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EXPERT INSIGHTS

How Artificial Intelligence is Reshaping Drug Development

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How Artificial Intelligence is Reshaping Drug Development

Executive Summary

In the challenging landscape of drug development, where bringing a medication to market has gotten slower and costlier over time, artificial intelligence (AI) solutions are gaining traction. This issue, coupled with escalating public and political calls for more affordable medications, has prompted a surge in AI-based initiatives aimed at reducing costs and expediting development timelines. Additional momentum behind AI adoption in drug development is being fueled by advancements in AI technology, heightened awareness, and improved computer processing speed.

Estimates point to a substantial total addressable market of around $50 billion for AI-enabled drug development, with the expectation that 30% of new drugs will be discovered using AI by 2025. Notably, the field is witnessing a proliferation of players, categorized into biopharma companies with a robust AI focus and providers of AI tools working in tandem with large biopharma players. Meanwhile, collaboration is on the rise with big tech companies like Nvidia, Alphabet and Amazon.

In clinical trials, AI-derived drug candidates are gaining traction, while future AI applications are expected to expand into challenging areas such as biologics, oncology, and rare diseases.

Experts are enthusiastic about the big picture while acknowledging that AI-based drug development is still in an early and uncertain stage, and offered varied opinions on its progress and challenges. Let's explore this topic and the key industry debates by leveraging AlphaSense, including first-person insights from our expert transcript library.

A Ripe Time for AI Solutions

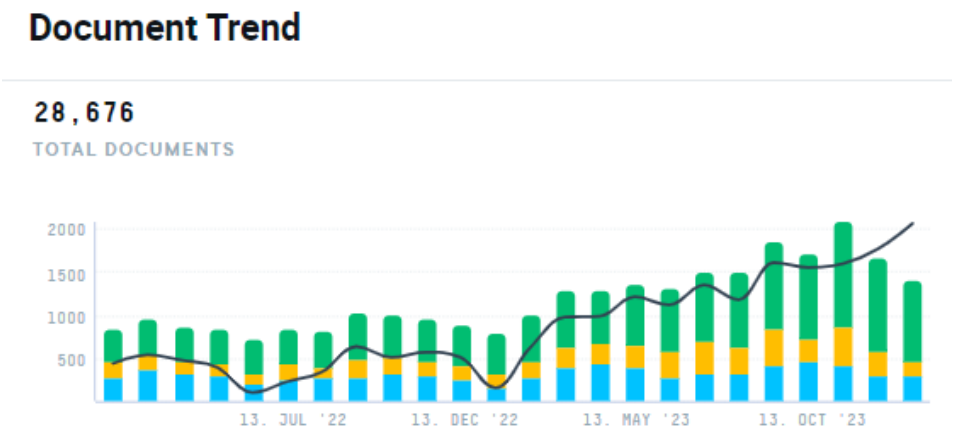
Drug development is an exceedingly time-consuming and expensive process. On average, it takes more than 12 years and $1.3 billion to bring just one drug to market. Factor in the high rate of drug candidates that never make it to market at all, mainly because of clinical trial failures (roughly 90%) and the average cost per approved drug balloons to $2.6 billion.

Against this backdrop, public and political demands for more affordable medications are mounting. It's no wonder drug makers are racing to find AI-based solutions aimed at reducing development costs and shortening lead times.

Also fueling the trend are AI technology advances and growing AI awareness, according to AlphaSense experts, as well as improved computer processing speed. Another driver is the recent explosion in the size and volume of data sets that feed AI models. AI models are aggregating data collected from diverse sources such as electronic health records, clinical trials, genomic sequencing, prescriptions, insurance claims, and remote monitoring devices like fitness trackers, pacemakers and glucose monitors.

> "As the cost of clinical trials continues to go out of sight, and [there is] pressure on drugs to come down in cost, this [AI drug discovery] stuff will just continue. It makes sense; you have all this data, why not analyze it versus trying to run an experiment yourself?"
>
> "Using computer-driven algorithms to enhance drug design is not new. This has been going on for decades. What's different now?…the emergence of [ChatGPT] and things like this that are now accessible to almost anybody with a computer. This, I think, captured the imagination [of] a much broader swath of people. On the back of that is the fact that the computer processors are much faster."
>
> **– Former Senior Vice President, Biotech Company | Expert Transcript**

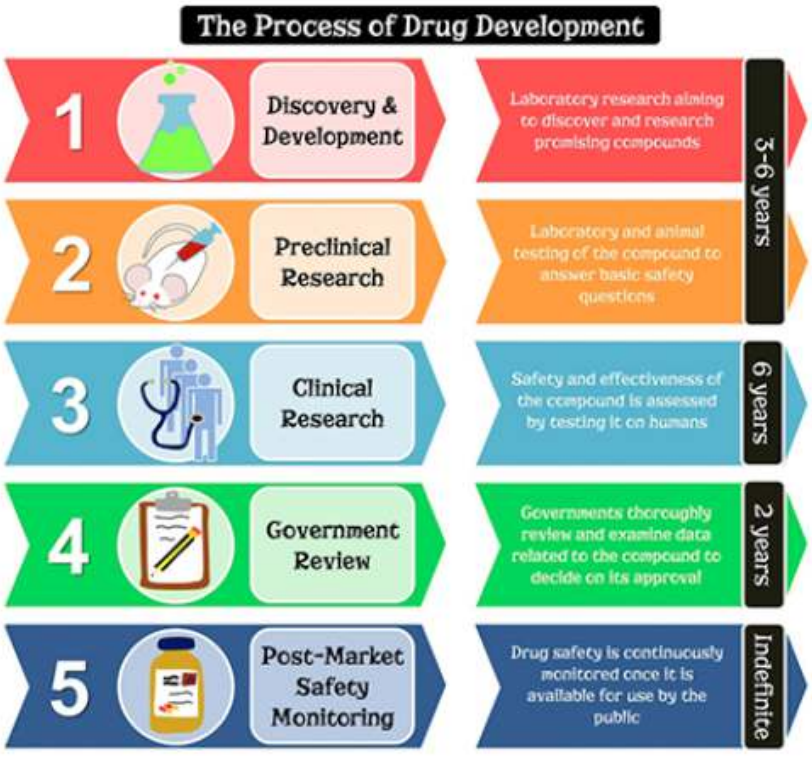
Drug companies' enthusiasm for AI-based solutions is reflected in the AlphaSense research platform, where documents mentioning AI in drug development have surged during the past 12 months.



Source: [AlphaSense]

Drug Development 101

Drug development is the process by which new medications are identified, designed, and developed for treating or preventing diseases. Here's a quick overview of how drug development works:

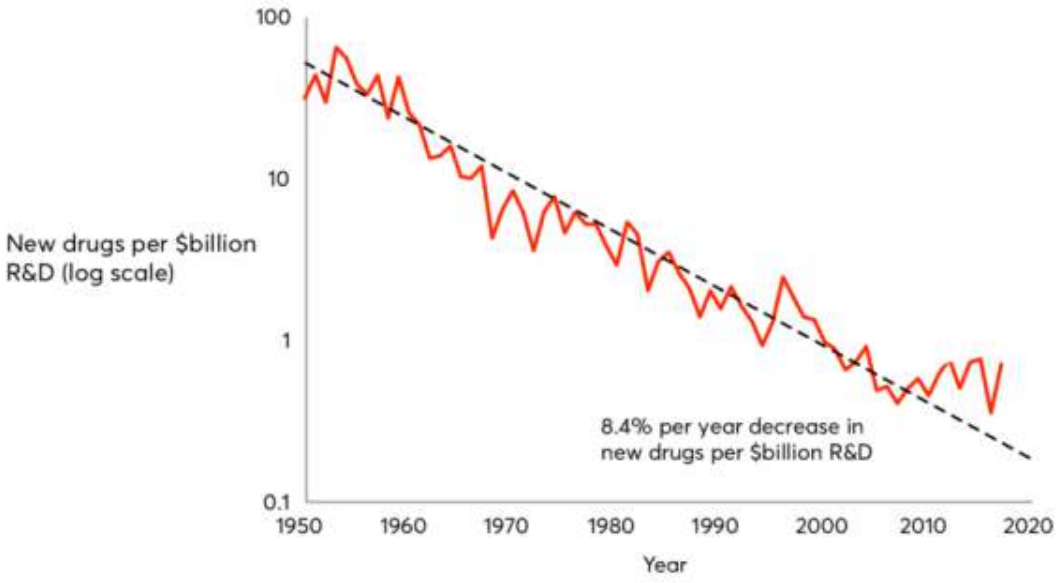


Source: [The University of Texas]

However, the traditional drug development process has several drawbacks, which we explore in greater detail in the following section. These drawbacks have contributed to a confounding phenomenon whereby drug development has gotten slower and costlier over time despite technological improvements and increased investment in research and development (R&D).

According to Eroom's Law (Moore's Law spelled backwards), the number of new drugs approved per $1B spent on R&D has halved roughly every nine years since 1950.

Eroom's Law: New Drugs Per $1B in R&D Spend Halves Every ~9 Years



Source: Nature Reviews Drug Discovery

Challenges with Traditional Methods Equate to AI Opportunities

AI refers to the simulation of human intelligence in machines that are programmed to think, learn, and problem-solve like humans. The goal of AI is to develop systems that can perform, enhance, and augment tasks that typically require human intelligence, such as visual perception, speech recognition, decision-making, and language translation.

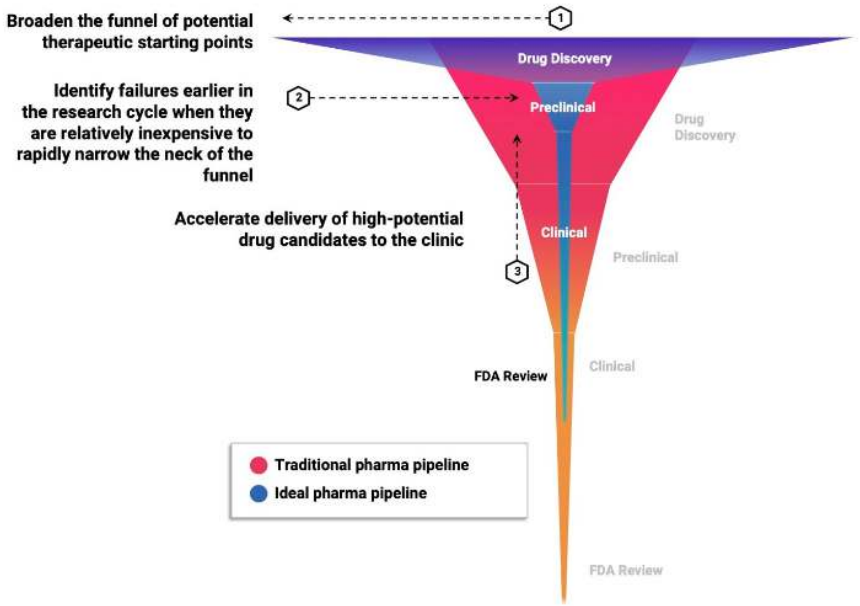
Drug development is attracting a disproportionate share of AI investment dollars flowing into the healthcare sector because of its compelling return on investment. Estimates are scarce, but a BCG report released last year said that AI could yield time and cost savings of at least 25%–50% in the drug discovery steps leading up to the preclinical stage.

> [Drug] discovery is where the biggest investment is being made at the moment… In discovery, anything that can [drive] improvement could have dramatic consequences on the return of investment downstream, the net present value, for example. It's more or less understandable that the most [AI investment] is there."
>
> **– Associate Director, Pharmaceutical Company | Expert Transcript**

In comparison to traditional methods, AI-enabled drug development promises to expand the number of potential therapeutic starting points, while identifying failures earlier in the research process and accelerating the pace of bringing drugs to market.

> As a grim reaper, [AI] is really good. It can catch, let's say, possible toxicology effects so that you can kill a potential molecule much earlier. Does it increase the probability of technical success? No. What it does is it reduces the aggregate cost. You will still need 22 projects to get one [candidate], but you will be able to kill the [first] 21 much earlier."
>
> **– Associate Director, Pharmaceutical Company | Expert Transcript**

AI-Enabled vs. Traditional Drug Development



Source: Recursion Pharmaceuticals

Below, we explore some of the specific issues AI addresses in the drug development process.

Identifying Drug Targets and Candidates

Issue: Traditional methods are mostly based on singular hypothesis-driven approaches and are limited by human capacity to analyze multi-dimensional data and prone to human error and bias.

Solution: AI takes a more holistic approach by analyzing vast and diverse datasets to expedite and improve the accuracy of identifying drug targets and candidates.

> “AI and ML and analytics, basically these are ways to really take vast amounts of disparate data, and when queried in the correct way, can provide some insights that you just can’t do single-handedly in the human mind.”
>
> **– Former Managing Director, Biotech Company | Expert Transcript**

Preclinical Testing

Issue: Predicting the safety and efficacy of drug candidates in preclinical testing is a significant challenge and dependent on trial-and-error in the lab.

Solution: AI uses predictive tools that can help reduce experimental failures and animal testing requirements.

> “Nobody wants to kill millions of animals through high-throughput in vivo [experiments]... There is a huge market opportunity [for AI] there.”
>
> **– Associate Director, Pharmaceutical Company | Expert Transcript**

Clinical Trial Design and Patient Recruitment

Issue: Designing efficient clinical trials with suitable patients can be challenging. Clinical trials often involve relatively small and homogeneous patient populations.

Solution: AI assists in optimizing trial design, predicting patient responses, and improving patient recruitment. It also analyzes patient data from real-world sources across a wider demographic, which has become increasingly important for regulatory approval according to FDA guidance issued last year.

> [A] key opportunity [for AI] is the optimization of clinical trials, optimization of clinical trial design, optimization of the numbers of subjects, optimization of the patient selection, patient selection criteria... That's a huge area of opportunity."
>
> **– Former Senior Vice President, Biotech Company | Expert Transcript**

Personalized Medicine

Issue: Identifying patient subgroups that respond differently to treatments is critical for personalized medicine.

Solution: AI analyzes patient data, including genetic information, to identify biomarkers and stratify patients based on their likelihood of responding to specific treatments.

Natural Language Processing (NLP) for Literature Mining

Issue: Keeping up with the vast and constantly evolving scientific literature is difficult.

Solution: AI, specifically NLP, helps researchers extract relevant information from literature, patents, and clinical trial reports to aid decision-making.

Drug Repurposing

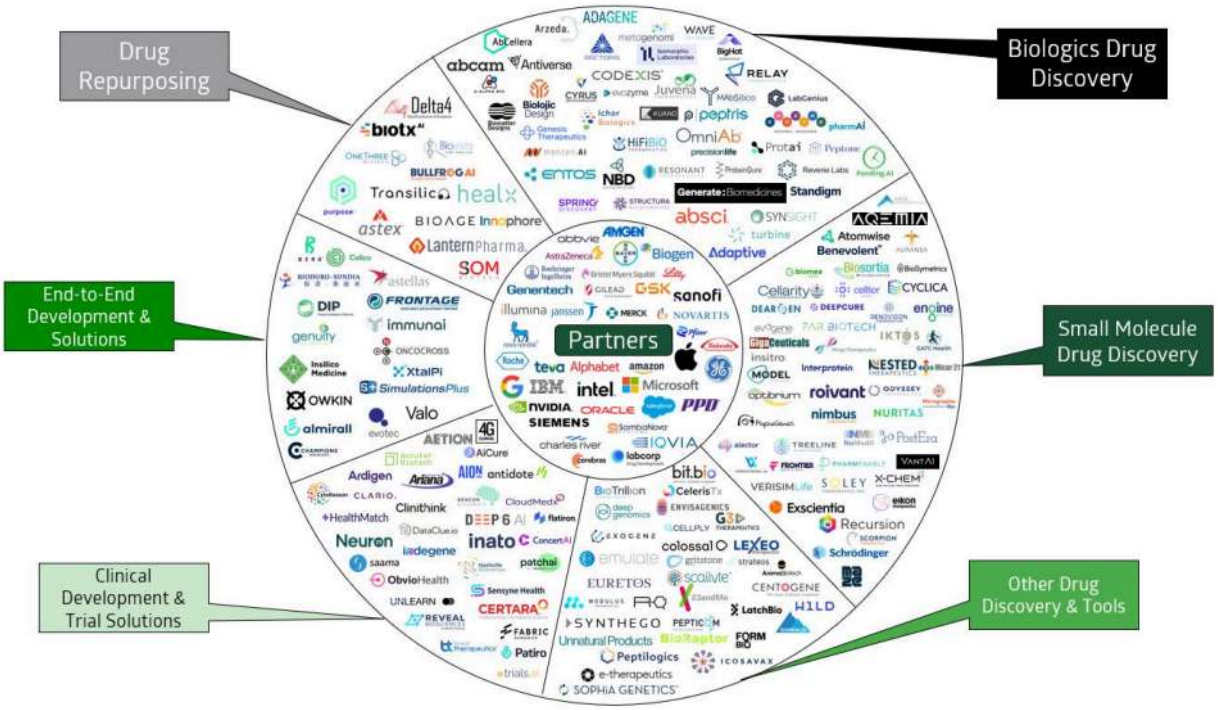
Issue: Finding new uses for existing drugs is challenging.

Solution: AI can analyze large datasets to identify potential connections between drugs and diseases, allowing drugs to be repurposed.

Market Opportunity Attracting Plethora of Players

Estimates of the total addressable market (TAM) for AI-enabled drug development are scarce, though TD Cowen and Morgan Stanley both approximate the TAM at ~$50B. An estimated 30% of new drugs are expected to be discovered using AI technology by 2025, up from zero today, and AI is expected to lead to 50 novel therapies over a 10-year period.

AI Drug Development Universe



Source: TD Cowen

The field of players looking to capitalize on AI drug development trends is plentiful and falls into two main categories: biopharma companies with a strong AI focus and providers of AI tools.

Biopharma Companies with a Strong AI Focus

These companies use internal databases, algorithms, and computing power to discover drug candidates and move them into clinical trials. In addition, this group often depends on external partners to provide AI tools or applications.

> “Merck and some of these larger companies are well-positioned to use treasure troves of their own internal data repositories, high-quality data to fish out certain patterns in that data across whether it's chemistry or pharmacokinetics or safety talks.”
>
> **– Senior Scientist, AI Tools Provider | Expert Transcript**

Providers of AI Tools

These companies serve as partners and collaborators with biopharma players by offering vast databases for feeding AI models, deep AI expertise, or AI platforms that can be applied across a range of products and therapeutic areas.

Collaborations on the Rise Among Big Pharma and AI Tools Providers

High Profile Drug Discovery Collaborations in Big Pharma

Announced Date	Pharma Partner	Collaborator	Deal Value ($M)	# of Programs
Sep-20	Bayer	RECURSION	$1,000	10
Oct-20	Bristol Myers Squibb	insitro	$2,000	N/A
May-21	Bristol Myers Squibb	Exscientia	$1,200	N/A
Nov-21	sanofi	OWKIN	$270	N/A
Dec-21	Roche Genentech	RECURSION	$12,000	40
Jan-22	AMGEN	Generate:Biomedicines	$1,900	10
Jan-22	MERCK	absci	$610	3
Jan-22	sanofi	Exscientia	$5,200	15
Jan-22	Lilly	evotec	$1,000	5
May-22	Bristol Myers Squibb	evotec	$5,000	N/A
Apr-22	Takeda		$400	4
Aug-22	sanofi	Atomwise	$1,200	5
Oct-22	Bristol Myers Squibb	SyntheX	$550	N/A
Nov-22	sanofi	Insilico Medicine	$1,200	6
Jan-23	janssen	evotec	$350	N/A
Mar-23	Bristol Myers Squibb	evotec	$4,000	N/A
May-23	Boehringer Ingelheim	GINKGO BIOWORKS	$406	N/A
Jun-23	Lilly	XtalPi	$250	N/A
Sep-23	AstraZeneca	VERGE GENOMICS	$840	N/A
Nov-23	Bayer	RECURSION	$1,500	7
Nov-23	almirall	absci	$664	2
Total Deal Value			$41,540	

Source: [TD Cowen]

Experts believe AI tools providers that have the most established relationships with large biopharma companies are best positioned in the market.

> A number of companies that have started out like Recursion, Exscientia, BenevolentAI, Owkin, maybe they're five-, six-, seven-years old, they've really started to come into their own now and get the credibility with the pharma companies that they're trying to partner with. They're actually delivering now in terms of the promise of the application of advanced analytics."
>
> "What I'm just starting to see is that people are leaving the pharma companies and joining these [AI tools provider] companies. It's been really important to get experienced drug developers in these AI analytics companies, and so that's happening now."
>
> **– Former Managing Director, Biotech Company | Expert Transcript**

One expert said providers of AI tools that offer a combination of proprietary data and AI capabilities are particularly well-positioned:

> You have [a] category of companies that are hybrid. They are generating their own data and they're also having AI/ML capabilities. Now, these are the most interesting ones… companies… like Recursion, companies like Insitro who have understood [that] the biggest differentiator in the world of AI is not really the technology itself, but rather the data. There are not that many of those [companies], but you see that the market is moving in this direction."
>
> **– Associate Director, Pharmaceutical Company | Expert Transcript**

Other experts said no clear winners among the AI tools providers have distinguished themselves:

> It's a Wild West right now… In my opinion, no one's emerged. There are definitely some [companies] that are more notable than others. Some have received large investment rounds and things of that nature that help to raise their profile. I'm not really convinced yet there's a single company or even type of company that's really emerged as the go-to source or a go-to source."
>
> **– Former Senior Vice President, Biotech Company | Expert Transcript**

Meanwhile, big tech players such as Nvidia, Alphabet, Amazon, and Microsoft are expanding into biopharma to provide the underlying computing power and tools to enable AI drug development at scale.

Clinical Pipeline Reflects AI Momentum

As of last year, there were 15 AI-derived drug candidates in clinical trials, and the pipeline of AI-derived assets continues to expand. One company in particular has been garnering attention–Hong Kong-based Insilico Medicine–for making the first drug entirely based on AI.

Insilico Medicine developed INSO18-055 for the treatment of idiopathic pulmonary fibrosis (IPF) with the first AI-discovered molecule that was based on a novel AI-discovered target.

> “Insilico Medicine has [been] democratizing their own internal platform, which I think is really cool. They're essentially saying, ‘We've developed these algorithms, the generative algorithm to identify new molecules and also our predictive models, and we're making those models and algorithms available to you as a fee-for-service.’ They're using that internally for a set of targets and then also partnering with large- and medium-sized pharma companies.”
>
> **– Senior Scientist, AI Tools Provider | Expert Transcript**

Notably, Insilico’s drug was selected as a preclinical candidate in February 2021–less than 18 months after the project began–and announced its first Phase I trial just nine months later.

Altogether, it took INSO18-055 under 30 months to progress to Phase 1–about half the time it takes in traditional drug discovery. In doing so, INSO18-055 was the first fully derived AI drug to be tested in human trials. INSO18-055 has since entered Phase II clinical trials, and, if successful, the drug will proceed to further studies with larger cohorts, potentially leading to Phase III trials.

Focus Increasing in Challenging Areas

AI applications in drug development are expected to expand into areas posing particularly complex problems, including biologics, oncology, and rare diseases.

Biologics

So far, the majority of AI-based drug development has been focused on small-molecule drugs, which are chemical-based, have simple structures, and make up the majority of the pharmaceuticals market (~90% of global pharmaceutical sales in 2021). But in recent years, drug development has been moving toward large-molecule drugs, or biologics, which are derived from living organisms such as proteins or antibodies.

Biologics offer greater targetability and less toxicity than small molecules, and have facilitated personalized medicines such as CAR-T cell therapy for cancer. Yet biologics have complex

structures, which makes developing them both difficult and expensive. On average, the daily dose of a biologic costs roughly 22 times more than that of a small molecule.

AI lends itself to the complexities of biologics because it is able to rapidly analyze large-scale data sets, predict protein structures, and design new molecules. Consequently, there is a growing list of AI-derived biologics projects in the drug development pipeline.

> “There is tremendously promising work in generating protein sequences for biologics that will hopefully start delivering products within maybe [the] next five years or so.”
>
> **– Former Vice President, AI Tools Provider | Expert Transcript**

Furthermore, there are now 80 or so AI-focused companies working in the biologics arena, most of which were founded within the past seven years.

Companies Working in AI-Driven Development of Biologics

Founding year	Companies identified
Pre-2012	AbSci, Arzeda, Evaxion Biotech, iBio, Meridigen, Pepticom, Schrödinger, Second Genome, Syntekabio, ZielBio
2012	AbCellera, Exscientia, G3 Therapeutics, LabGenius
2013	Molcure, Peptilogics
2014	Anima Biotech, Deep Genomics, Resonant Therapeutics, Serotiny, XtalPi
2015	Ardigen, Orionis Biosciences, SEngine Precision Medicine, Systems Oncology
2016	Arbor Biotechnologies, Kintai Therapeutics, Unnatural Products
2017	3T Biosciences, A-Alpha Bio, Abalone Bio, Antiverse, Arontier, Gatehouse Bio, MAbSilico, METiS Therapeutics, Modulus, Neon Biotechnology, Polymaths AI, ProteinQure, RubrYc Therapeutics (acquired), VeriSIM Life
2018	1910 Genetics, Celsius (Biotechnology), Character Biosciences, deepCDR, Dyno Therapeutics, Erasca, Generate Biomedicines, Menten AI, neoX Biotech, Soley Therapeutics, Sparx Therapeutics
2019	Basecamp Research, BigHat Biosciences, Bio Simulytics, Denovo Sciences, Etcembly, Evozyne, Pharm CADD, Ordaos, Stellanova Therapeutics, Valo
2020	Athae Bio, Fresh Wind Biotechnologies, Known Medicine, Nabla Bio, NextPoint (Drug Discovery), Outpace Bio, Patch Biosciences, POLARISqb, Primary Biotech, Promatix, ROME Therapeutics
2021	20n Bio, Ainnocence, Atomic AI, Cradle Bio, Gandeeva Therapeutics, Huashen Zhiyao, Protai
2022	Profluent Bio

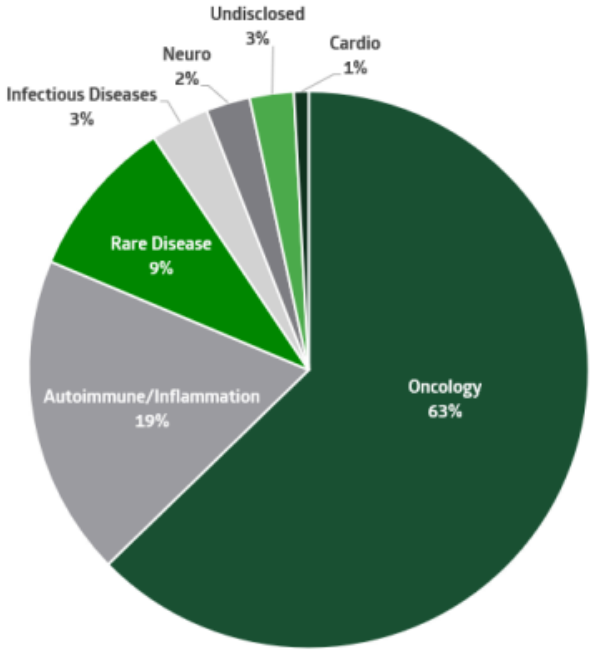
Source: Nature

Oncology

In terms of therapeutic areas, AI is expected to continue to focus its powerful capabilities on difficult-to-treat disease types, with oncology at the top of the list. Oncology is attractive not only

because of its complexity, but also because of the prevalence of cancer patients and, in turn, patient data to feed AI models.

Indications of Focus Across Active AI-Enabled Clinical Trials



Source: TD Cowen

Rare Diseases

On the other hand, rare diseases do not have large commercial markets, yet AI-based drug development is also trending toward rare disease. This is explained by regulatory incentives, such as orphan drug designations, which have encouraged pharmaceutical companies to invest in treatments for rare diseases. But AI's ability to overcome unique challenges in rare diseases is also a factor.

These challenges include a dearth of patient data as well as experts with sufficient knowledge and experience to run trials, both owing to the fact that rare disease patient populations are small and variable. In the United States, rare disease is defined as a disease or condition that affects less than 200,000 people.

In addition, rare diseases most commonly develop in early childhood. Conducting clinical trials in children is complicated not only due to ethical questions but also due to variability in children's physiology and the ways in which drugs act in their bodies.

AI can help bridge these gaps by mining existing drug databases for candidates, which can then be repurposed for rare diseases. Repurposing this data also has the benefit of reducing the number of preclinical and clinical safety studies required because the FDA has already

considered safety effects in the approved drug. In addition, AI can help identify subpopulations within a group of rare disease patients, allowing for more targeted and efficient clinical trials.

Looking Ahead

Experts agree that although AI-based drug development is exciting, we are still in early innings, and chaotic innings at that:

> “There's a lot of confusion in the industry about how [AI is] being applied.”
>
> **– Senior Scientist, AI Tools Provider | Expert Transcript**

Likewise, visibility is limited into how the field will advance and how quickly, with experts offering a range of opinions. The same expert quoted above offered a relatively upbeat view:

> “[In] two years… I’m cautiously optimistic that we’ll be able to start seeing the reward that AI offers the industry, but you just don’t know. I hate to be ambiguous like that, but that’s my take.”
>
> **– Senior Scientist, AI Tools Provider | Expert Transcript**

Another expert was more reserved:

> “At the moment I see a lot of reasonable expectations, but [there are also people] expecting things too soon in some areas. [There are] areas where people hope [AI] will work, [but] it may not work at all. In cheminformatics, in model analysis for small molecule discovery, in binding prediction, in generation of biologics, I'm pretty sure it will work pretty well. In things like target discovery using cell biological models, I'm less sure.”
>
> **– Former Vice President, AI Tools Provider | Expert Transcript**

As dynamics continue to unfold in AI-based drug development, new questions will most certainly arise and AlphaSense will be there to monitor developments with the help of first-person expert insights. Stay tuned!

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