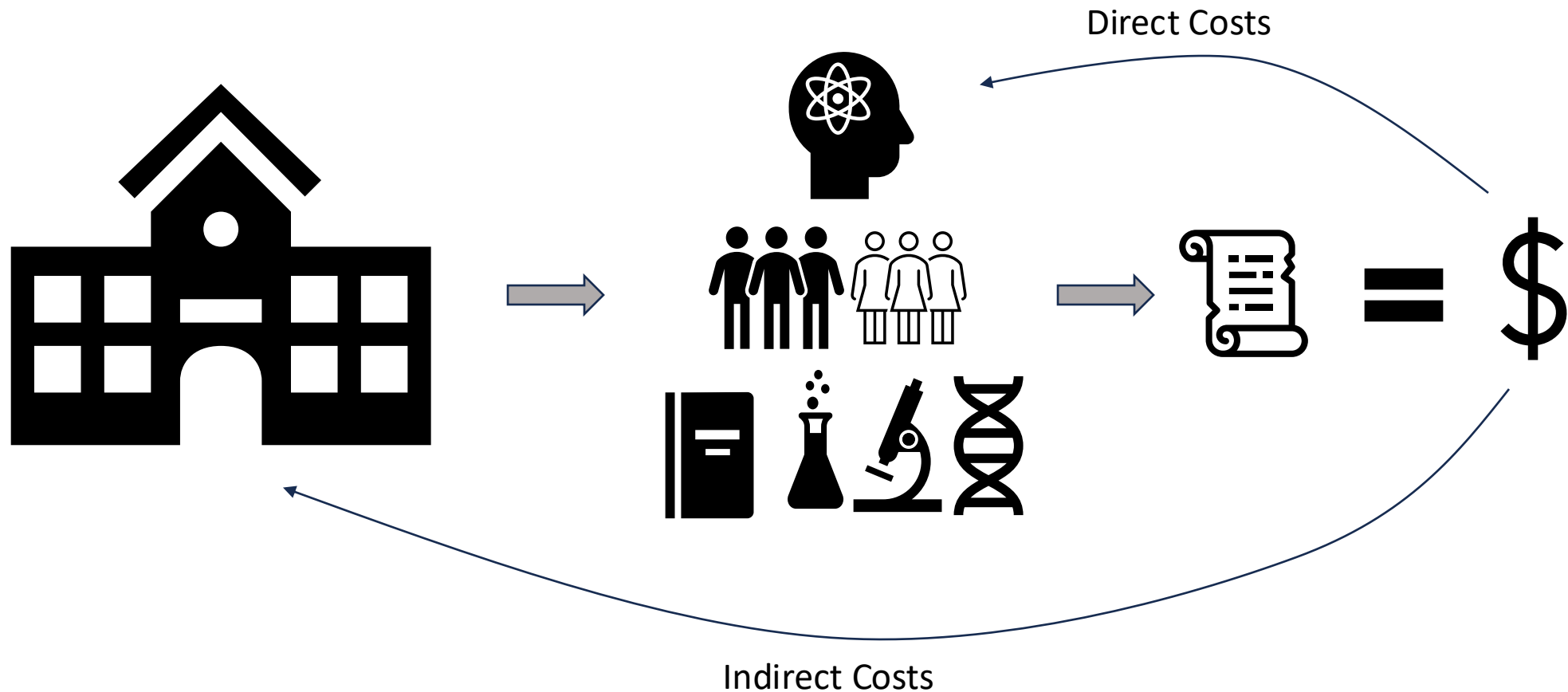
Research Universities

The largest flea markets in the world



Academic Research

An unusual business model measured in grant dollars and publications



Academic Collaborators

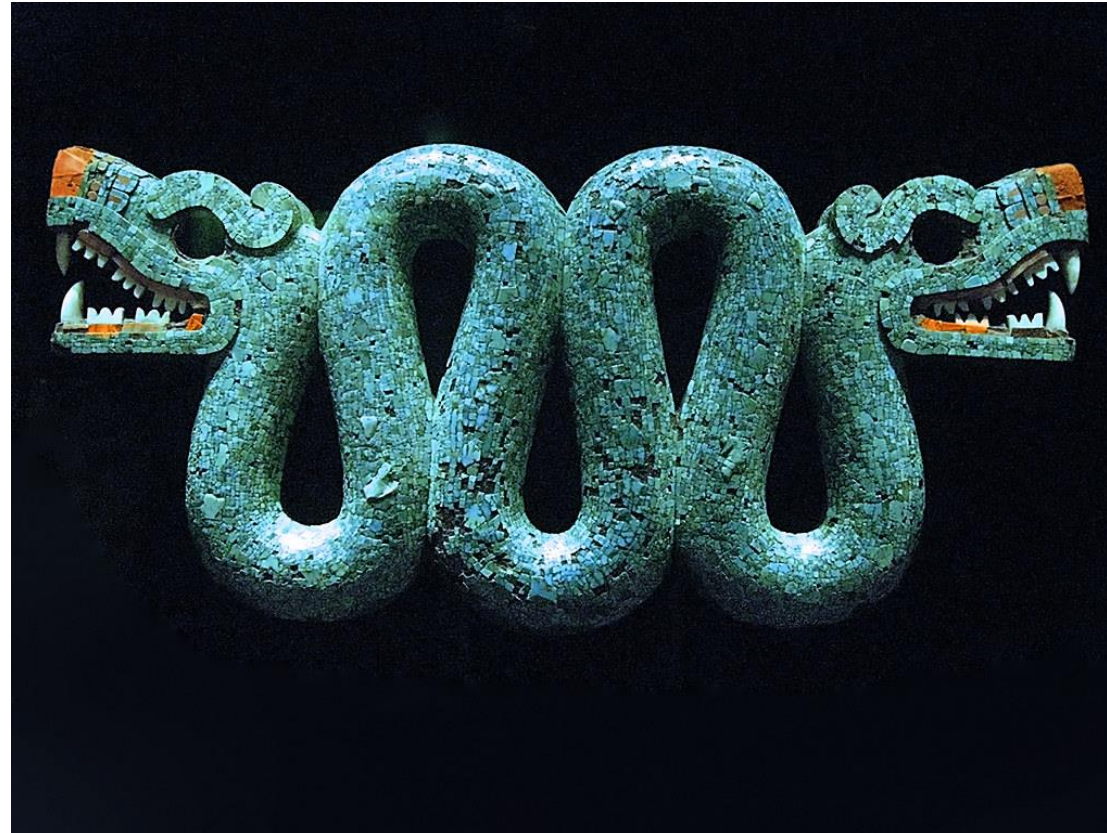
Several entities often with different priorities

Principal Investigator Priorities

- Tenure and Promotion
- Grants
- Publications
- Trainees
- Teaching

Departmental Priorities

- Research mission
- Institutional mandates
- Budgets
- Indirect costs
- Promotion & Tenure



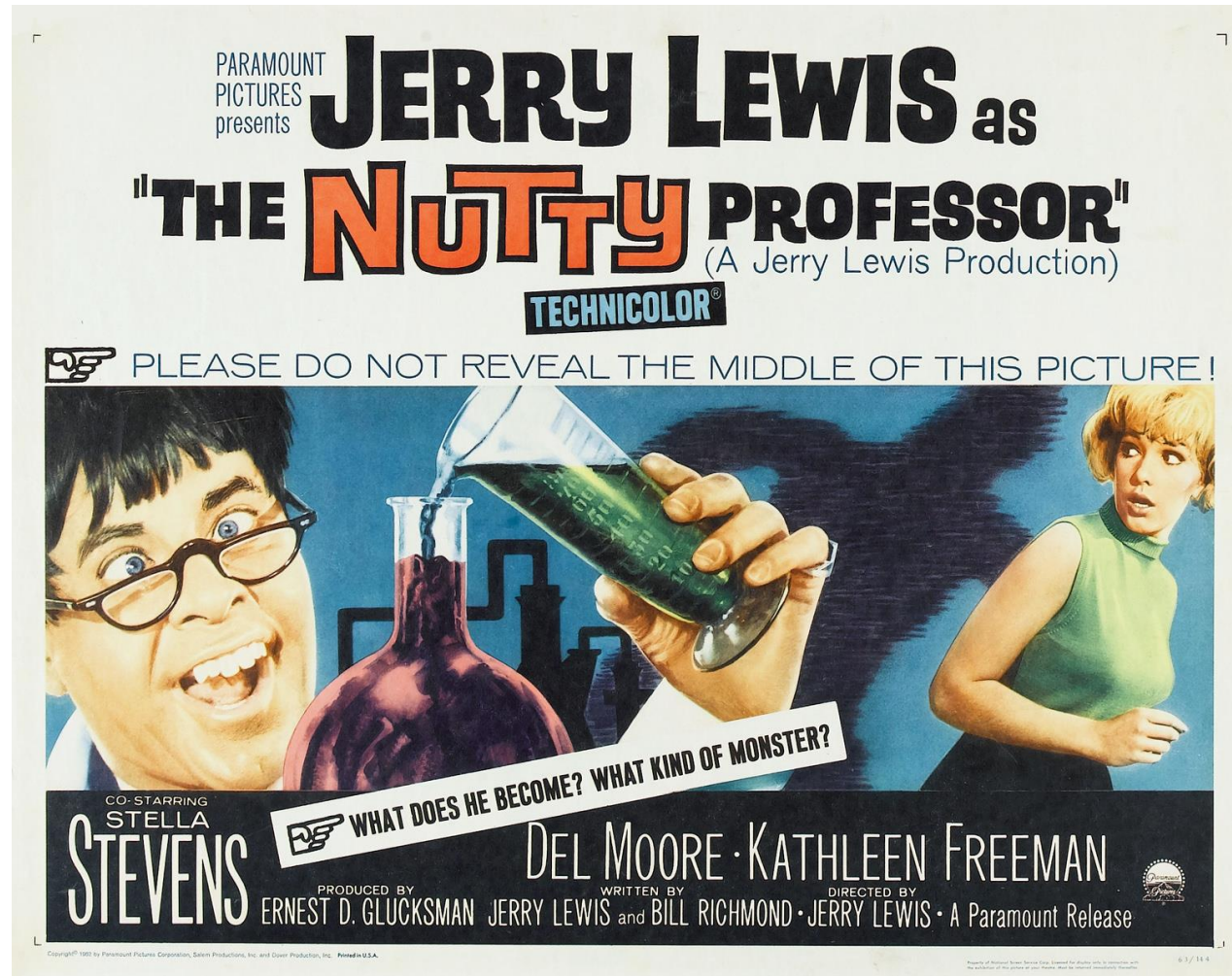
Institutional Priorities

- Academic freedom
- Protecting faculty and trainees
- Indirect costs
- Intellectual property
- Political mandates
- Stakeholder opinions
- National status

and the UGLY

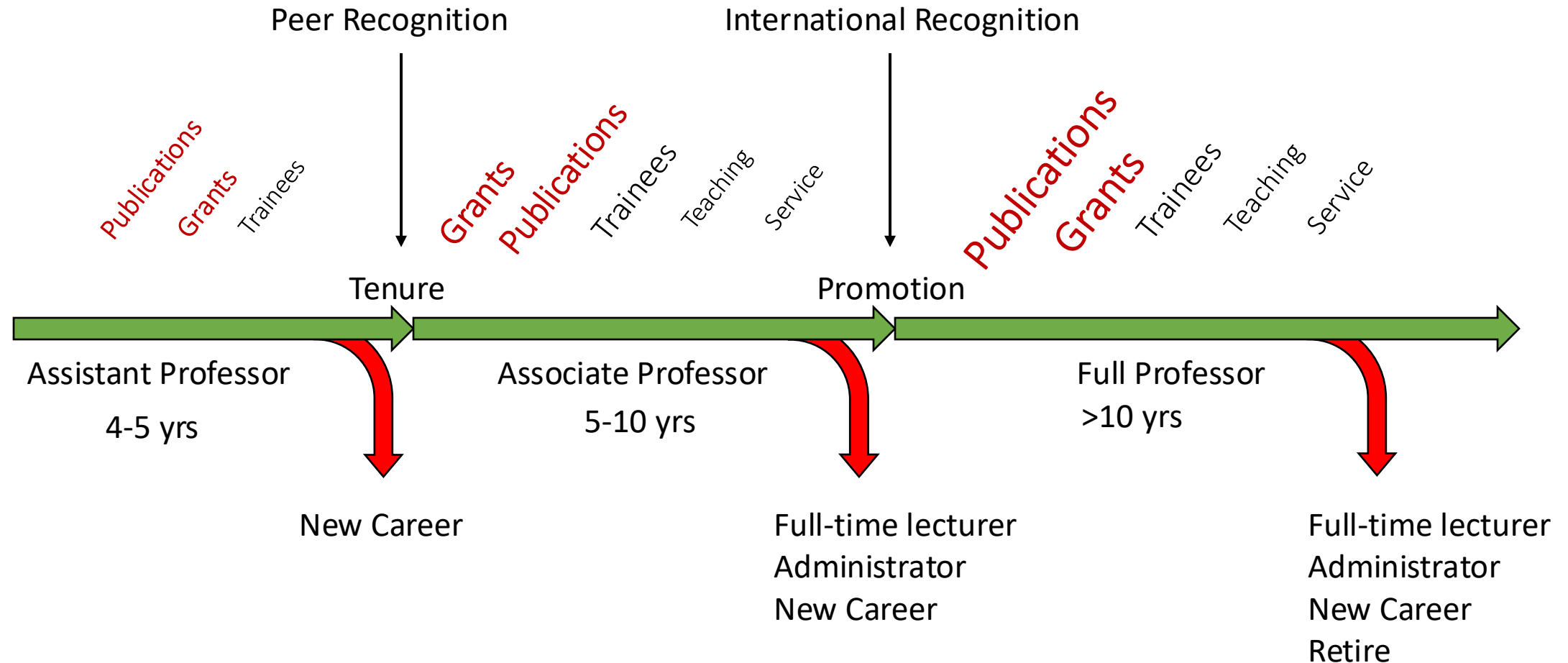
Academic Researchers

An unusual business model creates the most unusual people




The Life-Cycle of an Academic Researcher

It's either up or out




Scientific Publications

Publish-or-perish = too many publications

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National Center for Biotechnology Information

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Angelman syndrome

Search

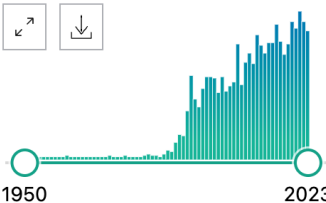
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RESULTS BY YEAR



1950 2023

TEXT AVAILABILITY

☐ Abstract

☐ Free full text

☐ Full text

☐ **Angelman syndrome: a journey through the brain.**

1 Maranga C, Fernandes TG, Bekman E, da Rocha ST.

Cite FEBS J. 2020 Jun;287(11):2154-2175. doi: 10.1111/febs.15258. Epub 2020 Mar 14.

PMID: 32087041 **Free article.** Review.

Share **Angelman syndrome** (AS) is an incurable neurodevelopmental disease caused by loss of function of the maternally inherited UBE3A gene. ...

☐ **Angelman Syndrome.**

2 Margolis SS, Sell GL, Zbinden MA, Bird LM.


Cite Neurotherapeutics. 2015 Jul;12(3):641-50. doi: 10.1007/s13311-015-0361-y.

PMID: 26040994 **Free PMC article.** Review.


Share In this review we summarize the clinical and genetic aspects of **Angelman syndrome** (AS), its molecular and cellular underpinnings, and current treatment strategies. ...

Scientific Articles

Publish-or-perish = fosters poor science and questionable ethics

 **National Library of Medicine**
National Center for Biotechnology Information

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SNHG14

×

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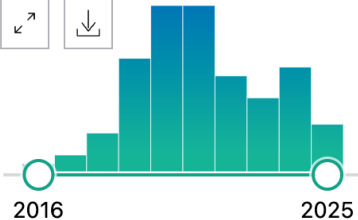
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163 results

Page 1 of 17

RESULTS BY YEAR



☐ **A pan-cancer analysis of small nucleolar RNA host gene 14 (SNHG14) in human tumors.**

1

Cite Feng C, Li Q, Lv M, Ji Q, Ye H, Jiang S, Shao M, Shao Q, Cao L.

Gene. 2025 Jun 5;951:149410. doi: 10.1016/j.gene.2025.149410. Epub 2025 Mar 10.

Share PMID: 40074048 Review.

In particular, patients with increased **SNHG14** expression had reduced overall survival with mesothelioma and stomach adenocarcinoma. ...The review underscores that **SNHG14** is frequently overexpressed in numerous cancers and predominantly functions as an oncogene. ...



Alasdair Taylor
Biomedical Sciences PhD Candidate
Texas A&M University

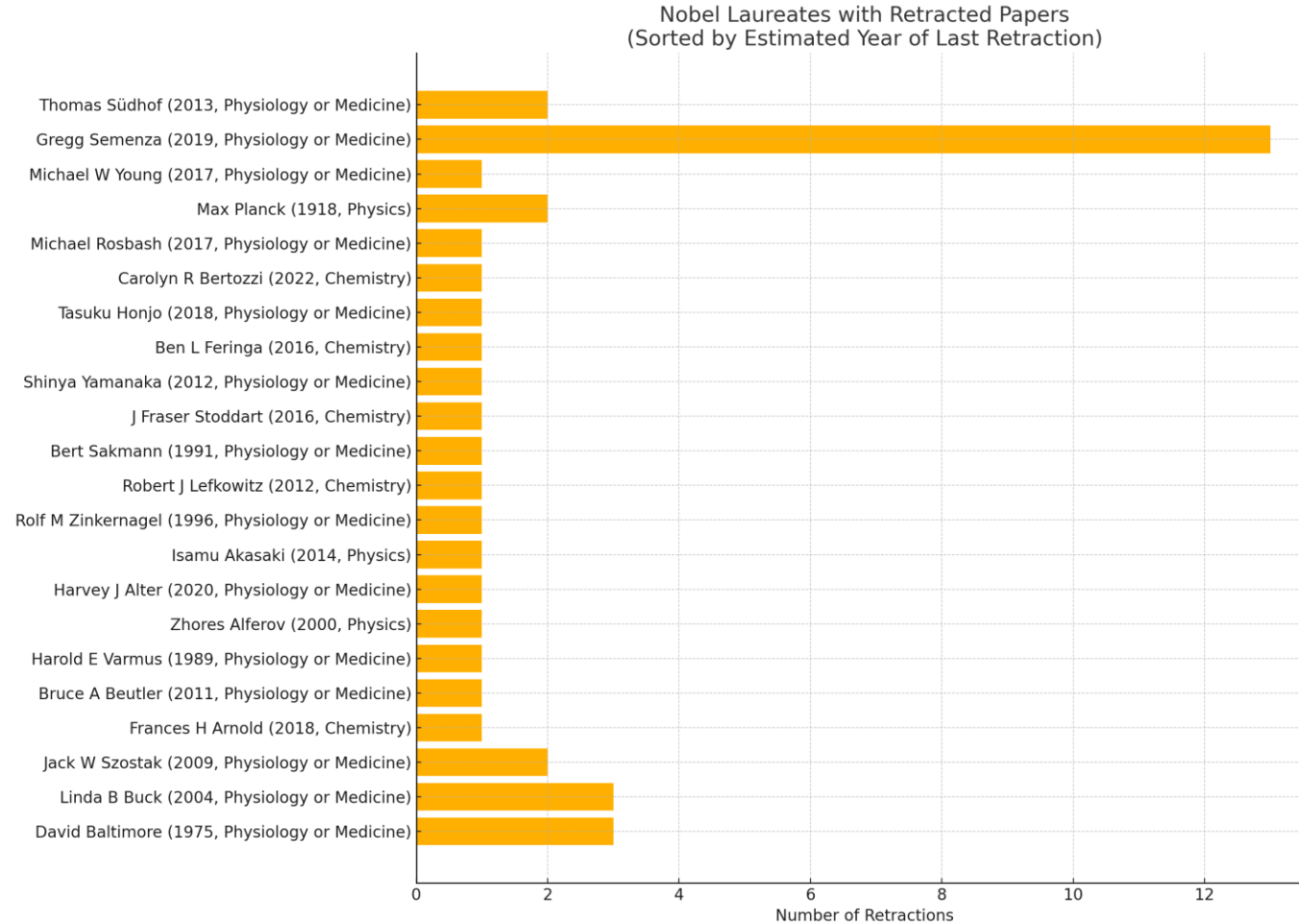
Sequence errors are ‘canaries in a coal mine’ in genetics studies, sleuth says

A genetics researcher came across an interesting paper earlier this year on the gene he studies. The scientist, a doctoral candidate who asked not to be named, decided to take a closer look at which part of the gene, *SNHG14*, the authors targeted to measure its expression. He ran the sequence of the short strand of DNA, called a primer, given in the paper through a database and found the sequence matched with a completely different gene.



21 *SNHG14* Published Articles Retracted

Being famous doesn't mean being right



Academic Grants

Shameless self-promotion

Expectation



Reality



Academic Grants

A grant is not a contract

Grant



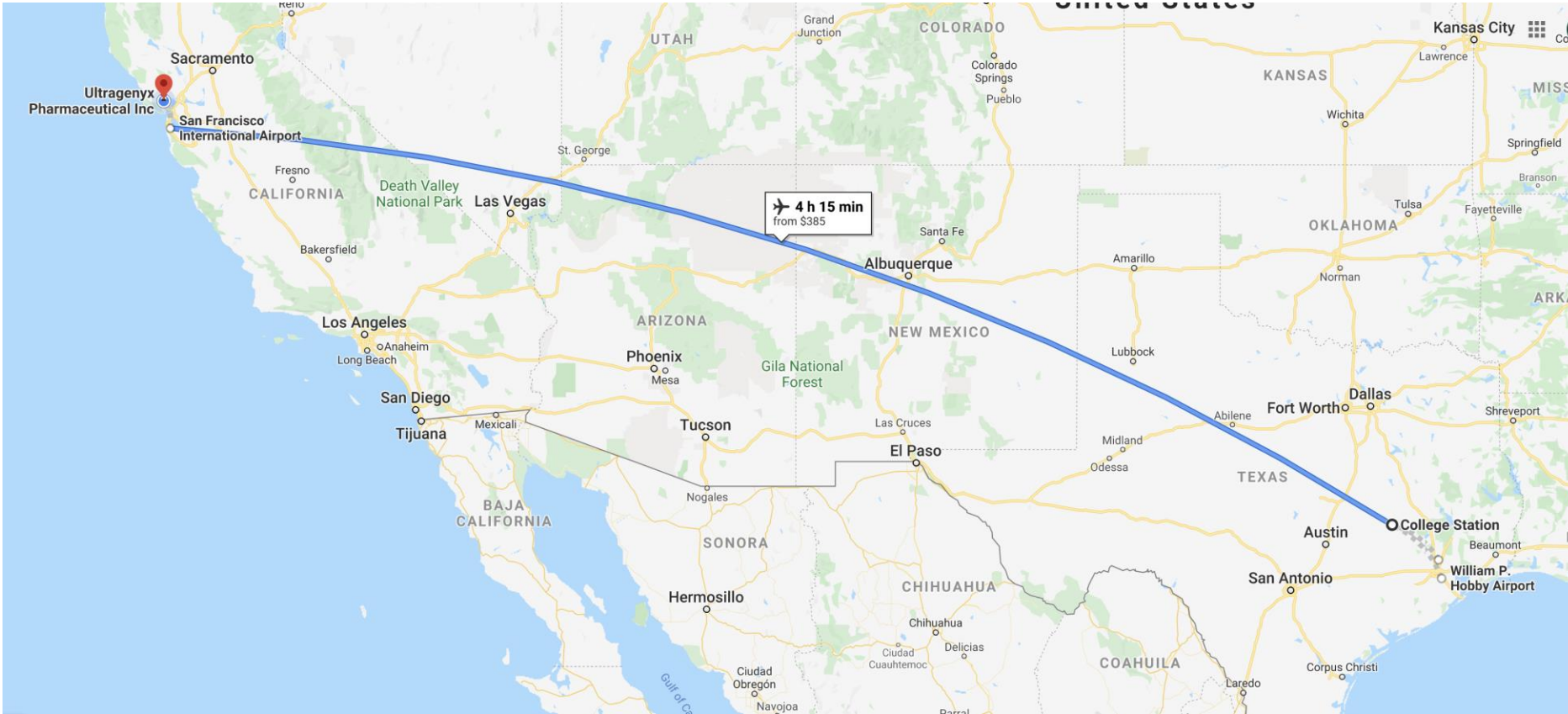
Contract



FINAL THOUGHTS

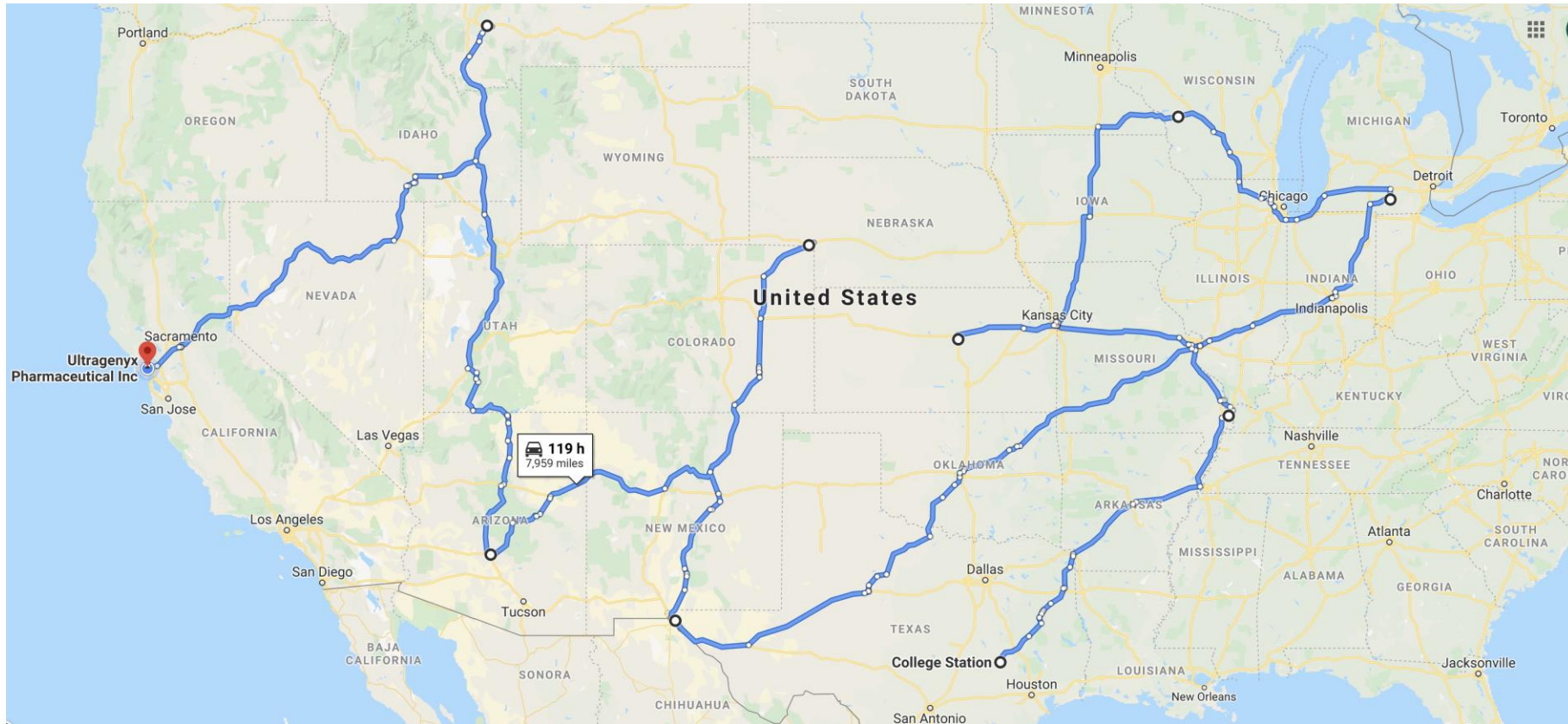
Your Journey with an Academic Collaborator

Expectation



Your Journey with an Academic Collaborator

Reality



Conclusions

- Academic research is the engine that drives innovation, but it is a unique and often misunderstood ecosystem.
- Find a collaborator who understands your objectives and timelines. Try to understand their objectives and timelines – every person and situation differs.
- Trust your academic collaborator, but do not make assumptions about their situation, environment, institution, etc. – find out as much as you can as early as possible.
- Be dubious about scientific publications, scientific claims, timelines, etc. — it is the nature of research to embellish and overpromise.
- Be focused. Expect twists and turns and failures and frustrations — they are inevitable.

Thank you!

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