
# Emerging Value and Pitfalls of Large Language Models in Drug Development

Chairs:

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# Disclosures

**Anzar Abbas:**
Employee, hold shares in Brooklyn Health

**Amir Kalali:**
Co-Chairman, Digital Trials and Research Alliance (DTRA)
Founding Chairman of the International Society of CNS Drug Development (ISCDD)
Chairman and Chief Curator of CNS Summit
Advisor to Brooklyn Health


# Introduction to the session

Anzar Abbas, PhD

# What do we mean by large language models

## The technical definition

Artificial intelligence, a broad term that can mean lots of things

↳ Machine learning, use of statistical methods to enable machines to learn from data

↳ Deep learning, a subset of ML based on artificial neural networks

↳ Generative AI, an application of deep learning designed to create new content

↳ **Large language models**, a specific type of GenAI focused on text and language

A Large Language Model (LLM) is a massive-scale, deep learning architecture—almost always based on the Transformer—trained via self-supervised learning to model the probability distribution of sequences of tokens.

At its core, an LLM is a function $f(x) = y$ where $x$ is a sequence of numerical representations (vectors) and $y$ is a probability distribution over a known vocabulary, used to predict the next most likely token in a sequence.

# What do we mean by large language models

## A non-technical definition

Models that predict the next word in a sequence based on probability

**The cat sat on the _____ .**

a) Mat ← 90%

b) Hat ← 10%

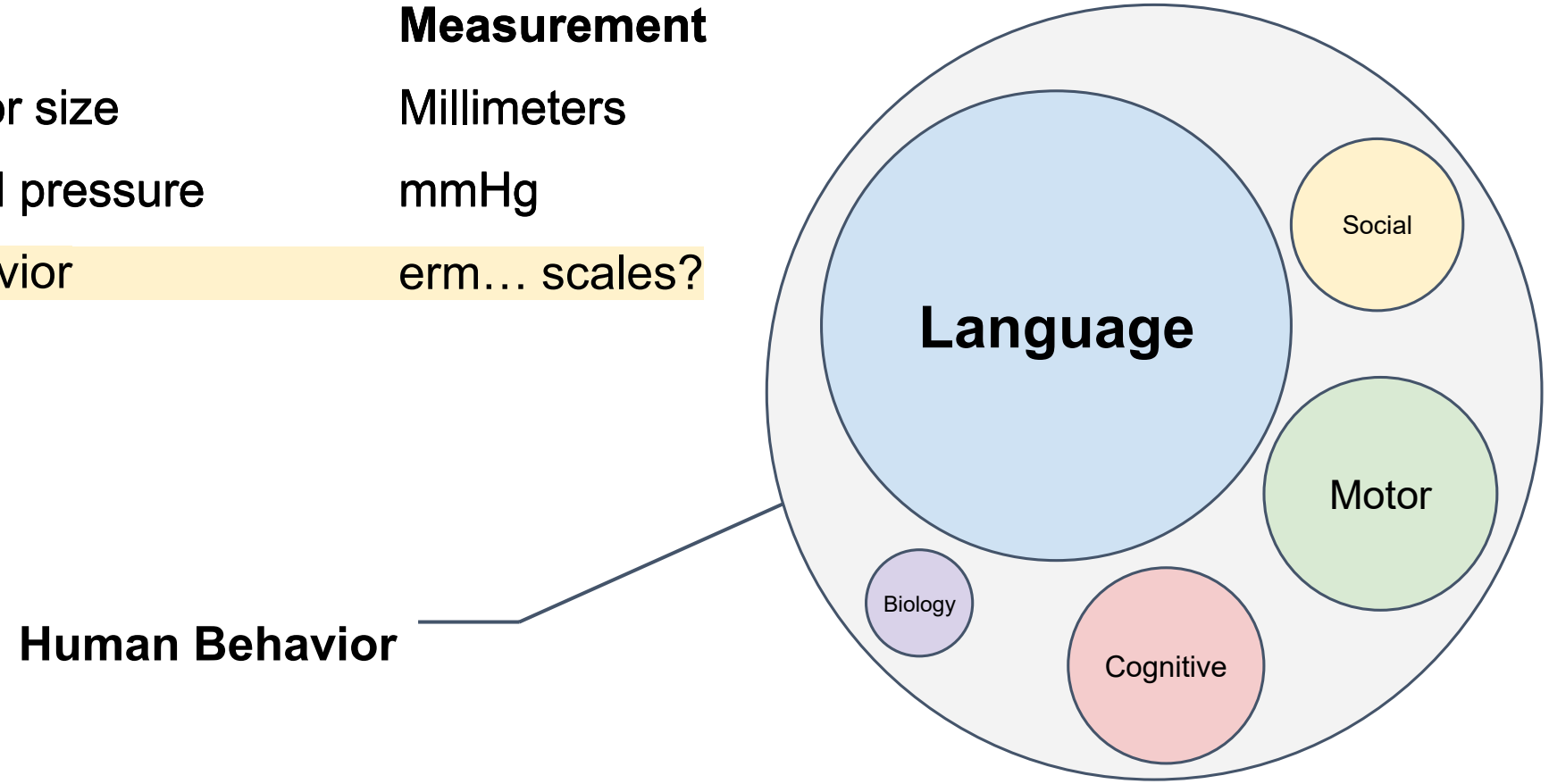
c) Banana ← 0%

LLMs do this on a massive scale, being familiar with content used in their training, including medical literature, predicting words based on learnings during training.

# Why this matters for CNS drug development

Measurement of unstructured data

| Field | Data | Measurement |
|---|---|---|
| Oncology | Tumor size | Millimeters |
| Cardiology | Blood pressure | mmHg |
| Psychiatry | Behavior | erm… scales? |

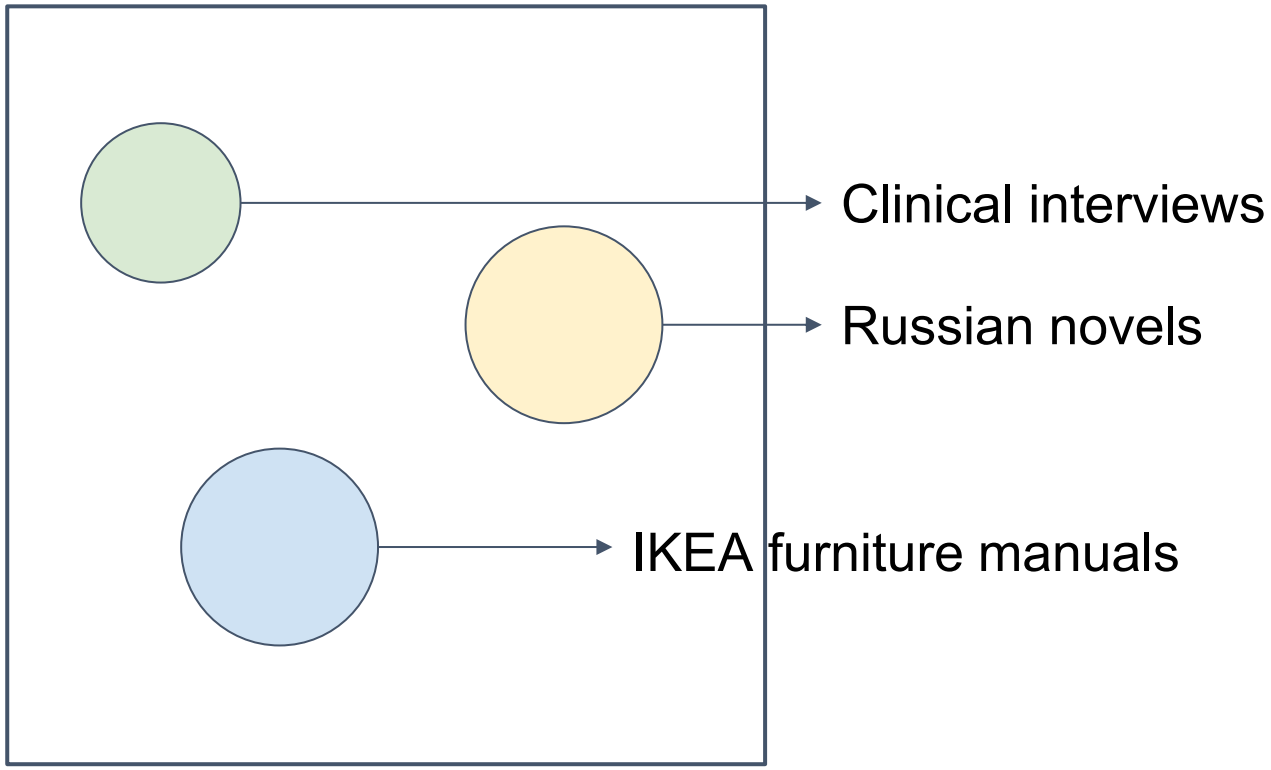


**For the first time, we have a tool that treats *language* as the data point.**

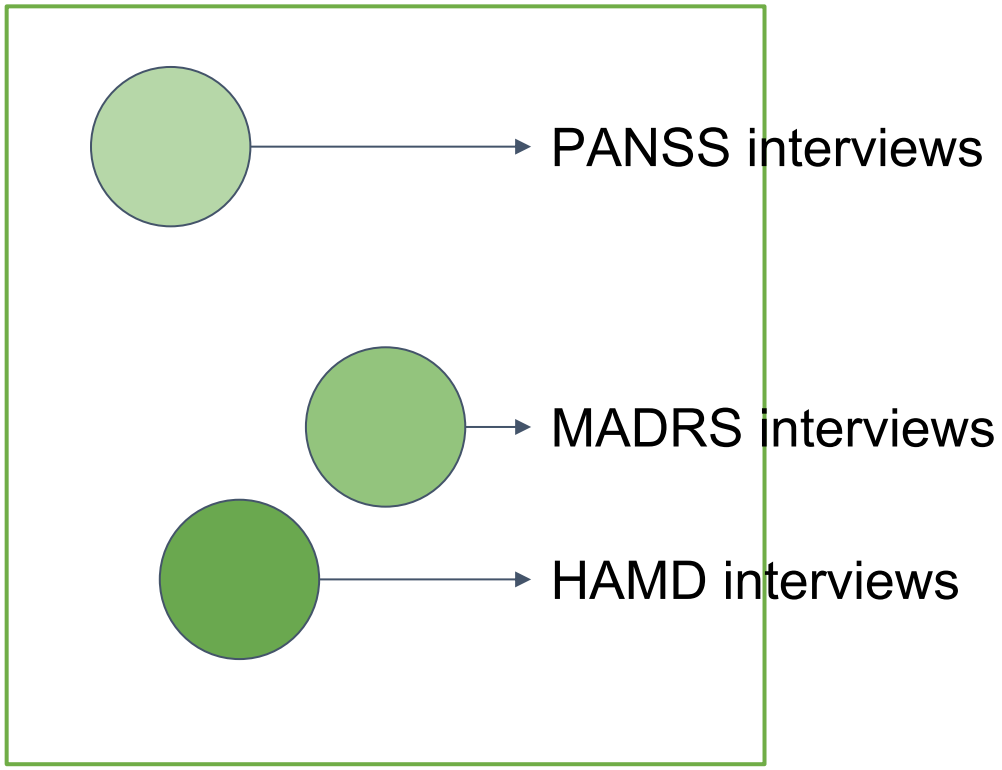
# Specializing LLMs to a use case

Increasing how well we can differentiate between content in an embedding space

General embedding space




Fine-tuned embedding space



# Back to the session!

# How can we use LLMs in drug development *methodology*

All focused on potential for utility in drug development

- **Todd Solomon:** Using LLMs to re-score HAM-A clinical interviews, use the scoring as a triaging mechanism to send interviews for manual review, improve efficiency, increase confidence in data
- **Atul Mahabaleshwarkar:** Using LLMs to monitor dosing sessions, a task not realistic / tenable with traditional methods; LLMs enabling us to do something we simply couldn't do before
- **Jeff Cochran:** Using LLMs to analyze drug response in schizophrenia trials with greater sensitivity than previously possible; deriving additional functional domains from the PANSS clinical interviews

The talks all have regulatory implications – so we're lucky to have **Ruihao Huang** and **Valentina Mantua** with us to give us their general perspectives as well as comment on the talks themselves.

Plus some more bonus content if we have the time…

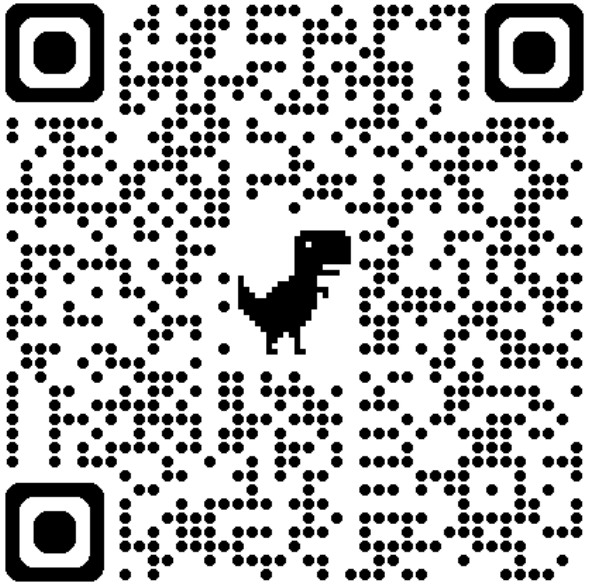
# Some housekeeping!

## Each talk is 20 minutes, followed by 10 minutes of questions

Two options to submit your questions:

1. **Come up to the mic**
   Pros: You can talk for a few minutes until we yell at you for not getting to your question sooner
   Cons: Unfair advantage to the bold

2. **Ask your question using the link:**
   Pros: No social anxiety, merit based, can submit anonymously
   Cons: The chairs will decide whether your question gets brought up



# Working group on the same topic *tomorrow!*

To continue the conversation and discuss future efforts

- Continue the discussions started today
- Expand the list of use cases discussed

It’s the breakfast working group 🫠 **7:45am sharp!**

# Speakers

Jeff Cochran, PhD
Ruihao Huang, PhD
Atul Mahableshwarkar, MD
Valentina Mantua, MD, PhD
Todd Solomon, PhD
Anzar Abbas, PhD
Amir Kalali, MD