Insilico Medicine
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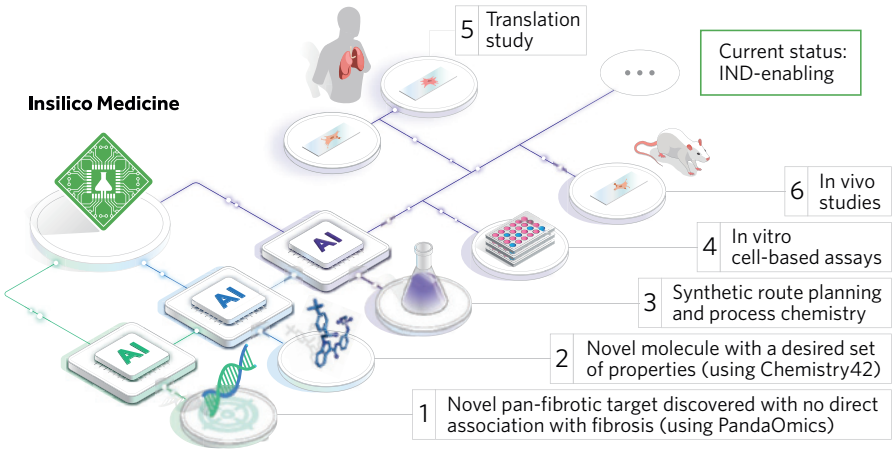
# Insilico: linking target discovery and generative chemistry AI platforms for a drug discovery breakthrough

**With its unique target discovery platforms, PandaOmics and Chemistry42, AI company Insilico is transforming the world of research and development.**

Deep learning in biopharma has come of age. After working for years to understand how to apply an artificial intelligence (AI) approach to biotechnology, Insilico Medicine recently disclosed the discovery of a novel drug target and novel molecule using AI. The discovery, a world first, took less than 18 months and cost 10% as much as a conventional program. Having validated its platforms, Insilico is now making the technology available to big pharmaceutical companies.

Insilico was founded in 2014 by a team of long-term academic collaborators, Alex Zhavoronkov and Alex Aliper. At that time, deep learning systems had just begun to outperform humans in image recognition. The breakthroughs triggered a surge in AI interest, but most projects focused on imaging, voice, and text. Training and validating deep neural networks to analyze those types of data takes days. Biology is far more challenging and time-consuming.

Insilico documented how its AI, biology and chemistry experts rose to that challenge and established ways to apply deep learning to biotech through the publication of more than 120 peer-reviewed papers in academic journals and at top AI conferences. The work culminated in establishing interconnected deep learning models and advanced AI approaches capable of delivering a preclinical drug candidate in under 18 months for just over $2 million.




**Linking biology and chemistry with AI.**

## Validating breakthrough AI platforms

Insilico validated its AI platform's end-to-end discovery capabilities in a project to create a drug for the lung condition idiopathic pulmonary fibrosis (IPF). Building on an initial hypothesis, Insilico trained its deep neural network on omics and clinical datasets to predict tissue-specific fibrosis.

This work led to the use of Insilico's PandaOmics target discovery system to identify targets through deep feature selection, causality inference and de novo pathway reconstruction. Insilico used a natural language processing engine to assess the targets' novelty and disease association via the analysis of data sources, including patents, research publications, and clinical trial databases. The process revealed 20 targets for validation that Insilico narrowed down to the most promising option.

Insilico applied its generative chemistry platform for drug discovery, Chemistry42, to the chosen novel intracellular target. The platform uses generative and scoring engines to come up with hit compounds from scratch. All molecules created by Chemistry42 automatically have drug-like molecular structures and suitable physicochemical properties. The application of Chemistry42 to the novel target revealed by PandaOmics led to the generation of a library of small molecules.

Multiple molecules showed promising on-target inhibition, with one hit achieving nanomolar IC50 values without showing any sign of CYP inhibition. Optimization of that hit, named ISM001, improved solubility and resulted in good ADME properties. Subsequent studies found the molecules improved fibrosis in a Bleomycin-induced mouse lung fibrosis model and were safe when given to mice in a 14-day dose range-finding experiment.

The whole process took less than 18 months. In that time, Insilico spent $2.6 million and emerged with a novel molecule against a novel target. Insilico is now preparing to rethink human studies for the AI age by training clinical trial prediction and optimization engines further, and developing new AI concepts to support clinical development of the IPF candidate.

## Opening the platforms to partners

Insilico will retain ownership of the candidate as it strives to digitize more of the R&D process before potentially bringing a co-development partner on board after phase 2. Pharmaceutical companies that want to benefit from Insilico's breakthroughs in deep learning today can partner to access the PandaOmics and Chemistry42 platforms it used to discover the novel target and novel molecule.

Insilico has shown that PandaOmics can identify novel molecular targets for various diseases, including IPF targets beyond the one it is pursuing. By pairing the target discovery power of PandaOmics to the generative chemistry capabilities of Chemistry42, Insilico stands to help its pharmaceutical partners transform drug discovery timelines and budgets.

The target and drug discovery platforms are just the beginning. Insilico has R&D centres in six countries or regions staffed with the winners of AI competitions worldwide, giving it the expertise needed to empower innovation in drug discovery globally and digitize the entire R&D process.

Insilico started more than a dozen preclinical programs and intends to take at least two of these programs into human clinical trials inventing new ways to apply AI to improve both enrollment and monitoring of clinical trials.

However, while focusing on internal development, Insilico also made its target discovery platforms, PandaOmics and Chemistry42 available for the other companies to substantially improve their drug discovery capabilities with multiple pharmaceutical companies deploying these systems.

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