

# SciOpen Research Group

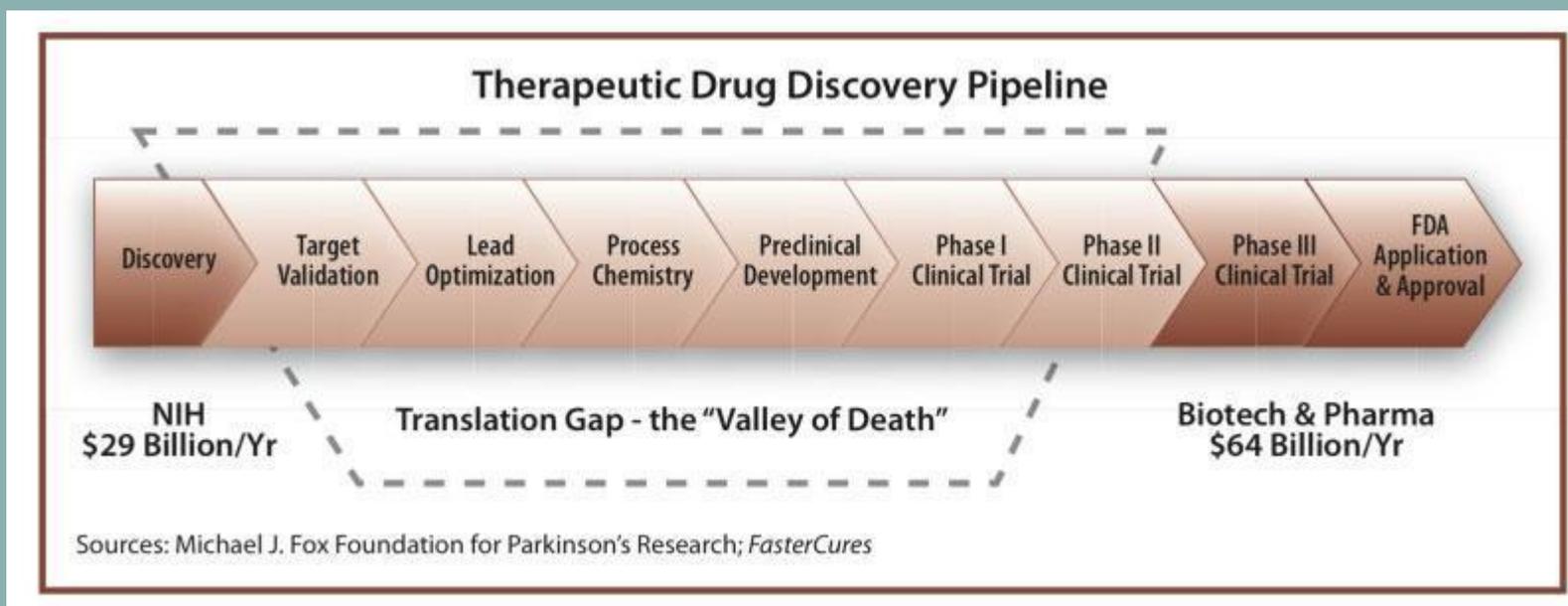
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## Introduction to SciOpen Research Group And Our Mission To End ALS

# Executive Summary

- ALS is a progressive neurodegenerative disease that afflicts 5 million people in the US annually
- Drug development for ALS has yet to see success and few corporations are prepared to invest in early-stage preclinical development with a relatively high perceived risk
- Many novel and interesting molecules lie on laboratory shelves while ALS patients die
- A leaner early research model is needed to de-risk development of these novel molecules
- SciOpen can identify interesting candidate molecules, organize their screening, and reduce the risk in a business model not dependent on generating profit

# The Valley Of Death



There is a lot of basic research, but it rarely crosses the "Valley of Death" into the clinic/bedside.

# ALS is Evading Success

## ALS is poorly understood

- ALS is a rapidly progressive neurodegenerative disease of the motor neurons that afflicts 5 million people in the US annually
- Up to 10% of ALS is inherited (familial)
- 90% of ALS is of unknown origin (sporadic)
- ALS is poorly understood and there are no truly validated targets for drug development

## Failures have dampened further investment

- BDNF
- Minocycline
- Creatine
- Ritonavir
- Celecoxib
- Ceftiaxone
- Dexamipexole

## Trial endpoints are controversial

- Function
- Strength
- Survival

## Broken model

- Pharma companies are reluctant to invest in ALS drug development due to the long string of failures
- When targets are uncertain and mechanisms poorly understood, risk outweighs potential return and capital flows elsewhere

# SciOpen is Stepping into the Market Failure

## New model

- Traditional research continues
- SciOpen will supplement it by identifying shelved molecules and efficiently screening them in the SOD1 mouse model – the industry standard ALS model
- SciOpen is a not for profit devoted to getting programs started
- The new model is the old model – test more molecules

## Founders

- Eric Valor – ALS patient and patient advocate. Originator of several DIY trials.
- Andrew Gengos – Biotechnology company executive
- Ryan Bethencourt – President at Berkeley Biolabs
- Joseph Jackson – Founder of Open Science Summit, cofounder of BioCurious



# Why SciOpen?

- For a fraction of the cost of big pharma, SciOpen can do initial screens of novel, interesting, and relevant molecules
- “Guerrilla Biotech” model attracts young and creative talent not utilized by big pharma
- We can move quickly and nimbly
- More shots on goal = more hits, more hits = more therapies for ALS
- Donations are tax-deductible, allowing patients and families a direct weapon in the fight against ALS
- Failure is irrelevant - we learn from every experiment and will keep trying until we succeed

# Thank You!

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