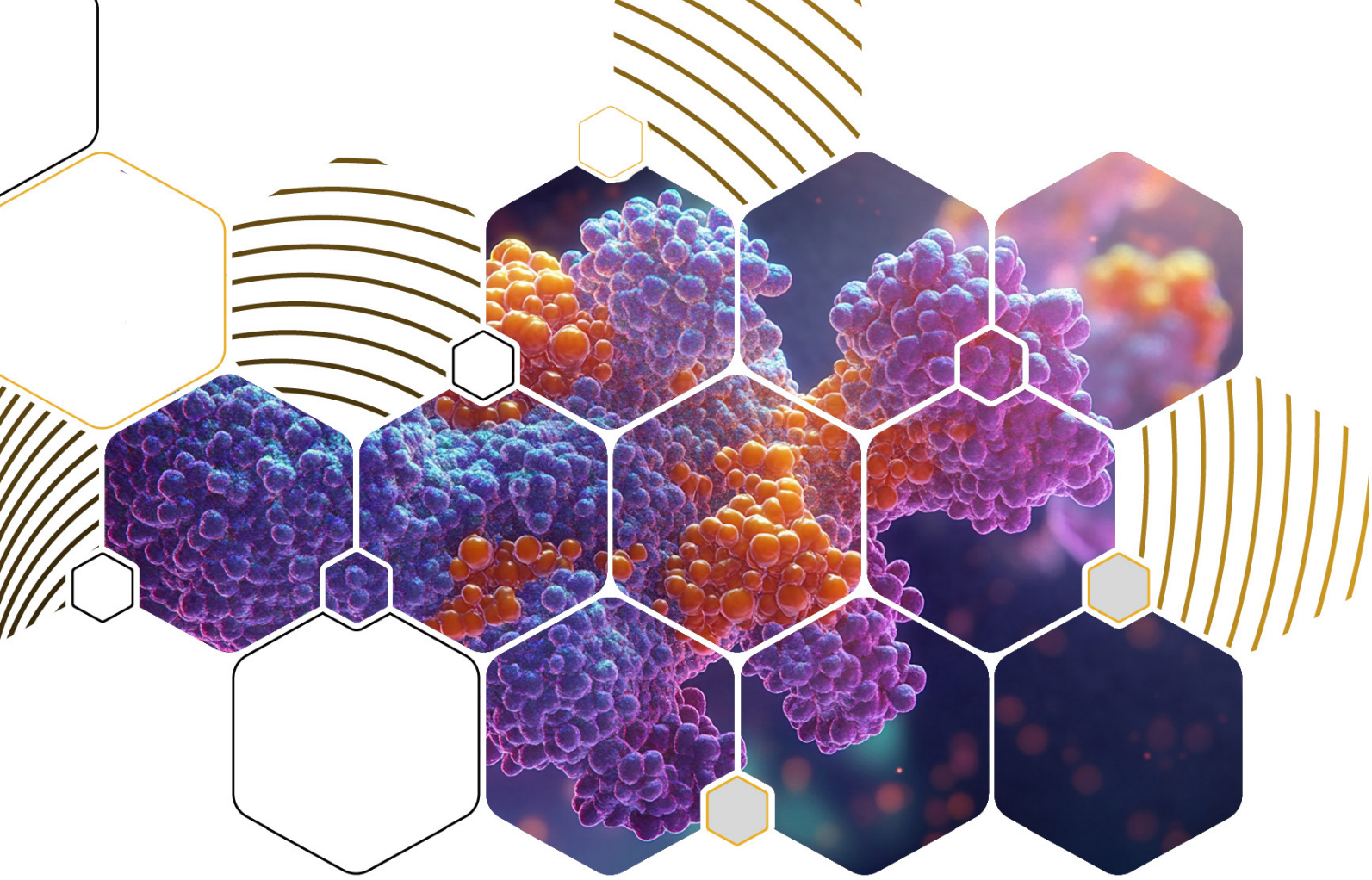

Collaboration Spotlight: UCSF

How UCSF Transformed Neurodegenerative Drug Development with Quantitative AI

SANDBOXAQ

Partner

University of California, San Francisco Institute for Neurodegenerative Diseases

Organization

The UCSF Institute for Neurodegenerative Diseases is developing diagnostics and therapeutics to address neurodegen diseases including Alzheimer's, Parkinson's and dementia.

Industry

Biopharma & Healthcare

Application Area

Drug Development

Neurodegenerative diseases such as Parkinson's and Alzheimer's have long resisted therapeutic breakthroughs.

Drug development in these areas remains one of the most difficult and costly undertakings in modern medicine: 1) tens of billions of dollars are spent on drug development, 2) timelines often span over a decade, and 3) there have been repeated failures to discover drugs that slow or stop the progression of disease. The traditional model is no longer sustainable, particularly in the face of urgent and growing patient needs.

A recent collaboration between the Institute for Neurodegenerative Diseases (IND) at UCSF and SandboxAQ signals a paradigm shift. Leveraging a new class of AI known as Large Quantitative Models (LQMs), UCSF has not only accelerated its research efforts but also redefined what is possible in the development of novel therapeutics. This case study highlights how SandboxAQ's AQBioSim platform is fundamentally transforming the drug discovery process, delivering unprecedented speed, efficiency, and success.

For years, the development of neurodegenerative therapies has been constrained by limited computational tools and inefficient screening techniques. Progress was slow and expensive, with researchers relying on brute-force screening of hundreds of thousands of compounds, typically yielding a handful of weak leads after months of work. Despite advances in automation and bioinformatics, the core bottlenecks in modeling and prediction remained.

UCSF and SandboxAQ's AI-Driven Collaboration

Under the leadership of Nobel laureate Dr. Stanley Prusiner, a team at UCSF confronted these same challenges. In 2024, the team projected that a promising Parkinson's therapy would not be ready for clinical trials until 2031. After exploring traditional computational platforms without success, Dr. Prusiner turned to SandboxAQ. He was searching for an AI solution that could do more than just augment productivity, he needed a platform that could materially de-risk and accelerate drug development.

> My life's work has been to investigate neurodegenerative diseases, including Alzheimer's and Parkinson's disorders. As my colleagues and I are focused on treatments for these illnesses, I was looking for AI software that could speed up our development time and de-risk the drug candidate before clinical trials. We found SandboxAQ and their software has been transformative for our work.
>
> ~~Dr. Stanley Prusiner~~

The team used a powerful combination of technologies and resources: cutting-edge cryo-electron microscopy (cryo-EM) to resolve the 3D structures of protein—small molecule complexes, privileged access to human brain tissue for testing, and SandboxAQ's groundbreaking software optimized explicitly for modeling prion proteins in their pathogenic conformations.

How AQBioSim Achieved the Impossible: The Power of LQMs

The core of AQBioSim's power lies in its use of Large Quantitative Models (LQMs). Unlike Large Language Models, which learn from text data, LQMs are trained on first-principles equations rooted in math, chemistry, biology, and physics. This core distinction enables LQMs to rapidly and accurately predict the behavior of molecules and biological systems with unparalleled precision. Through LQM-powered simulations, researchers gain deep insights into molecular interactions with unprecedented detail.

According to Dr. Prusiner, the shift was transformative. "Prior to SandboxAQ, we were only able to explore 250,000 compounds, which yielded a limited number of hits over the course of a year. With SandboxAQ's platform, we increased the chemical exploration space from 250,000 molecules to 5.6 million. We identified candidate molecules faster and more efficiently with a hit rate 30 times greater. As a scientist, I am deeply impressed with the technical depth and impact of SandboxAQ's AI technology."

Here is a comparison of the impact of AQBioSim versus traditional screening at UCSF:

Metric	Traditional Approach	With AQBioSim	Improvement
Molecule Library Size	250,000	5.6 Million	**22x Increase**
Phenotype Screening	250,000 Tested	7,000 Tested	**36x Cheaper**
Hit-Rate	0.01%	0.34%	**>30x Increase**
Screening Time	1 Year	1 Month	**>10x Faster**

Compared to conventional methods, AQBioSim delivered significantly better results, more than 10 times faster, while exploring a much broader chemical space. This shows that an AI-driven LQM approach dramatically improves both the speed and probability of success in developing new drugs.

Strategic Implications for Big Pharma

This success at UCSF is not an isolated fortunate result. It indicates the broader impact LQMs will have across biopharma, chemicals, energy, and materials science. For drug development executives, this signals a critical shift:

- **From Trial-and-Error to Drug Design:** LQMs enable a move away from slow, costly lab experimentation towards highly targeted, AI-powered drug design.
- **Accelerated Development Cycles:** Significantly reduce the time from discovery to clinical trials, bringing life-saving therapies to patients faster.
- **Enhanced R&D Efficiency:** Drastically lower screening costs and improve the probability of identifying viable drug candidates, optimizing resource allocation.
- **Unlocking New Chemical Space:** Explore and identify promising molecules from libraries orders of magnitude larger than previously feasible.
- **Value Creation:** LQMs deliver value creation by solving quantitative challenges previously deemed unsolvable, directly impacting the bottom line through accelerated and more successful drug development.

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>
> **Dr. Stanley Prusiner,** Director of the Institute for Neurodegenerative Diseases at UCSF

The Future of Drug Discovery is Here

Large Quantitative Models represent the next evolution of AI tools that will radically accelerate drug discovery and new product development while lowering costs. The UCSF-SandboxAQ collaboration demonstrates that the right AI, when applied correctly, can do more than assist drug developers. It can redefine the economics and timelines of the entire drug development lifecycle.

For pharma companies, LQMs represent a unique opportunity to redefine what's possible in drug discovery, accelerate the development of safe and more effective treatments, and ultimately lead the charge in solving some of humanity's most pressing health challenges. The chance to dramatically improve R&D outcomes and deliver breakthrough therapies faster is no longer a distant dream — it's a reality today.