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CASE STUDY · MARLUC SYNOPSIS

Insilico Medicine: rentosertib for idiopathic pulmonary fibrosis

IMPLEMENTED 2023–2026 · HEALTH AND LIFE SCIENCES · HONG KONG · BAND C · PARTLY PROVED

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Insilico used generative AI to pick a new target and design a molecule for idiopathic pulmonary fibrosis. A company-sponsored, peer-reviewed randomised trial showed a lung function gain at the top dose, and a phase III trial began in September 2026. No approval has been reported.

ABOUT THIS SYNOPSIS

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Facts and figures are as reported by the sources listed below, which may be incomplete, self-reported or out of date. Check the original source before relying on any figure.

The impact band, proof marker and SEAM view are MarLuc's opinion. The organisation is not a MarLuc client and has not reviewed or endorsed this synopsis. Names and trademarks belong to their owners.

NOTICE WORDING LAST UPDATED 16 NOVEMBER 2026

AT A GLANCE

Frontier case: AI used for a scientific, medical or engineering breakthrough. The transformation is potential and unproven; MarLuc grades what has changed in practice so far.

AI-native company: built around AI from the start and attempting transformational change. It claims band A; MarLuc grades it on what is evidenced.

AI used: Generative AI drug discovery platform: PandaOmics for target discovery and Chemistry42 for molecule design.

Implemented: 2023–2026. Grade: band C, Process change, Partly proved.

Headline result, as reported: AI-discovered target and molecule reached a randomised phase 2a trial (+98.4 ml lung function at 60 mg against -20.3 ml on placebo over 12 weeks) and a first phase III patient in September 2026

1 · The case

The facts, as reported by the 5 sources listed at the end. Where a source is silent, the field says "Not reported".

Organisation

Insilico Medicine. AI-native drug discovery company, founded in 2014 and listed on the Hong Kong stock exchange since December 2025. Described by pharmaphorum as US and Hong Kong based. Staff numbers: Not reported in the sources used. Canvas block: 6. Data and technology. Also 4. Operations and process, 3. Customers and proposition.

Business challenge

The challenge. Idiopathic pulmonary fibrosis (IPF) is an age-related progressive lung condition, and the paper says no current therapy reverses its course. The paper also says AI-discovered drugs had shown similar phase 2 failure rates to other drugs and none had passed phase 3.

How it was addressed. Insilico used its PandaOmics platform to rank fibrosis targets and named TNIK the top candidate, then used generative chemistry to design the TNIK inhibitor rentosertib (formerly ISM001-055). Phase I trials in healthy volunteers, including 78 people in one trial, reported comparable safety. The phase 2a trial randomised 71 patients with IPF to three doses of rentosertib or placebo for 12 weeks. Insilico began the GENESIS-IPF-3 phase III trial in China in September 2026. [1] [2] [3] [4] [5]

Who implemented it

In-house (In-house): Insilico Medicine: Developed the AI platform, designed the molecule and sponsored the trials..
Trial management and data analysis for the phase 2a were run by Fortrea Clinical Pharmacology Services, Leeds, per the paper. Peking Union Medical College Hospital leads the phase III trial, per the company release.

Technology (SEAM pillar E)

SEAM types: T5 Custom / fine-tuned models, T8 Predictive / ML models. Deployment: Not reported. Vendors and products: PandaOmics, Chemistry42, Pharma.AI.

Where it was built in: Built into Insilico's own discovery pipeline, from fibrosis target ranking to small-molecule design, followed by conventional preclinical and clinical testing.

What changed

Before: AI-discovered drugs had reached early trials but none had passed phase 3, per the paper. After: one AI-discovered target and molecule has reported a positive-direction randomised phase 2a result, with a phase III trial now under way, by the company's account. The way IPF drugs are tested has not changed, and no approval has been reported. [1] [2] [3] [4] [5]

What it cost

Not reported. Insilico raised HKD 2.28 billion (about $293 million) in its December 2025 IPO, per pharmaphorum.

What it saved

By the company's account in a peer-reviewed paper, about 18 months from target discovery to preclinical candidate nomination Savings in cost: Not reported. [1] [2] [3] [4] [5]

Governance and control

Randomised, double-blind, placebo-controlled design under ethics-committee approval and ICH Good Clinical Practice. Independent review came through Nature Medicine peer review. The sponsor employs several authors; accountable owner and AI-specific controls: Not reported.

S2 · Accountable owner · Not reported
Not reported. The paper names Insilico Medicine as the study sponsor. [1]

A1 · Safe use · Evidenced
The phase 2a trial followed the Declaration of Helsinki and ICH Good Clinical Practice, ethics committees approved the protocols and all patients gave written informed consent, per the paper. [1]

A2 · Human in the loop · Evidenced
Clinicians and ethics committees controlled testing in people: a randomised trial in which subjects, investigators and analysts were blinded to treatment. [1]

A3 · Monitoring and assurance · Evidenced
The paper reports adverse events by arm, including liver-related discontinuations, and was peer reviewed by Nature Medicine. The sponsor's employees are among the authors. [1]

A4 · Vendor AI · Not reported
Not reported. The paper describes PandaOmics as a commercially available platform; Insilico is the developer. [2]

A5 · Agent controls · Not reported
No agents described.

A6 · Standards and regulation · Evidenced
Trials were registered (NCT05938920 for phase 2a; NCT07687459 and CTR20262475 for phase III) and run under ethics-committee approval. No marketing approval is reported in the sources. [1] [4]

What worked, what did not, what is unproven

WORKED

– The primary endpoint, the share of patients with at least one adverse event, was similar across arms: 72.2%, 83.3% and 83.3% on rentosertib against 70.6% on placebo.

– At 60 mg once daily, forced vital capacity changed by +98.4 ml (95% confidence interval 10.9 to 185.9) against -20.3 ml (-116.1 to 75.6) on placebo.

– The drug moved to a phase III trial and the company raised funds on the Hong Kong exchange.

DID NOT

– 16 of 71 patients (22.5%) stopped treatment early; adverse events were the most frequent reason, and 7 of the 12 adverse-event discontinuations were for liver injury or dysfunction.

– The authors list small arms, a homogeneous population (all residents of China) and short follow-up as limits on judging long-term safety and efficacy.

– Employees of the sponsor are among the authors and were involved in trial design, data collection and analysis, per the paper.

UNPROVEN

– Whether the lung function gain holds in the 52-week phase III trial in 320 patients.

– Whether AI improves the odds of success in drug development; one trial cannot settle that.

– Regulatory approval: none reported. The company says approval would take three to four years from phase III start under favourable conditions.

Timeline

Mar 2024 Nature Biotechnology paper: AI-identified target and molecule, about 18 months from target discovery to preclinical candidate [2]

Jun 2024 Phase 2a trial ends [1]

Jun 2025 Phase 2a results published in Nature Medicine [1]

Dec 2025 Insilico lists in Hong Kong, raising HKD 2.28 billion [3]

Sept 2026 First patient dosed in the GENESIS-IPF-3 phase III trial in China [4]

2 · Grade and SEAM assessment

MarLuc's opinion, made on the evidence above. How cases are graded: marluc.co.uk/case-studies/how-we-rate

Level of change and impact

Claimed: A. Grade: C, Process change. Proof: Partly proved. Route: Model-led.

Process change (Level 2, band C): early discovery work redesigned around AI, with one candidate through to phase III. Claimed A: the company describes the drug as potentially transformative. That is potential and unproven.

Graded C. The company redesigned early drug discovery around AI and reports about 18 months from target discovery to preclinical candidate, and the drug has reached phase III. The effect on how the field works is not yet proved: no AI-discovered drug has been approved, the phase 2a trial was small and short, and the phase III trial has only just begun. Insilico and its trial lead describe the drug as potentially transformative; MarLuc treats that as potential and unproven.

The SEAM view and lessons learnt

The AI did the part it was built for: picking a target and designing a molecule quickly. Whether the drug works is decided where AI does not help, in patients over months and years. For a larger organisation, the transferable point is to judge AI by the independent test at the end of the chain, and to treat speed in the early steps as a separate claim.

1. Separate speed claims from outcome claims and prove each on its own.
2. Use randomised, blinded tests for results that matter.
3. Report the dropouts and the sponsor's role next to the headline.

Evidence

Score 3 of 4. Partly independent: a peer-reviewed randomised trial in Nature Medicine, sponsored and co-authored by the company; later trial milestones are the company's own releases.

Evaluation method. Peer-reviewed randomised phase 2a trial (Nature Medicine, 2025), company-sponsored; phase III start and IPO from the company's release and trade press. No regulator decision on approval.

Sources disagree. Insilico's release refers to Phase IIa positive results; the paper's primary

3 · Original sources

Read these in full. To challenge a fact or a grade, use the contact form at marluc.co.uk or the link on https://marluc.co.uk/case-studies/insilico-rentosertib-ipf

[1] Nature Medicine: A generative AI-discovered TNIK inhibitor for idiopathic pulmonary fibrosis: a randomized phase 2a trial (June 2025)
https://www.nature.com/articles/s41591-025-03743-2

[2] Nature Biotechnology: A small-molecule TNIK inhibitor targets fibrosis in preclinical and clinical models (March 2024)
https://www.nature.com/articles/s41587-024-02143-0

[3] pharmaphorum: Insilico ends 2025 with $293m Hong Kong IPO
https://pharmaphorum.com/news/insilico-ends-2025-293m-hong-kong-ipo

[4] News-Medical (Insilico Medicine release): Generative AI-driven drug Rentosertib enters Phase III trial for idiopathic pulmonary fibrosis (September 2026)
https://www.news-medical.net/news/20260910/Generative-AI-driven-drug-Rentosertib-enters-Phase-III-trial-for-idiopathic-pulmonary-fibrosis.aspx

[5] RTTNews: Insilico Medicine Doses First Patient In GENESIS-IPF-3 Phase III Trial (September 2026)
https://www.rttnews.com/3689895/insilico-medicine-doses-first-patient-in-genesis-ipf-3-phase-iii-trial.aspx

Insilico Medicine website: https://insilico.com

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