

---

## 에스티팜 신약개발 현황

---

2023. 03. 08

에스티팜

# Open Innovation Virtual R&D Strategy

Open Innovation Virtual R&D 전략을 통해 혁신신약 글로벌 임상 2건 진행 및 Out-licensing 1건 완료

## Background

- **Open Innovation Virtual R&D 전략**
  - 핵심역량만을 이용한 효율적인 신약개발전략
  - Virtual R&D 전략으로 신약개발 착수 (2013년)
- 총 47건의 혁신신약 프로젝트 검토 (2013~2021년)
- 약 15건의 프로젝트 구축 및 진행
- 미국과 유럽에서 글로벌 임상 2건 진행\*
- 국내 바이오텍에게 전임상후보물질 out-licensing (NASH 치료제, 2021년 11월)\*\*

## Full Time Employee (FTE)

- 신약연구팀 (4 FTEs)
  - Med. Chem. & ADME 핵심역량
- 신약개발팀 (4 FTEs)
  - Project Management & Business Development

## Key Projects

STP0404 (HIV 치료제)*	Emory & Colorado Univ.
STP1002 (항암제)*	Colorado Univ. 한국원자력의학원
NASH 치료제**	한국화학연구원
STP3725 (경구용헤파린)	B&L Deli Pharm
Dengue fever	온코크로스
Influenza	한국화학연구원

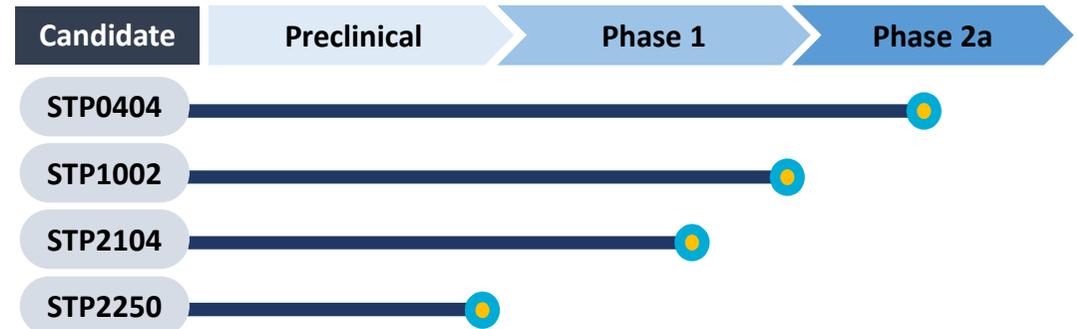
## R&D Cost\*

- 연 ~20억원 신약연구개발비 투입
    - 국책과제 활용 (KDDF 국책과제 – STP1002, 3725)
  - 글로벌 임상 프로젝트 개발비용 (~152억원/8 yrs)
    - STP0404 (72억원), STP1002 (80억원)
- \* 인건비 제외

## 에스티팜 신약 &amp; 백신 개발 현황

- **Pirmitegravir (STP0404)**
  - ✓ 임상 1상 (프랑스) 종료, 안전성/내약성 확보
  - ✓ 임상 2a상 (미국) 임상시험 승인 완료
  - ✓ 4월, First-in-Patient 예정
- **STP1002 (항암제)**
  - ✓ 임상 1상 cohort 7 진행 중, 안전성 확보
  - ✓ TKI와 병용투여를 위한 검증시험 진행 중
- **STP2104 (COVID-19 mRNA 백신)**
  - ✓ 한국·남아공에서 임상 1상 진행 중
  - ✓ KDDF 국책과제 선정 (연구지원비 29억원)
- **STP2250 (팬코로나 mock-up 백신)**
  - ✓ 국제백신연구소 (IVI)와 협업
  - ✓ 보건복지부 국책과제 선정 (1차 연구지원비 50억원)
  - ✓ 임상 1/2a상 국내 IND 접수 완료 (2월 23일)
  - ✓ 르완다 3월 10일 접수 예정

## 신약 &amp; 백신 파이프라인



## 2023년 목표 및 전략

- **STP0404:** 임상 2a상 진행
- **STP1002:** 임상 1상 완료 및 병용투여 기초연구 완료
- **STP2104:** 임상 1상 완료 및 에스티팜 플랫폼 기술 검증
- **STP2250:** 임상 1/2a상 완료 및 2b/3상 진입 준비
- **New Modality 구축**
  - ✓ Antibody-encoding RNA (AER) 신규 플랫폼 구축
  - ✓ mRNA-PROTAC & IL-23+Ab encoding mRNA 프로젝트

# 3 Publications & Presentation of Pirmitegravir (STP0404)

## PLOS PATHOGENS

RESEARCH ARTICLE

A highly potent and safe pyrrolopyridine-based allosteric HIV-1 integrase inhibitor targeting host LEDGF/p75-integrase interaction site

Tatsuya Maehigashi<sup>1</sup>, Seohyun Ahn<sup>2</sup>, Uk-II Kim<sup>2</sup>, Jared Lindenberg<sup>3</sup>, Adrian Oo<sup>1</sup>, Pratibha C. Koneru<sup>3</sup>, Bijan Mahboubi<sup>1</sup>, Alan N. Engelman<sup>4,5</sup>, Mamuka Kvaratskhelia<sup>3\*</sup>, Kyungjin Kim<sup>2\*</sup>, Baek Kim<sup>1,6\*</sup>

1 Department of Pediatrics, School of Medicine, Emory University, Atlanta, Georgia, United States of America, 2 ST Pharm Co., Ltd., Seoul, South Korea, 3 Division of Infectious Diseases, University of Colorado Anschutz Medical Campus, Aurora, Colorado, United States of America, 4 Department of Cancer Immunology and Virology, Dana-Farber Cancer Institute, Boston, Massachusetts, United States of America, 5 Department of Medicine, Harvard Medical School, Boston, Massachusetts, United States of America, 6 Center for Drug Discovery, Children's Healthcare of Atlanta, Atlanta, Georgia, United States of America

2022년

31 July–4 August 2023, Boulder, CO, USA

The 7th International Conference on Retroviral Integration

The 7th International Conference on Retroviral Integration  
Boulder, Colorado, USA: July 31 – August 4, 2023



## The Drug-Induced Interface That Drives HIV-1 Integrase Hypermultimerization and Loss of Function

Matthew R. Singer,<sup>a</sup> Tung Dinh,<sup>b</sup> Lev Levintov,<sup>c</sup> Arun S. Annamalai,<sup>b</sup> Juan S. Rey,<sup>c</sup> Lorenzo Briganti,<sup>b</sup> Nicola J. Cook,<sup>a</sup> Valerie E. Pye,<sup>a</sup> Ian A. Taylor,<sup>d</sup> Kyungjin Kim,<sup>e</sup> Alan N. Engelman,<sup>f,g</sup> Baek Kim,<sup>h,i</sup> Juan R. Perilla,<sup>c</sup> Mamuka Kvaratskhelia,<sup>b</sup> Peter Cherepanov<sup>a,d</sup>

<sup>a</sup>Chromatin Structure & Mobile DNA Laboratory, The Francis Crick Institute, London, United Kingdom

<sup>b</sup>Division of Infectious Diseases, School of Medicine, University of Colorado, Aurora, Colorado, USA

<sup>c</sup>Department of Chemistry and Biochemistry, University of Delaware, Newark, Delaware, USA

<sup>d</sup>Macromolecular Structure Laboratory, The Francis Crick Institute, London, United Kingdom

<sup>e</sup>ST Pharm Co. Ltd., Seoul, South Korea

<sup>f</sup>Department of Cancer Immunology and Virology, Dana-Farber Cancer Institute, Boston, Massachusetts, USA

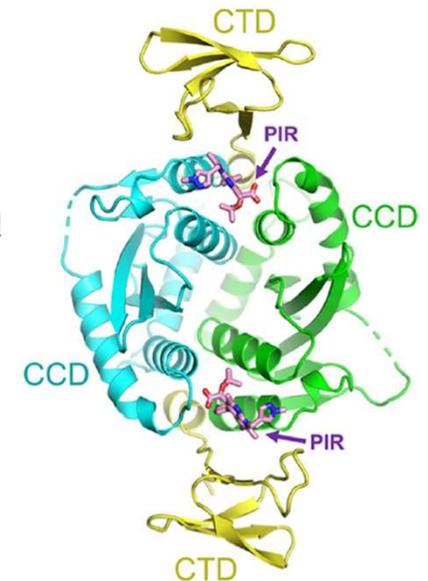
<sup>g</sup>Department of Medicine, Harvard Medical School, Boston, Massachusetts, USA

<sup>h</sup>Center for Drug Discovery, Children's Healthcare of Atlanta, Atlanta, Georgia, USA

<sup>i</sup>Department of Pediatrics, School of Medicine, Emory University, Atlanta, Georgia, USA

<sup>j</sup>Department of Infectious Disease, St-Mary's Campus, Imperial College London, London, United Kingdom

2023년



European Journal of Cancer 173 (2022) 41–51



Tankyrase-selective inhibitor STP1002 shows preclinical antitumour efficacy without on-target toxicity in the gastrointestinal tract

Dong Young Kim<sup>a,1</sup>, Young-Ju Kwon<sup>a,b,1</sup>, Won Yong Seo<sup>c,1</sup>,  
Uk-Il Kim<sup>c,1</sup>, Seohyun Ahn<sup>c</sup>, Seong Mi Choi<sup>c</sup>, Hyung Tae Bang<sup>c</sup>,  
Kyungjin Kim<sup>c,\*\*</sup>, Jae-Sung Kim<sup>a,b,\*</sup>

<sup>a</sup> Division of Radiation Biomedical Research, Korea Institute of Radiological and Medical Sciences, Seoul, South Korea

<sup>b</sup> Radiological and Medico-Oncological Sciences, University of Science and Technology, Seoul, South Korea

<sup>c</sup> ST Pharm Co., Ltd., Seoul, South Korea

Received 15 March 2022; received in revised form 10 June 2022; accepted 15 June 2022



April 14-19, 2023  
Orange County Convention Center  
Orlando, Florida

Tankyrase-selective inhibitor STP1002 reverses resistance to MEK inhibitors in colorectal cancer with *KRAS* mutations

Young-Ju Kwon<sup>1,2†</sup>, Dong Young Kim<sup>1†</sup>, Uk-Il Kim<sup>3</sup>, Xue Meng<sup>3</sup>, Ho Kyun Lee<sup>3</sup>, Hyung Tae Bang<sup>3</sup>, Jae-Sung Kim<sup>1,2\*</sup>, Kyungjin Kim<sup>3\*</sup>

<sup>1</sup>Division of Radiation Biomedical Research, Korea Institute of Radiological and Medical Sciences, Seoul, Korea

<sup>2</sup>Radiological and Medico-Oncological Sciences, University of Science and Technology, Seoul, Korea

<sup>3</sup>ST Pharm Co., Ltd., Seoul, South Korea

자체 유전자 치료백신개발 및 신규 CDMO 플랫폼 기술 구축  
Levatio와 Vernagen의 치료백신 연구 및 ST PHARM CDMO 생산을 통한 시너지 창출

### Levatio (San Diego, US)

Circular RNA 플랫폼을 기반으로 차세대 자가면역질환, 항암백신 개발 및 CAR-NKT 세포 치료제 개발

- **Autoantigen 백신 (자가면역질환)**
  - ✓ 효능 평가 및 비임상 시험 진행 중
  - ✓ 2024년 임상 1상 IND 제출 예정
- **Neoantigen 백신 (항암백신 – 폐암)**
  - ✓ 효능 평가 및 비임상 시험 진행 중
  - ✓ 2024년 임상 1상 IND 제출 예정
- **CAR-NKT 세포 치료제**
  - ✓ xRNA 전달기술을 통한 CAR-NKT 세포 치료제 개발
  - ✓ 기술력 확보 후 2024년 임상 1상 IND 제출 예정

### Vernagen (Atlanta, US)

에스티팜의 mRNA 플랫폼을 이용한 mRNA 기반 항바이러스 백신 및 치료제 개발

1. **예방백신 (Prophylactic vaccine)**
  - SFTSV (중증열성혈소판감소증후군) & RSV (호흡기세포융합) 백신
    - ✓ Immunogenicity 및 challenge 동물 실험 진행 중
  - VZV (수두대상포진) & HRTV (하트랜드) 백신
    - ✓ 효능평가 위한 비임상 시험 진행 예정
2. **치료백신 (Therapeutic vaccine)**
  - Humira (adalimumab)
    - ✓ 생물학적 활성 시험 진행 중



- 연면적 5,166m<sup>2</sup>, 지상 12층
- 올리고연구소, 합성연구소, 품질관리실 (QA)
- 대강당, 중소 회의실
- 카페 (ST-ONE)

# FAQ



## Why new drug development in CDMO?

- ✓ Synergic effect with CRO/CMO/CDMO business
- ✓ Total solution service in new drug development



# Win-Win Strategy in ST PHARM

차별화된 신약개발을 통해 신약 CDMO 핵심역량 강화 및 사업영역 확장  
신약개발+CDMO+CRO사업 결합으로 신약개발에서의 Seamless Platform Service 제공

## CRO

- 2019년 인수한 자회사 AnaPath CRO를 통해 CDMO사업 영역 확장 – 고객사에게 CDMO/CRO total package 제공
- 자체신약 및 백신개발 속도 향상으로 시너지 창출 – 시험일정 우선배정 및 신속한 피드백 확보
- 자체신약 out-licensing 이후에도 개발에 필요한 비임상 시험 서비스 지원 통해 안정적인 비즈니스 확보

## 신약개발 비임상부터 상업화 까지

## CDMO

- 신약개발에서의 핵심역량을 통해 신규사업 창출 – AnaPath 인수, mRNA 플랫폼 기술 및 연관 원료사업으로 확장
- 신약개발에서의 know-how와 경험을 통해 CDMO 사업영역의 확장 – AMD/AMV, CMC, Qbd/DoE 고부가가치 서비스 구축
- 자체 CMC 역량 확보를 통해 IND/NDA filing 서비스 제공
- 자체신약 out-licensing 이후에도 신약 API 공급권 확보

## Why still working on COVID vaccine?

- ✓ Beyond COVID-19 pandemics
- ✓ Response to another pandemics



# Beyond COVID-19 Strategy

## Beyond COVID-19: Collaborations with Vernagen

- Located in Atlanta, GA, USA
- Antibody-Encoding mRNA (AER) strategy: mRNA-based vaccines for infectious diseases
- Applied ST PHARM mRNA platform technology & LNP-mRNA GMP manufacturing



Target	Collaboration & Co-Development
Varicella Zoster Virus	Emory University, USA
Respiratory Syncytial Virus	Emory University, USA
SFTS Virus	Jeonbuk University, Korea
Nipah Virus	Duke-NUS University (Singapore)
Heartland Virus	US-Centers for Disease Control and Prevention
Langya Virus	Duke-NUS University (Singapore)
Pancorona Virus	Duke-NUS University (Singapore)



EMORY  
UNIVERSITY



*\* Two other internal pipelines at R&D stage*

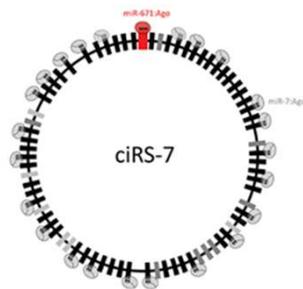
# Beyond COVID-19: Collaborations with Levatio



- Located in San Diego, CA, USA
- Building-up circRNA platform technology in Levatio & planting circRNA GMP manufacturing in ST PHARM



The emerging landscape of circular RNA in life processes, S. Qu and et al, 2016, 992-999



RNAs	Stability	Storage	Purification	Expression	Size
mRNA	X	X	X	X	○
Self-replicating RNA	X	X	X	○	△
CircRNA	○	○	○	○	△



# Response to future pandemic

Healthcare & Pharmaceuticals

2 minute read · October 21, 2022 3:11 PM GMT+9 · Last Updated 4 months ago

## Pfizer expects to hike U.S. COVID vaccine price to \$110-\$130 per dose

By Michael Erman

NEW YORK, Oct 20 (Reuters) - Pfizer Inc expects to roughly quadruple the price of its COVID-19 vaccine to about \$110 to \$130 per dose after the United States government's current purchase program expires, Pfizer executive Angela Lukin said on Thursday.

TRENDING —

## Moderna CEO: 400% price hike on COVID vaccine “consistent with the value”

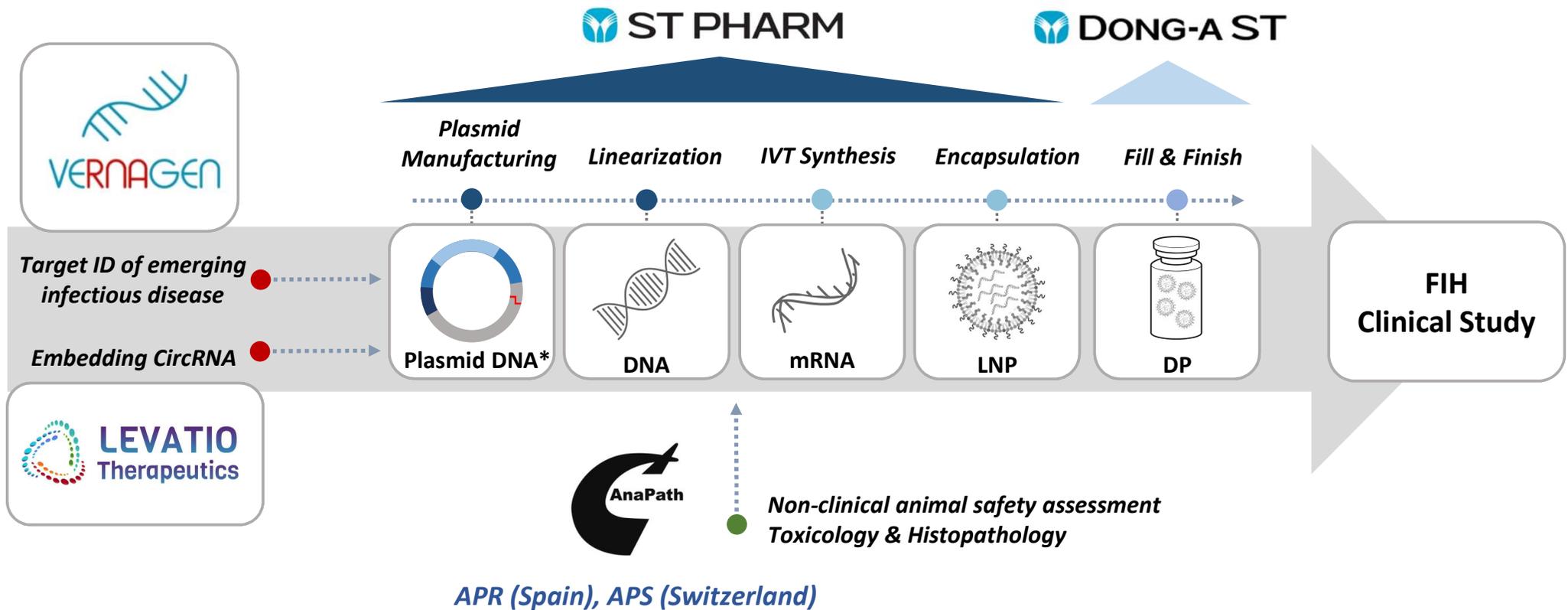
Lawmakers have already called Pfizer's similar plan "pure and deadly greed."

BETH MOLE - 1/11/2023, 4:21 AM

But now that the federal government is backing away from distributing the vaccines, their makers are moving to the commercial market—with price adjustments. Financial analysts had previously anticipated Pfizer would set the commercial price for its vaccine at just \$50 per dose but were taken aback in October when Pfizer announced plans of a price between \$110 and \$130. Analysts then anticipated that Pfizer's price would push Moderna and other vaccine makers to follow suit, which appears to be happening now.

# Pandemic response: Expedite-100 days strategy

*Developing xRNA vaccine against emerging infectious disease within 100 days*



감사합니다 Natick Dankon Taing  
Grazie Danke Ευχαριστώ Dalu 唔該  
Thank You köszönöm شكرا  
Tack Takk  
Спасибо Dank Gracias धन्यवाद  
谢谢 Merci Tak  
ありがとう Toda  
Tesekkür ederim Дякую khop kun  
Asante Gratias Shokran cảm ơn

Q&A

