



Development of a Generative AI for Life Science Experimental Results and Its Application to Neurological Disease Drug Development



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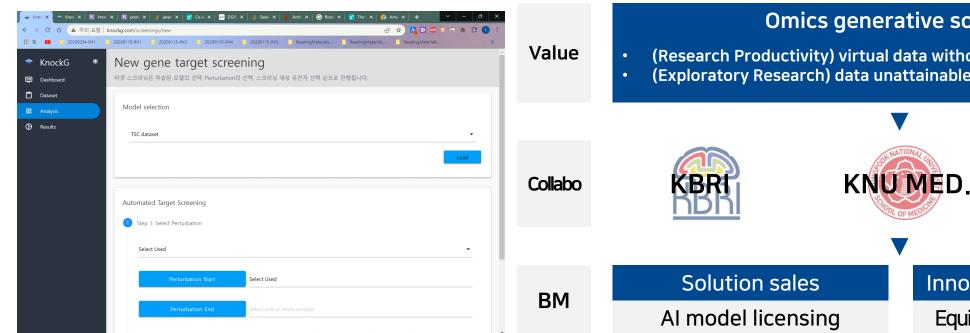
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# SilicoPharm, Inc.

### Al company developing generative Al solutions that enable researchers to virtually simulate experimental results

- · Core Technology: An AI technology that generates omics data to virtually produce experimental outcomes.
- · Business Model: Primarily S/W licensing of the Al solution, with selective in-house development of drug candidates for technology transfer



**Screenshot of solution** We aim to develop webbased(www.knockg.com) easy-to-use solution for researchers

### **Omics generative solution**

- (Research Productivity) virtual data without physical experiments
- (Exploratory Research) data unattainable by experimental methods

Researcher & Pharma

Innovative joint research

Equity in research outcomes

**Research Grant and collaborations** Founding by graduate students of DGIST's Brain Science program in 2021, SilicoPharm has pursued diverse research projects using its software platform.



# SilicoPharm, Inc.

Founded in July 2021 by Ph.D. students in Brain Science at DGIST, now led by the co-founders after completing their degrees

#### 2021

- Lab based Startup Univ. program
- Founded / SEED investment
- Selected for TIPS program
- DGIST CPRC MOU
- Prostate Cancer POC (target)

#### 2022

- Samsung–Daegu CCEI 13th C-Lab
- Early Start-up Package (Lab)
- Epliepsy POC (target)
- Liver Cancer POC (candidate)

#### 2024

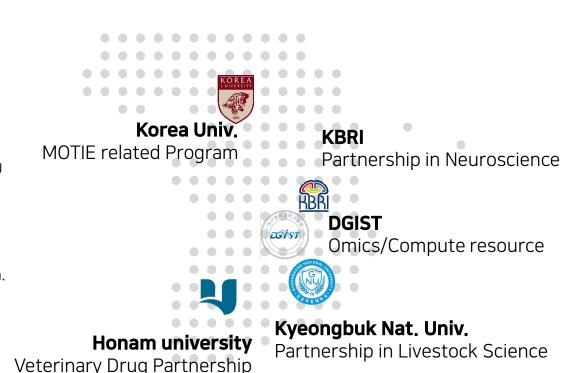
- **Deep Tech Startup** Grant
- KBRI / IBS solution deployment
- AD POC (candidate)
- NSCLC POC (candidate)
- Jintsbio, etc. Partenership

#### 2023

- DGIST Supercomputing MOU
- KBRI MOU
- Brain Strategic Tech Grant
- **ALS** POC (target)
- EWU solution deployment
- KIBO Venture Camp (13th)
- Business Meetings Pharma.
- KODIT Step-Up Guarantee

#### 2025

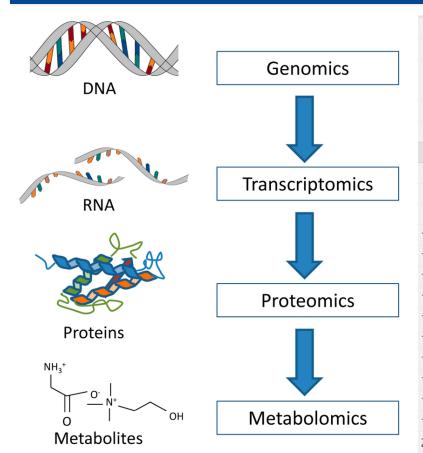
- Kyeongbuk Nat. Univ. MOU
- Kyeongsang Nat. Univ. MOU
- KNU Med., GNU, etc. solution deployment
- Human Resource Development Program by the Ministry of Trade, Industry and Energy (MOTIE)
- Honam univ, MOU
- Seoul Bio Hub residency
- K-Global AC program





## The Core of Modern Life Science – Omics

### -Omics is a terminology for wholistic approach in life science, produce few thousands by tens thousand information



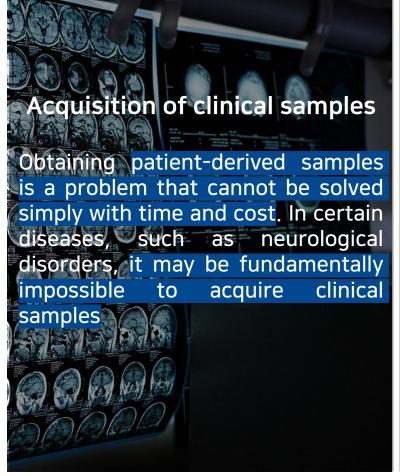
	А	В	С	D	Е	F	G	Н
1	gene	U937cell_Monocytesgenotype	U937cell_N	U937cell_N	U937cell_N	U937cell_N	U937cell_N	U937cell_NL
2	ENSG00000149531.15	7.784208506	3.535688	7.034182	5.369058	7.356506	7.126199	6.288241
3	ENSG00000141759.15	36.21779575	30.31863	30.74413	28.93852	28.06538	24.21635	24.08808
4	ENSG00000015133.20	6.049473639	2.474681	2.680479	2.44967	5.700953	4.477414	5.391512
5	ENSG00000242797.3	0.293700026	0.4145	0.427591	0.734337	0	0.866369	0.448725
6	ENSG00000203791.15	1.860495533	1.717456	1.533199	2.106472	2.51619	1.691323	2.681631
7	ENSG00000164663.15	2.148227007	1.687264	1.921859	1.718786	2.3629	3.049075	3.120406
8	ENSG00000106459.15	8.281820215	11.05827	9.530329	9.274757	12.17999	9.654999	10.60751
9	ENSG00000254470.3	128.4139827	98.35276	104.4698	124.6536	105.4694	107.4766	108.739
10	ENSG00000175322.12	0.051756095	0.06087	0.062792	0.012941	0.109112	0.152672	0.065896
11	ENSG00000250988.7	3.879741083	4.380396	4.142178	4.656243	3.271708	3.051901	3.754147
12	ENSG00000090615.16	24.36556974	26.51655	25.46799	29.08228	26.0905	21.39706	24.27025
13	ENSG00000265393.1	0	0	0.586567	0.604416	0.637039	0	0
14	ENSG00000249459.11	0.11910692	0.149419	0.192672	0.119121	0.125551	0.078077	0.202195
15	ENSG00000097021.20	108.9042214	87.1993	73.9792	77.86468	53.89134	48.77864	47.67518
16	ENSG00000226822.4	0.294405527	0.36933	0.428618	0.637954	0.362055	0.289483	0.399825
17	ENSG00000198010.13	0.013501776	0.025407	0.039314	0.013503	0.014232	0.013276	0.068762
18	ENSG00000165527.8	82.14917518	86.13742	95.19059	98.96067	124.3934	112.5	120.3559
19	ENSG00000108506.13	6.01416083	3.876323	5.170794	6.393766	6.813743	6.728479	6.825157
20	ENSG00000252274.1	2.982019971	1.402846	2.894302	1.491187	1.571673	0	1.518677
21	ENICCOOOOOOOTE 16	100 2206066	טחט חבבט	107 0066	212 1067	16/1202	100 1500	172 7070

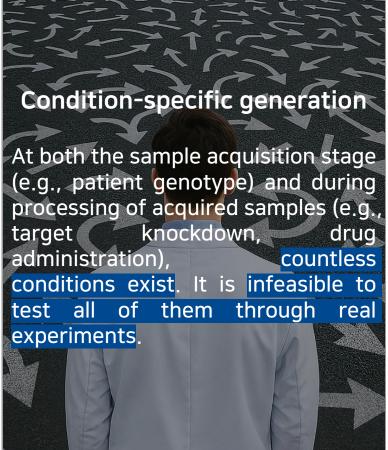
**Major omics domain(left) and example of omics data(right)** -Omics such as genomics, transcriptomics, proteomics, metabolomics produce high-dimensional data – Single-cell transcriptome which includes few thousand gene expression ly of each tens thousand cells.

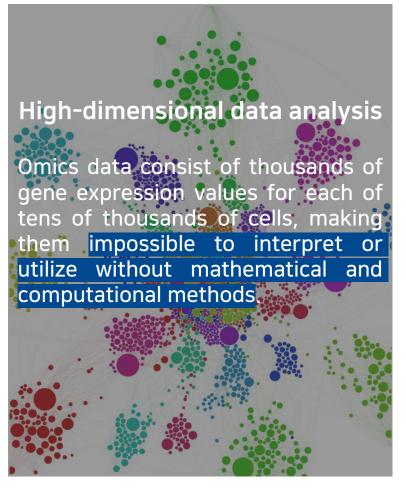


# Challenges in Acquiring, Producing, and Analyzing Omics Data

Omics enable comprehensive insights, three barriers continue to drive unmet medical needs and conservatism in market









## Virtual Experimental Data Generation Using an Omics Foundation Model

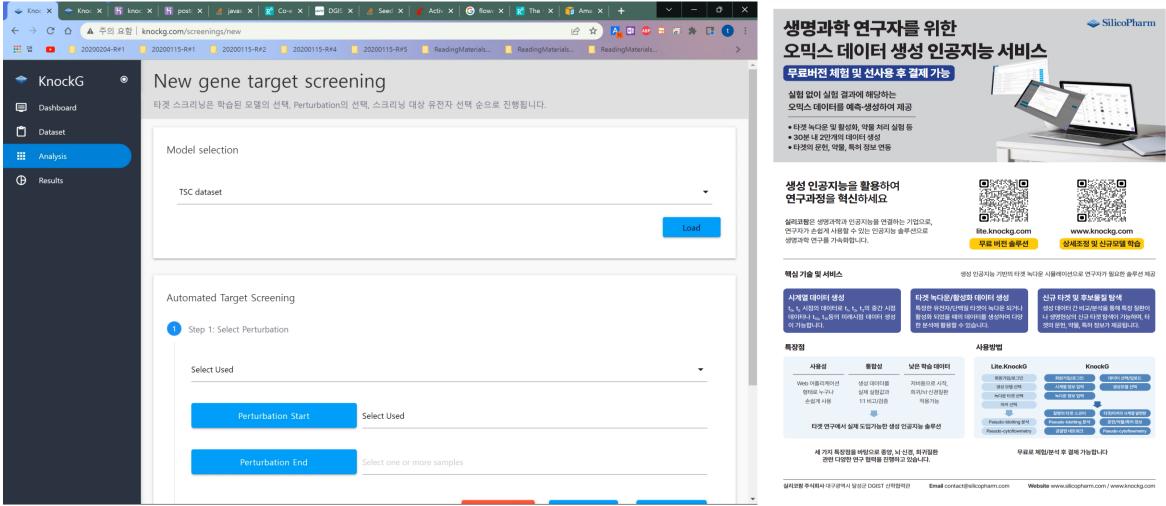
Omics foundation model enables generation of unseen/artificial omics data - like other foundation models

Model Name	Input	Output	Usage
GPT (ChatGPT)	Natural language data	Natural language data (Generated)	Text generation, translation, question answering, etc.
Alphafold	Amino acids sequence	Protein Structure	Drug development, functional protein design, etc.
KnockG	Experimental condition	Omics data (Generated)	Drug development, precision medicine, diagnostics, and therapeutics, etc.



## Virtual Experimental Data Generation Using an Omics Foundation Model

We have developed its AI solution as a web-based application (KnockG) - any researcher can easily access and utilize it





# **Application Cases: Validation in Tumors and Neurological Disorders**

We have completed data generation and experimental validation in liver cancer, epilepsy, Alzheimer's disease, ALS, and NSCLC

Disease Name	Data Generation & Derivation	Efficacy Verification	Results & Current Status
Liver Cancer		K-medihub Preclinical Center	<ul> <li>New target / repurposed cardiovascular drug</li> <li>Own cell-based experiments</li> <li>→ Project suspended due to newly arising issues</li> </ul>
Epilepsy	Generate knock-down data ↓ New target /drug candidate	Zefit	<ul><li>New target / repurposed drug</li><li>CRO efficacy evaluation confirmed effectiveness</li></ul>
Alzheimer's Disease			<ul> <li>New target / repurposed cardiovascular drug</li> <li>Efficacy evaluation confirmed one valid candidate</li> <li>→ Exploring related technology transfer</li> </ul>
ALS (Lou Gehrig's Disease)	Generate condition specific Knock-down data ↓ Disease mechanisms and therapeutic discovery	Korea Brain Research Institute	<ul> <li>Seven new targets identified</li> <li>Based on effective model results, 12 candidate substances s elected</li> <li>→ Exploring related technology transfer</li> </ul>
Non-Small Cell Lung Cancer (NSCLC)	Generate drug combination data ↓ Drug which enhance efficacy of the primary drug	K-medihub Preclinical Center	<ul> <li>Candidate identified for new target-based treatment for erlotinib-resistant patients</li> <li>Multiple target knockdown simulations identified one valid tr eatment regimen candidate</li> <li>→ Exploring related technology transfer</li> </ul>



## A Life Science Al Market Where Conservatism and Innovation Coexist

Ultimately, the key is not performance scores, but the ability to generate data that users can tangibly perceive and value.

Metric	AlphaFold	Insilico Medicine	KnockG (H1 2025)	KnockG (H2 2025)
Reproducibility & Scalability	<b>High</b> Numerous validation cases; 1:1 comparison possible wit h actual results	<b>High</b> Dozens of clinical pipelines	Medium Several lab-level validations, 1: 1 verification	<b>High</b> Clinical pipeline derivation, etc.
Third-Party Usability	<b>High</b> Partial code released and pu blic database available	<b>Low</b> No solution available with code release or usability	<b>High</b> Web-based solution provided	<b>High</b> Web-based solution provided
Effectiveness & Benefit	High Visually understandable res ults; scalability from tens to hundreds of existing proces ses	<b>Low</b> Effective only for pharmaceutic al companies, not for academic researchers	<b>Low</b> Limited academic outputs / au tomation	High Numerous publications; improved automation and diverse visualization features
Pioneering Use Cases	<b>High</b> Awarded Nobel Prize in 2024	<b>High</b> Lead compound/Phase 1-2 can didate derivation requires 9-18 months	<b>Low</b> Limited institutional POC	<b>High</b> Collaborations with pharma an d research institutions



## Foundation Models and Analytics with Standardization and Generalizability

Standardized and generalizable foundation model accelerate diversification and application across various domains

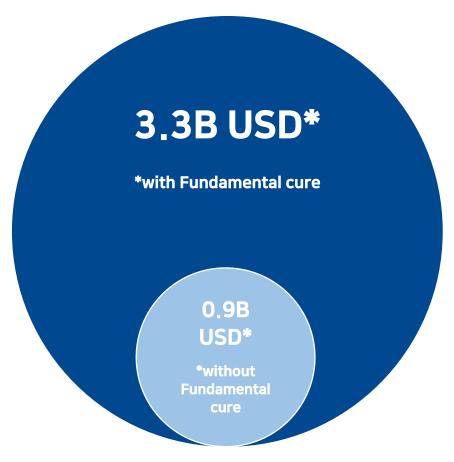
Model	Data generation and application
	Generate text that maintains contextual coherence to respond to user queries, serving as Chatbot
GPT (ChatGPT)	Draft text content tailored to various domains, such as marketing, advertising, articles, and website content
	Translate user-input text into other languages (language translation)
Alphafold	Generate protein structural data to facilitate research on protein structure and function
Alpharolu	Generate protein-drug binding structure data to predict protein-drug interactions and study protein function
	Generate and compare target knockdown data to discover novel disease targets or candidate compounds
KnockG	··· Organ- or tissue-specific data to predict potential drug toxicity
KIIOCKO	··· Quantitative omics data on dose-response relationships to determine optimal drug dosages
Colo Di	··· cohort-specific omics data responsive to a given drug to identify optimal clinical trial cohorts before initiation



# Markets Addressing Unmet Medical Needs such as ALS

### We aim to address previously untapped unmet medical needs and realize potential markets

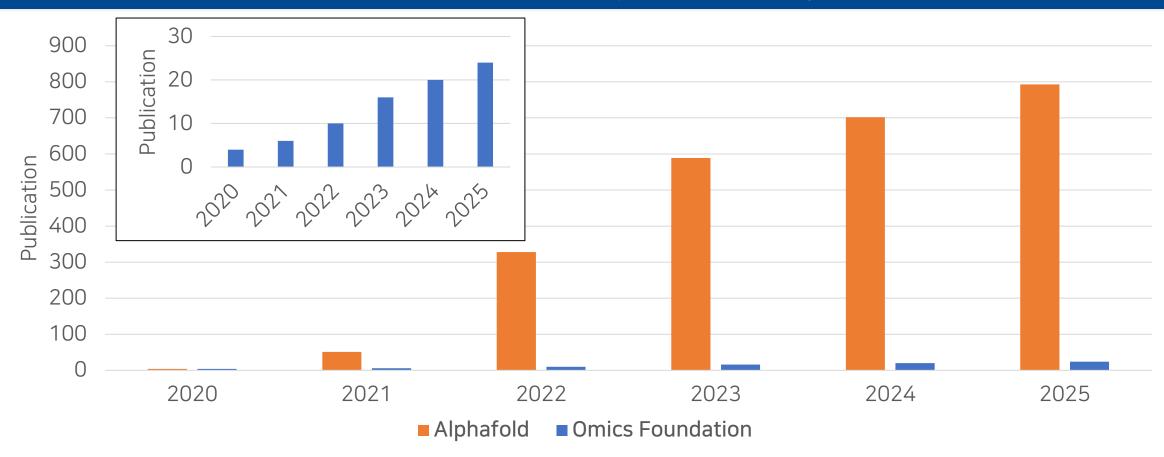
ltem	Details		
Target Disease	ALS (Amyotrophic Lateral Sclerosis) and motor neuron diseases		
Collaborating Institutions	Korea Brain Research Institute, Kyungpook National University School of Medicine		
Problem & Background	The cause of ALS remains unclear, and there is currently no fundamental tr eatment. Patients rely on a few drugs that can only alleviate certain sympto ms, and as the disease progresses, it leads to severe social costs due to th e loss of motor function.		
Solution	Utilizing an omics foundation model to generate condition-specific virtual d ata (e.g., motor neuron transcriptome/protein datasets), which cannot be o btained through actual experiments, and conducting fundamental research on the pathogenesis of ALS using the generated data.		
Outcomes & Current Status	<ul> <li>Selected and conducted as a national project (Brain Industry Strategic Te chnology Development Program)</li> <li>New causative genes of ALS identified, with subsequent research in progress</li> </ul>		



Addressing unmet medical needs (ALS therapeutics) In collaboration with the Korea Brain Research Institute, we are conducting research that was previously impossible by conventional methods. Through this, we aim to target the fundamental therapeutic market for ALS, which has remained only a potential market until now.

# Perspective & Milestone

### There has been no product in the omics foundation model that truly achieved explosive growth, unlike Alphfold



**An NCBI-based analysis of yearly publications** Unlike AlphaFold which experienced explosive growth and became a standard, omics foundation models—despite continuous publications—have not yet demonstrated such momentum.



# Perspective & Milestone

### Targeting explosive growth within 3 months after the free version launch in late 2025

	Sep. 2021	1H 2025	1H 2026	-2027
Key Goal	y Goal - Proof of concept & initial business		Scalable structure & Explosive user growth via free version	Market leadership in omics foundation models
Milestone	<ul> <li>SEED Funding</li> <li>Lab-level tests, 3rd-party validation</li> <li>Tech validation</li> <li>Product development, init users acquiring</li> <li>TIPS program</li> <li>Pro foundation model (0.5B-1B scale)</li> </ul>		<ul> <li>Pre-Series A funding</li> <li>Global entry (US pharma partners)</li> <li>mid-scale foundation model (5B-10B)</li> <li>3 months rapid user growth</li> </ul>	<ul><li> Top-tier partnerships</li><li> Clinical approvals</li><li> Large foundation model</li></ul>
Valuation	KRW 2B (~\$2M)	KRW 15B (~\$15M)	KRW 60B (~\$60M)	KRW 240B (~\$240M)
Notes	-	<ul> <li>~KRW 1.6B funding secured</li> <li>&gt;12 public validations</li> <li>Early revenue</li> <li>Partnerships (KBRI, Kyungpook Univ., etc.)</li> <li>Exclusive datasets &amp; Validation results</li> <li></li> </ul>	<ul> <li>Target 25 institutional clients</li> <li>Global support projects (K-Global AC)*</li> <li>Compute resource partners (Oracle)*</li> <li>Complement metric (6 publications, etc.)</li> <li>Scalable KPIs achieved</li> </ul>	-



# Perspective & Milestone



**Revenue projection** Based on projected adoption and the expected growth curve of foundation models, we anticipate entering a phase of explosive growth starting in 2026.



## **Team Status**

### **Key Executives & Team**



### Sangho Ji (CEO)

2018-2025 Ph.D. in Brain Science, DGIST 2017-2018 Post-Master Researcher at CPRC 2016-2018 M.S. in Medical Science, KNU School of Medicine;

#### Key Experience & Skills

- Life science and protein property experiments with data analysis
- mass spectrometer operation and data processing;
- molecular dynamics simulation

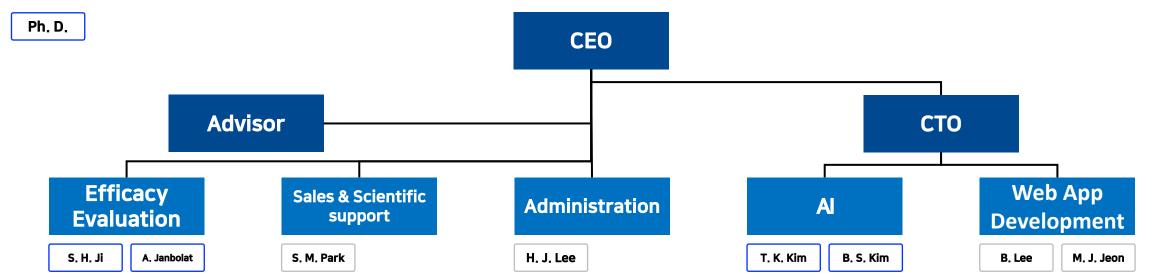


### Taehyeong Kim (CTO)

2020-2025 Integrated M.S./Ph.D. program in Brain Science, DGIST 2016-2018 Leader of "LikeLion" program at JBNU 2015-2019 B.S. in Biological Sciences, JBNU

#### Key Experience & Skills

- Life science and omics data analysis;
- leveraging AI in life science research;
- Web application development and server/database





## **Team Status**

#### **Advisors**



### **Wookyung Yu**



2016-Present Assistant/Associate Professor of Brain Science, DGIST

2017-Present Deputy Director (joint appointment) of Core Protein Resources Center

2016-Present Deputy Director (joint) of Supercomputing and Big Data Center ...

2012-2016 Postdoctoral Fellow at University of Chicago







REPORTS







### **Mookyung Cheon**



2016-Present Senior Researcher, Korea Brain Research Institute (KBRI)

2015-2016 Research Professor, DGIST Brain & Cognitive Science

2013-2014 Research Professor, DGIST New Biology

2009-2012 Research Professor, Pusan National University

2007-2009 Postdoctoral Fellow, North Carolina State University

2005-2007 Postdoctoral Fellow, University of Cambridge

2003-2005 Postdoctoral Fellow, Pusan National University

17 first-author SCI papers Machine learning and computational analysis techniques





112 Cited

240 Cited



